UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-K/A
(Amendment No.1)

(Mark One)

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES AND EXCHANGE ACT OF 1934**
For the fiscal year ended December 31, 2023

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
For the transition period from to

Commission File Number: 001-39450

HARMONY BIOSCIENCES HOLDINGS, INC.
(Exact name of registrant as specified in its charter)

Delaware	**82-2279923**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
630 W. Germantown Pike, Suite 215, Plymouth Meeting, PA	**19462**
(Address of principal executive offices)	(Zip Code)

(484) 539-9800
(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.00001 value per share	HRMY	The Nasdaq Stock Market LLC (Nasdaq Global Market)

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☒ No ☐

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes ☐ No ☒

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act:

Large accelerated filer	☒	Accelerated filer	☐
Non-accelerated filer	☐	Smaller reporting company	☐
		Emerging growth company	☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☒

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to §240.10D-1(b). ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

The aggregate market value of the voting and non-voting stock held by non-affiliates of the Registrant, as of June 30, 2023, the last business day of the registrant's most recently completed second fiscal quarter, was $1,174.5 million. As of February 16, 2024, the registrant had 56,769,081 shares of common stock, $0.00001 par value per share, outstanding.

Auditor Firm ID: 34 Auditor Name: Deloitte & Touche LLP Auditor Location: Philadelphia, Pennsylvania

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive proxy statement relating to its 2024 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission within 120 days after the end of the fiscal year ended December 31, 2023, are incorporated herein by reference in Part III where indicated.

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Part I

Cautionary Note Regarding Forward-Looking Statements

This Annual Report on Form 10-K contains forward-looking statements. We intend such forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). All statements other than statements of historical facts contained in this Annual Report on Form 10-K, including statements regarding our future results of operations and financial position, our business strategy, products, prospective products, product approvals, research and development costs, anticipated timing and likelihood of success of clinical trials, expected timing of the release of clinical trial data, the plans and objectives of management for future operations and future results of anticipated products, are forward-looking statements. These statements involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements.

In some cases, you can identify forward-looking statements by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target," "project," "contemplate," "believe," "estimate," "predict," "potential", or "continue" or the negative of these terms or other similar expressions. The forward-looking statements in this Annual Report on Form 10-K are only predictions. We have based these forward-looking statements largely on our current expectations and projections about future events and financial trends that we believe may affect our business, financial condition and results of operations. These forward-looking statements speak only as of the date of this Annual Report on Form 10-K and are subject to a number of important factors that could cause actual results to differ materially from those in the forward-looking statements, including the factors described under the sections in this Annual Report on Form 10-K titled "—Item 1A. Risk Factors." and "Part II—Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations."

Moreover, we operate in an evolving environment. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties.

Unless otherwise indicated, information contained in this Annual Report on Form 10-K concerning our industry, including industry statistics and forecasts, competitive position and the markets in which we operate is based on information from independent industry and research organizations, other third-party sources and management estimates. Management estimates are derived from publicly available information released by independent industry analysts and other third-party sources, as well as data from our internal research, and are based on assumptions made by us upon reviewing such data, and our experience in, and knowledge of, such industry and markets, which we believe to be reasonable. In addition, projections, forecasts, assumptions and estimates of the future performance of the industry in which we operate and our future performance are necessarily subject to uncertainty and risk due to a variety of factors, including those described herein under "—Item 1A. Risk Factors." These and other factors could cause results to differ materially from those expressed and forecasts in the estimates made by the independent parties and by us.

You should read this Annual Report on Form 10-K and the documents that we reference in this Annual Report on Form 10-K completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of our forward-looking statements by these cautionary statements. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

As used herein, the terms "Harmony," "we," "us," "our" and "the Company" refer to Harmony Biosciences Holdings, Inc., a Delaware corporation, and our operating subsidiary, Harmony Biosciences, LLC.

Further, we have in-licensed from Bioprojet the registered trademark product name WAKIX® in the United States. We also have registered trademark protection in the United States for KNOW NARCOLEPSY®, REM AT THE WRONG TIME® and NON-REM AT THE WRONG TIME®, as well as our brand and logo HB®, HB HARMONY BIOSCIENCES® and HARMONY BIOSCIENCES®. This report also includes trademarks, service marks and trade names of other companies. Trademarks, service marks and trade names appearing in this Annual Report on Form 10-K are the property of their respective owners.

Summary Risk Factors

Our business is subject to numerous risks and uncertainties. You should carefully consider these risks and uncertainties when investing in our securities. The principal risks and uncertainties affecting our business include the following:

- We are substantially dependent on the commercial success of our only approved product, WAKIX. If we are unable to maintain or increase sales of WAKIX, our ability to generate revenue and our financial condition will be adversely affected.

- The continued commercial adoption of WAKIX and any other product candidates we develop will depend on the degree of their market acceptance.

- We rely on our license agreements with Bioprojet to provide rights to the core intellectual property relating to pitolisant, and any termination or loss of significant rights under the agreement would adversely affect our development and/or commercialization of pitolisant.

- Because a number of companies compete with us, many of which have greater resources than we do, and because we face rapid changes in science in our industry, we cannot be certain that our products will be accepted in the marketplace or capture market share.

- Public health pandemics, including the COVID-19 pandemic, may result in disruptions to our commercialization, clinical trials, manufacturing and other business operations, which could have a material adverse effect on our business, financial condition, operating results, cash flows and prospects.

- We have a limited operating history and history of commercializing drugs, which may make it difficult for you to evaluate the success of our business to date and assess our future viability.

- We may not be successful in our efforts to identify, in-license or acquire, discover, develop or commercialize additional product candidates, or identify other indications for pitolisant beyond EDS or cataplexy in adult patients with narcolepsy.

- The regulatory approval process of the FDA is costly, lengthy and inherently unpredictable, and our business may be substantially harmed if experience insufficient results in the course of our business development or are ultimately unable to obtain regulatory approval for any of our ongoing development programs in other potential indications.

- If we fail to obtain and sustain an adequate level of coverage and reimbursement for WAKIX and other product candidates by third-party payors, sales would be adversely affected.

- We have made and may be required in the future to make significant payments to Bioprojet under our licensing and collaboration agreements for pitolisant.

- Raising additional funds by issuing securities may cause dilution to existing shareholders, raising additional funds through debt financings may involve restrictive covenants, and raising funds through lending and licensing arrangements may restrict our operations or require us to relinquish proprietary rights to our technologies or product candidates.

- WAKIX has been approved by the FDA for the treatment of EDS in adult patients with narcolepsy, and cataplexy in adult patients with narcolepsy. Regulatory approval is limited by the FDA to the specific indication for which approval has been granted and, unless we seek regulatory approval for additional indications, we will be prohibited from marketing pitolisant for other indications. We may be subject to fines, penalties or injunctions if we are determined to have promoted or be promoting the use of pitolisant for unapproved or “off-label” uses, resulting in damage to our reputation and business.

- If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.

- If we fail to comply with our obligations under any license, collaboration or other agreements, we may be required to pay damages and could lose intellectual property rights that are necessary for developing and protecting our current and future product candidates.

- Our directors, officers and principal stockholders beneficially own a significant percentage of our stock and are able to exert significant control over matters subject to stockholder approval.

- Future sales of our common stock in the public market, including sales by our directors, officers, or significant shareholders, could cause our share price to fall.

Item 1. Business.

Overview

We are a commercial-stage, pharmaceutical company focused on developing and commercializing innovative therapies for patients living with rare neurological diseases as well as patients living with other neurological diseases who have unmet medical needs. Our product, WAKIX (pitolisant), is a first-in-class molecule with a novel mechanism of action ("MOA") specifically designed to increase histamine signaling in the brain by binding to H_3 receptors. In August 2019, WAKIX was approved by the U.S. Food and Drug Administration (the "FDA") for the treatment of excessive daytime sleepiness ("EDS") in adult patients with narcolepsy, and its U.S. commercial launch was initiated in November 2019. In October 2020, WAKIX was approved by the FDA for the treatment of cataplexy in adult patients with narcolepsy. WAKIX is the first-and-only approved product for patients with narcolepsy that is not scheduled as a controlled substance by the U.S. Drug Enforcement Administration (the "DEA").

We believe that pitolisant's ability to regulate histamine gives it the potential to provide therapeutic benefit in other rare neurological diseases that are mediated through H_3 receptors and histamine signaling. We are taking a mechanism-based approach to managing the life cycle of pitolisant and identified idiopathic hypersomnia ("IH"), another central disorder of hypersomnolence like narcolepsy, as our next potential new indication for pitolisant. We received orphan drug designation by the FDA for IH in September 2023 and Fast Track Designation in November 2023. In April 2022, we initiated a Phase 3 registrational trial, the INTUNE Study, to evaluate the efficacy and safety of pitolisant in adult patients with IH. We completed enrollment in the INTUNE study in May 2023 and we announced topline data in October 2023. While the primary endpoint did not meet statistical significance, we believe the totality of the data showed favorable numerical trends for pitolisant in the treatment of adult patients with IH and we have scheduled a meeting with the FDA for March 2024 to discuss the path forward for IH. We are focusing our development efforts on other rare neurological disorders in which EDS is a prominent symptom, including Prader-Willi Syndrome ("PWS") and myotonic dystrophy, otherwise known as dystrophia myotonica ("DM"). Based on the positive signals from the Phase 2 proof-of-concept signal detection clinical trial to evaluate pitolisant for the treatment of EDS and other key symptoms in patients with PWS, an end-of-phase 2 meeting with the FDA was held in June 2023. We believe we aligned with the FDA on the proposed Phase 3 registrational study design for further investigation of pitolisant as a potential treatment to address the unmet medical need for children, adolescents and adults with PWS experiencing EDS, for which there is currently no approved treatment. In October 2023, we received FDA alignment regarding the protocol for the Phase 3 TEMPO study in patients with PWS, which we believe will satisfy the requirements for both the registrational trial and one of the two requirements for pediatric exclusivity for pitolisant, with the other requirement being data in pediatric narcolepsy. In February 2024, the FDA granted Orphan Drug designation to pitolisant for the treatment of PWS. We expect to initiate the Phase 3 study in the first quarter of 2024. In June 2021, we initiated a Phase 2 proof-of-concept signal detection clinical trial to evaluate pitolisant for the treatment of EDS, fatigue and cognitive dysfunction in adult patients with DM1 and announced positive topline results from this trial in the fourth quarter of 2023 in which clinically meaningful improvements were demonstrated in EDS and fatigue, and the safety profile was consistent with the established safety profile of pitolisant.

Our partner, Bioprojet completed a Phase 3 trial in pediatric patients with narcolepsy and submitted the trial data to the European Medicines Agency (the "EMA") seeking approval for a pediatric narcolepsy indication. On January 26, 2023, Bioprojet received a positive opinion from the EMA's Committee for Medicinal Products for Human Use ("CHMP") and in March 2023, the EMA granted approval for the marketing authorization of WAKIX for the treatment of narcolepsy in children

6 and older. Based on the data from the positive Phase 3 trial conducted by Bioprojet, we submitted an sNDA for pediatric narcolepsy in December 2023. On February 21, 2024, we announced that the FDA has granted priority review of our pediatric narcolepsy sNDA and has set a Prescription Drug User Fee Act ("PDUFA"), or target action date, of June 21, 2024. We remain committed to obtaining pediatric exclusivity for WAKIX.

We also seek to expand our pipeline through the acquisition of additional assets that focus on addressing the unmet needs of patients living with rare neurological diseases as well as patients living with other neurological diseases who have unmet medical needs. We are targeting assets that will allow us to further leverage the expertise and infrastructure that we have successfully built at Harmony so we can optimize the benefit of internal synergies. Consistent with this objective, in July 2022, we entered into a License and Commercialization Agreement (the "2022 LCA") with Bioprojet whereby we obtained exclusive rights to manufacture, use and commercialize one or more new products based on pitolisant in the United States and Latin America, with the potential to add additional indications and formulations upon the agreement of both parties. We have made progress in the development of two formulations, Next Gen 1 ("NG1") and Next Gen 2 ("NG2"). Both formulations entered clinical studies in the fourth quarter of 2023, and we anticipate data in the first half of 2024.

In addition, on October 10, 2023, the Company completed a tender offer to acquire all of the outstanding shares of common stock (the "Zynerba Acquisition") of Zynerba Pharmaceuticals, Inc. (together with its subsidiary, Zynerba Pharmaceutical Pty, Ltd., "Zynerba") for $60.0 million, and incurred transaction related costs of approximately $2.6 million. Zynerba is a clinical-stage pharmaceutical company focused on innovative pharmaceutically produced transdermal cannabidiol therapies for orphan neuropsychiatric disorders, including Fragile X syndrome ("FXS"). The phase 3 RECONNECT registration study in FXS is ongoing.

In August 2021, we acquired HBS-102, a Melanin-concentrating hormone receptor 1 (MCHR1) antagonist previously developed as CSTI-100/ALB-127258(a)/ALB-127258 (the "Compound"), along with intellectual property and other assets related to the development, manufacture, and commercialization of the Compound from ConSynance Therapeutics, Inc. In connection with the acquisition, we made an upfront payment of $3.5 million and will be required to make certain payments upon the achievement of certain development milestones, regulatory milestones, and sales milestones and pay ongoing royalties upon commercialization. We acquired full development and commercialization rights for HBS-102 globally, but we have provided an indication-limited grant-back license to ConSynance for the development and commercialization of the Compound in Greater China. We are conducting a preclinical proof-of-concept study to assess the effect of HBS-102 on hyperphagia, weight gain and other metabolic parameters in a mouse model of PWS and are also conducting a thirteen-week toxicology study. We anticipate data from both studies in the first half of 2024.

Pitolisant was developed by Bioprojet and approved by the EMA in 2016 for the treatment of narcolepsy in adult patients with or without cataplexy, and in 2021 for the treatment of EDS in adult patients with obstructive sleep apnea. We acquired an exclusive license to develop, manufacture and commercialize pitolisant in the United States pursuant to our license agreement with Bioprojet (as amended, the "2017 LCA") in July 2017. Pitolisant was granted Orphan Drug Designation for the treatment of narcolepsy by the FDA in 2010. It received Breakthrough Therapy designation for the treatment of cataplexy in patients with narcolepsy and Fast Track status for the treatment of EDS and cataplexy in patients with narcolepsy in April 2018.

Our operations are conducted by our wholly owned subsidiaries, Harmony Biosciences, LLC, which was formed in May 2017 and Zynerba.

Narcolepsy Market Overview

Narcolepsy is a rare, chronic and debilitating neurological disorder of sleep-wake state instability that is estimated to affect approximately 170,000 Americans, with fewer than 50% diagnosed. Narcolepsy is characterized by EDS, which is present in all patients with narcolepsy and is the primary reason why patients seek treatment. EDS is the inability to stay awake or alert throughout the day, including an irrepressible need for sleep, with lapses into drowsiness or sleep, which has a significant impact on a patient's ability to function. Additional symptoms of narcolepsy may include cataplexy (which is characterized by sudden and transient episodes of muscle weakness accompanied by full conscious awareness), hallucinations, sleep paralysis and disrupted nighttime sleep. In most patients, narcolepsy is caused by the loss of orexin/hypocretin, a neuropeptide in the brain that, along with histamine, works to support sleep-wake state stability. This disorder affects men and women equally, with typical symptom onset in adolescence or young adulthood; however, it can take up to a decade after onset of symptoms to be properly diagnosed. The U.S. narcolepsy market had an approximate net sales value of $2.5 billion in 2022. The market is expected to continue to grow based on several factors, including, but

not limited to, the introduction of new innovative therapies that offer novel mechanisms of action resulting in improved safety/tolerability profiles while delivering clinically meaningful efficacy, additional investment in education, increased rates of diagnosis, and population growth.

Prior to the FDA's approval of WAKIX in 2019, there were six approved medications to treat patients with narcolepsy, all of which are scheduled as controlled substances, and no new therapies had been approved for narcolepsy patients in the United States since 2007. These medications included Xyrem (sodium oxybate), Provigil (modafinil), Nuvigil (armodafinil), Ritalin (methylphenidate), Adderall (amphetamine salts) and Sunosi (solriamfetol). Following the approval of WAKIX, in July 2020 the FDA approved a new lower sodium formulation of Xyrem called Xywav, followed by generic versions of sodium oxybate in January 2023 and July 2023, as well as a once-nightly version of sodium oxybate (Lumryz) in June 2023. These approved drugs are prescribed in accordance with their individual labels for indications covering narcolepsy, cataplexy and/or EDS related to narcolepsy, and have demonstrated the ability to improve the lives of the patients suffering from these symptoms. In addition, Xywav was approved in August 2021 for the treatment of IH in adults. Other prescription drugs are used off-label for the treatment of either EDS or cataplexy in patients with narcolepsy, including stimulants for EDS and antidepressants for cataplexy. Despite the benefits provided by the available medications, according to the American Academy of Sleep Medicine ("AASM"), traditional stimulants, wake-promoting agents and sodium oxybate, at best, provide only moderate improvement in narcolepsy symptoms and side effects may limit their use. Some of the current therapies have significant side effects (such as increased heart rate and blood pressure) and boxed warnings due to the risk of respiratory depression, abuse, dependence and diversion. These therapies also have the potential for rebound and withdrawal symptoms. The Voice of the Patient report from the FDA's patient-focused drug development initiative, published in 2014, concluded that, based on the overall benefit-risk assessment of current medications, there is a continued need for additional effective and tolerable treatment options for patients with narcolepsy. In a retrospective electronic chart review conducted by Rush University Medical Center from June 2011 to December 2018, over 75% (73 out of 97 respondents) of patients with narcolepsy reported at least one residual symptom while on their current treatment. In a third-party survey that we commissioned prior to the commercialization of WAKIX, of the 200 patients with narcolepsy who were surveyed, 86% (173 out of 200 respondents) of patients reported narcolepsy is a life changing disorder and 93% (157 out of 169 respondents) expressed frustration with current treatment options, while 31 patients were not on treatment and, as such, did not provide a response to this question. The main drivers of patients' dissatisfaction were side effects and tolerability, loss of efficacy over time and concerns about abuse and dependence with current therapies.

In market research sponsored by us prior to the commercial release of WAKIX, both patients and HCPs expressed frustration and dissatisfaction with then-existing therapies, reflecting current unmet medical needs. These unmet needs included, in order of importance, the availability of: (i) non-scheduled treatment options, (ii) more tolerable treatment regimens, (iii) more effective treatment options, (iv) novel MOAs beyond currently available therapies and (v) once-daily treatment options. Based on our market research, we believe the most significant unmet need identified was the availability of non-scheduled treatment options. Other than WAKIX, all drugs approved by the FDA for the treatment of narcolepsy, including stimulants, are scheduled as controlled substances by the DEA. Controlled substances have the potential for abuse, misuse, and diversion. In addition, these products also have the potential for the development of tolerance and withdrawal symptoms. Despite their inherent drawbacks, due to the limited number of treatment options, stimulants have historically been a primary treatment for people with narcolepsy. In addition to having the potential for abuse, all of the treatments approved for narcolepsy, except WAKIX, require a Risk Evaluation and Mitigation Strategy ("REMS") program, which is required by the FDA for certain medications with serious safety concerns to help ensure the benefits of the medication outweigh its risks.

Our Solution

WAKIX (pitolisant) represents a novel approach to narcolepsy treatment. We believe that WAKIX offers a meaningfully differentiated product profile over current treatment options for the following reasons:

- **First-in-class molecule with a novel MOA**. WAKIX is the only selective H_3 receptor antagonist/inverse agonist approved by the FDA. It is approved for the treatment of EDS or cataplexy in adult patients with narcolepsy and is the only narcolepsy treatment that works primarily through histamine, a major wake-promoting neurotransmitter. Pitolisant is thought to work by regulating histamine, such that it activates wake-promoting neurons and inhibits sleep promoting neurons, which helps to stabilize states of sleep and wakefulness. We believe that these novel characteristics differentiate it from other narcolepsy treatments.

- **First-and-only non-scheduled treatment for narcolepsy**. WAKIX is the first-and-only FDA-approved treatment for narcolepsy that is not scheduled as a controlled substance by the DEA. We believe one of the most significant unmet needs is the availability of non-scheduled treatment options. In a clinical trial, pitolisant demonstrated statistically significantly lower abuse potential compared to phentermine (a Schedule IV stimulant), consistent with its lack of abuse potential.

- **WAKIX is not a stimulant**. Stimulants are one of the most commonly prescribed treatments for patients with narcolepsy. Unlike stimulants, in clinical trials, WAKIX showed no evidence for the development of drug tolerance or withdrawal symptoms. Therefore, there is no need for patients to temporarily stop the medication to reset efficacy. In addition, unlike stimulants, WAKIX does not increase dopamine levels in the brain's reward center, which contributes to its lack of abuse potential. According to the National Sleep Foundation, stimulants have the potential for abuse, so their use must be considered carefully by patients and HCPs. WAKIX gives patients and HCPs a different therapeutic option.

- **WAKIX can be used as monotherapy or administered concomitantly with other narcolepsy treatments**. Narcolepsy is a difficult disorder to manage and the majority of narcolepsy patients often require multiple medications to treat their symptoms. WAKIX was studied in combination with each of modafinil and sodium oxybate (two common treatments for narcolepsy) and demonstrated no effect on the pharmacokinetic ("PK") profile of either treatment, and neither treatment had a clinically relevant effect on the PK profile of WAKIX. We believe the ability of WAKIX to be taken as monotherapy or concomitantly with other narcolepsy medications affords HCPs the flexibility to better manage their patients with narcolepsy.

- **WAKIX is a once-daily oral tablet administered in the morning upon wakening**. Patients have identified a need for treatment options that are easier to take and are dosed less frequently. We believe that once-daily dosing with WAKIX addresses this need and may help improve patient compliance with treatment.

Our Strategy

Our goal is to become a leading pharmaceutical company dedicated to developing and commercializing novel treatment options for patients living with rare neurological diseases and/or neurological diseases where we can leverage our existing infrastructure and expertise. The key elements of our corporate growth strategy are outlined below:

- Continued Growth of WAKIX in Adult Patients with Narcolepsy.

    - **Continued Commercialization of WAKIX in the United States**. We have assembled a team of approximately 250 professionals who possess comprehensive life sciences experience. We have also established a robust company infrastructure to execute on our core business and growth strategies. This team includes over 80 dedicated and experienced sales professionals who call on the approximately 9,500 HCPs who treat more than 90% of narcolepsy patients in the United States. In November 2019, we launched commercial sales of WAKIX in the United States.

- Advance the clinical development programs, extend the pitolisant franchise, expand the pipeline and diversify the product portfolio beyond sleep/wake disorders.

    - **Pursue New Indications Beyond Narcolepsy.** We believe that pitolisant's novel MOA offers a portfolio in a product opportunity and has therapeutic potential in several other patient populations living with rare neurological diseases. We opened an IND for pitolisant for the treatment of IH in November 2021 and initiated a Phase 3 registrational trial, the INTUNE Study, in adult patients with IH in April 2022. We completed enrollment in the INTUNE study in May 2023 and we announced topline data in October 2023. While the primary endpoint did not meet statistical significance, we believe the totality of the data showed favorable numerical trends for pitolisant in the treatment of adult patients with IH. Pitolisant received Orphan Drug designation for IH by the FDA in September 2023 and Fast Track Designation in November 2023. We submitted a meeting request to the FDA in the fourth quarter of 2023 to discuss the path forward and we have scheduled a meeting with the FDA in March 2024. We opened an IND for pitolisant for the treatment of PWS in November 2019 and completed a Phase 1 PK clinical trial in pediatric patients with PWS in December 2019. We then initiated a long-term, open-label safety trial in those patients who participated in

the Phase 1 PK trial. In December 2020, we initiated a Phase 2 proof-of-concept signal detection clinical trial to evaluate pitolisant for the treatment of EDS and other key symptoms in patients with PWS, and based on the positive signals from the data, an end-of-phase 2 meeting with the FDA was held in June 2023. We believe we aligned with the FDA on the proposed Phase 3 registration study design for further investigation of pitolisant as a potential treatment to address the unmet medical need for children, adolescents and adults with PWS experiencing EDS, for which there is currently no approved treatment. In October 2023, we received FDA alignment regarding the protocol for the Phase 3 TEMPO study in patients with PWS, which we believe will satisfy the requirements for both the registrational trial and one of the two requirements for pediatric exclusivity, with the other requirement being data in pediatric narcolepsy. In February 2024, the FDA granted Orphan Drug designation to pitolisant for the treatment of PWS. We expect to initiate the Phase 3 study in the first quarter of 2024. In June 2021, we initiated a Phase 2 proof-of-concept signal detection clinical trial to evaluate pitolisant for the treatment of EDS, fatigue and cognitive dysfunction in adult patients with DM1 and announced topline results from this trial in the fourth quarter of 2023 in which clinically meaningful improvements were demonstrated in EDS and fatigue, and the safety profile was consistent with the established safety profile of pitolisant.

  - **Expand WAKIX Label in Narcolepsy**. Building upon the EDS and cataplexy indications in adult patients with narcolepsy, we are working with our partner Bioprojet in gaining a pediatric indication for both EDS and cataplexy. Bioprojet completed a Phase 3 trial in pediatric patients with narcolepsy and submitted the data to the EMA seeking approval for a pediatric narcolepsy indication. On January 26, 2023, Bioprojet received a positive opinion from the EMA's CHMP and in March 2023, EMA granted approval for the marketing authorization of WAKIX for the treatment of narcolepsy in children 6 and older. Based on the data from the positive Phase 3 trial conducted by Bioprojet, we submitted an sNDA for pediatric narcolepsy in December 2023. On February 21, 2024, we announced that the FDA has granted priority review of our pediatric narcolepsy sNDA and has set a PDUFA, or target action date, of June 21, 2024.

  - **Pursue Pediatric Exclusivity**. We continue to pursue pediatric exclusivity, and we recently received feedback from the FDA regarding our request for a pediatric written request ("PWR"). We plan to prepare a proposed pediatric study request ("PPSR") for submission to the FDA in order to align with the agency, in pursuit of pediatric exclusivity for WAKIX. We remain committed to obtaining pediatric exclusivity for WAKIX and the submission of a pediatric sNDA in the fourth quarter of 2023 and the planned initiation of a Phase 3 study in PWS in the first quarter of 2024 are consistent with our efforts to obtain pediatric exclusivity.

  - **Expand Pitolisant Franchise Potentially up to 2040 and Beyond**. Pursuant to the 2022 LCA with Bioprojet, we have made progress in the development of two new formulations of pitolisant, NG1 and NG2. Both formulations entered into clinical studies in the fourth quarter of 2023 and we anticipate data in the first half of 2024.

  - **Expand and Diversify our Product Portfolio**. As we continue our commercial growth and advancement of our clinical development programs with pitolisant, a key component of our strategy is to expand our pipeline and diversify our portfolio beyond sleep/wake disorders by partnering, co-developing or acquiring assets focused on rare neurological diseases and/or other neurological diseases serving patients with unmet medical needs that are complementary to our existing research and development expertise and/or commercial footprint. Consistent with this objective, in October 2023, we acquired Zynerba, a clinical-stage pharmaceutical company focused on innovative pharmaceutically produced transdermal cannabidiol therapies for orphan neuropsychiatric disorders, including FXS. The phase 3 RECONNECT registration study in FXS is ongoing.

- Maintain disciplined capital allocation strategy to maximize shareholder value.

  - **Execute on Businesses Development Opportunities**. We believe that with our existing cash, cash equivalents and investments on hand as of December 31, 2023, we are well positioned to execute on any business development opportunity to build out a robust pipeline.

  - **Share Repurchase Program**. We continue to opportunistically execute our share repurchase program to return value to shareholders.

Our Commercialization Strategy

We launched WAKIX into the narcolepsy market in November 2019 and we continue to engage with HCPs, patients and payors through the focused commercialization strategy outlined below to optimize adoption of WAKIX in the marketplace:

- **HCP Awareness and Adoption**: To facilitate HCP awareness and adoption of WAKIX, we have deployed our dedicated, over 80-person sales team to educate a defined prescriber base of approximately 9,500 HCPs comprised of neurologists, pulmonologists, sleep specialists, psychiatrists and high-prescribing primary care physicians who specialize in or focus on sleep disorders. We believe these HCPs diagnose and treat more than 90% of the narcolepsy patients in the United States. We began our commercial HCP outreach in August 2019 following FDA approval of WAKIX for the treatment of EDS in adult patients with narcolepsy and our efforts continue following the approval of the cataplexy indication in October 2020.

- **Patient Awareness**: It is estimated that narcolepsy affects approximately 170,000 Americans with fewer than 50% diagnosed. Of those living with narcolepsy in the United States, it is estimated that fewer than 40,000 are on narcolepsy medications, which we believe indicates a significant unmet medical need. To drive patient awareness of WAKIX and its differentiated product profile, we have been communicating with the narcolepsy patient community and providing them with educational materials and information on WAKIX.

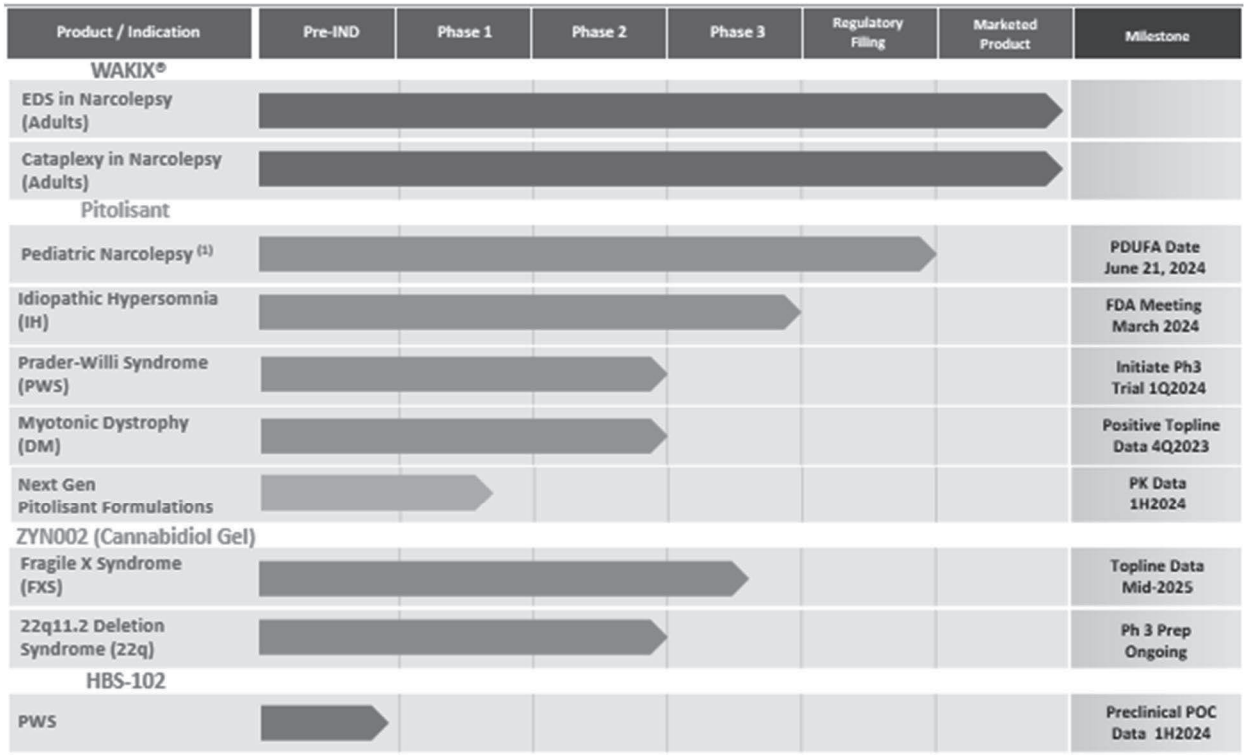
- **Payor Coverage*:*** Recognizing the importance of payor coverage, our field market access team has been engaging with national and regional payors over the past four plus years to educate them on the clinical data and value proposition of WAKIX. Through December 31, 2023, we have secured formulary access for more than 80% of all insured lives (Commercial, Medicare and Medicaid) in the United States. Within these covered lives, we have observed favorable access to WAKIX subsequent to the expanded approval of WAKIX for the treatment of cataplexy in adult patients with narcolepsy in October 2020.

We believe the differentiating attributes of WAKIX that have facilitated and will continue to facilitate awareness, adoption, and coverage include: (i) it is a first-in-class molecule with a novel MOA, (ii) it is the first-and-only non-scheduled treatment approved for EDS or cataplexy in adult patients with narcolepsy, (iii) it is not a stimulant, (iv) it has broad clinical utility because it can be used as monotherapy or administered concomitantly with other narcolepsy treatments, and (v) it is a once-daily oral tablet administered in the morning upon wakening. In September 2021, the AASM updated its clinical practice guidelines on central disorders of hypersomnolence, which serve as evidence-based treatment guidelines for such disorders, giving WAKIX a strong recommendation for the treatment of both excessive daytime sleepiness and cataplexy in adult patients with narcolepsy.

As of December 31, 2023, we continued to see growth in unique HCPs prescribing WAKIX since it became available in November 2019. The average number of patients on WAKIX in the fourth quarter of 2023 was approximately 6,150. Additionally, as of December 31, 2023, we had secured formulary access for more than 80% of all insured lives (Commercial, Medicare and Medicaid) in the United States. Within these covered lives, we observed favorable access to WAKIX subsequent to the expanded approval of WAKIX for the treatment of cataplexy in adult patients with narcolepsy in October 2020.

Our Development Pipeline

Development Pipeline Chart



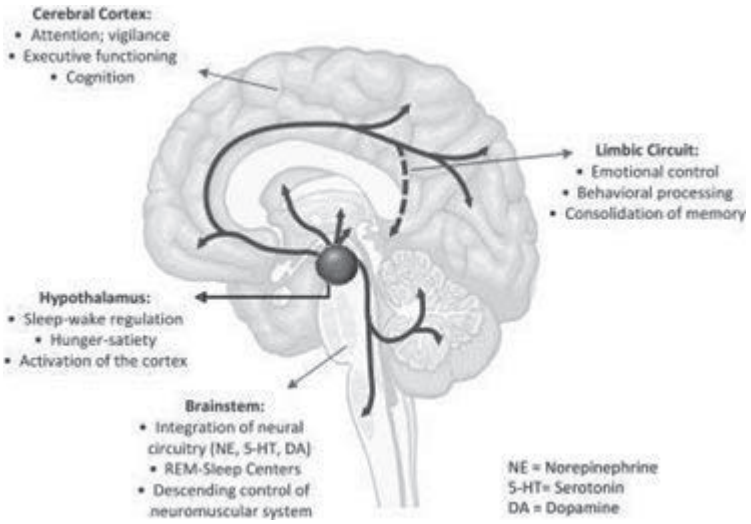
1. Trial conducted by Bioprojet; received EMA approval on March 15, 2023.

Develop Pitolisant in New Patient Populations in Pursuit of Additional Indications

We believe that pitolisant's ability to regulate histamine and histaminergic signaling gives it the potential to provide therapeutic benefit in other disorders that are mediated through the H_3 receptor and histamine signaling and offers a *portfolio in a product* opportunity with pitolisant. Histamine plays an important role in normal physiologic functioning beyond wakefulness in the areas of attention, vigilance, behavior and cognition. The presence of H_3 receptors in the hypothalamus, brainstem and cerebral cortex account for different functions, which could provide an opportunity for pitolisant to treat symptoms other than EDS in different disorders. In addition, H_3 receptors are located mainly in the CNS as opposed to other parts of the body outside the CNS, which contributes to its overall tolerability profile. This fact, along with pitolisant being highly selective for the H_3 receptor (as opposed to H_1 receptors, H_2 receptors and H_4 receptors), is the reason we

believe in pitolisant's unique MOA and its potential to improve symptoms in patients living with rare neurological diseases, in which impaired histamine signaling is part of the underlying pathophysiology.

- Role of histamine in normal physiologic functioning beyond wake promotion (e.g. attention, vigilance, behavior, cognition)
- Location of H_3 receptors in hypothalamus, brainstem, and cerebral cortex account for different functions (and potential symptoms in different disorders)
- Limited H_3 receptor populations outside the CNS



We have continued to seek new indications in rare neurological disease patient populations that have EDS as a prominent symptom, along with other symptoms mediated by CNS histamine signaling. We opened an IND for pitolisant for the treatment of IH in November 2021 and initiated a Phase 3 registrational trial, the INTUNE Study, in April 2022. We completed enrollment in the INTUNE study in May 2023 and we announced topline data on October 11, 2023. While the primary endpoint did not meet statistical significance, we believe the totality of the data showed favorable numerical trends for pitolisant in the treatment of adult patients with IH. We submitted a meeting request to the FDA in the fourth quarter of 2023 to discuss the path forward and we anticipate a meeting with the FDA towards the end of the first quarter of 2024. We plan to interact with the FDA to determine next steps. We opened an IND for pitolisant for the treatment of PWS in November 2019 and completed a Phase 1 PK clinical trial in pediatric patients with PWS in December of 2019. We then initiated a long-term, open-label safety trial in those patients who participated in the Phase 1 PK trial. In December 2020, we initiated a Phase 2 proof-of-concept signal detection clinical trial to evaluate pitolisant for the treatment of EDS and other key symptoms in patients with PWS, including behavioral symptoms and cognitive impairment. Topline results from this clinical proof-of-concept trial were reported in November 2022 and showed a positive signal on improvement in the primary outcome related to EDS. An end-of-phase 2 meeting with the FDA was held in June 2023. We believe we aligned with the FDA on the proposed Phase 3 registration study design to support investigation of pitolisant as a potential treatment to address the unmet medical need for children, adolescents and adults with PWS experiencing EDS, for which there is currently no approved treatment. In October 2023, we received FDA alignment regarding the protocol for the Phase 3 TEMPO study in patients with PWS, which will satisfy the requirements for both the registrational trial and one of the requirements for pediatric exclusivity, with the other requirement being data in pediatric narcolepsy. In February 2024, the FDA granted Orphan Drug designation to pitolisant for the treatment of PWS. We expect to initiate the Phase 3 study in the first quarter of 2024. In June 2021, we initiated a Phase 2 proof-of-concept signal detection clinical trial to evaluate pitolisant for the treatment of EDS, fatigue and cognitive dysfunction in adult patients with DM1 and announced topline results from this trial in the fourth quarter of 2023 in which clinically meaningful improvements were demonstrated in EDS and fatigue, and the safety profile was consistent with the established safety profile of pitolisant.

Idiopathic Hypersomnia

IH, like narcolepsy, is another central disorder of hypersomnolence resulting in sleep-wake state instability. It is a rare and chronic neurological disease that is characterized by EDS despite sufficient or even long sleep time. Sleep in patients with IH is not felt to be refreshing, no matter how much sleep they get. People living with IH experience significant EDS along with the symptoms of sleep inertia (prolonged difficulty waking up from sleep) and 'brain fog' (impaired cognition, attention, and alertness). Symptoms of IH often begin in the late teens or early twenties but patients often experience a long diagnostic journey and do not receive an accurate diagnosis until reaching their thirties. Based on insurance claims data, the number of patients diagnosed with IH in the US ranges from 30,000 to 40,000.

The Phase 3 double-blind, placebo controlled randomized withdrawal study evaluated the safety and efficacy of pitolisant compared with placebo in adult patients with IH (ages ≥18 years) The study consisted of an 8-week Open-Label Phase, which included a 6-week Dose Optimization Period followed by a 2-week Stable Dose Period. Patients determined to be responders at the end of the Stable Dose Period entered the Double-Blind Randomized Withdrawal Phase of the study for 4 weeks of blinded treatment with pitolisant (at the same dose administered in the Stable Dose Period) or matching placebo. The primary efficacy endpoint in the study was the change in the Epworth Sleepiness Scale (ESS) score from the end of the Stable Dose Period (Week 8) to the end of the 4-week Double-Blind Randomized Withdrawal Phase (Week 12) for pitolisant compared with placebo. Secondary efficacy endpoints evaluated the effect of pitolisant on other symptoms of IH, including overall burden of disease, sleep-related impairment, sleep inertia, and cognitive function. Patients who completed the trial are eligible to participate in an open-label extension phase to assess the long-term safety and effectiveness of pitolisant in adult patients with IH.

We completed enrollment in the INTUNE study in May 2023 and we announced topline data in October 2023. Topline study results include:

- Approximately 83% of patients who completed the 8-week open-label treatment period with pitolisant were responders (as defined by a decrease on the ESS of ≥3 points) and experienced a robust clinical response, with an average ESS change from baseline of – 9.4 points.

- A positive trend favoring pitolisant was observed during the 4-week double-blind randomized withdrawal period, however no statistically significant difference was observed between pitolisant and placebo groups on ESS, the primary endpoint.

- Positive trends favoring pitolisant were also observed across additional prespecified endpoints including the IH Severity Scale, which approached statistical significance, as well as on the Sleep Inertia Questionnaire. Further data analyses are being conducted.

- Approximately 88% of patients in the Double-Blind Randomized Withdrawal Phase continued into a long-term extension study, which is ongoing.

- The safety and tolerability profile of pitolisant in adult patients with idiopathic hypersomnia was consistent with the established safety profile and no new safety signals were observed.

While the primary endpoint did not meet statistical significance, we believe the totality of the data showed favorable numerical trends for pitolisant in the treatment of adult patients with IH. We are currently analyzing the full data set and we have scheduled a meeting with the FDA for March 2024 to discuss the path forward for IH.

Prader-Willi Syndrome

PWS is a rare genetic disorder caused by a loss of function of specific genes on chromosome 15 resulting in hypothalamic dysfunction and decreased levels of orexin/hypocretin in some patients with PWS. The hypothalamus controls both sleep-wake states and hunger-satiety; therefore, two of the main symptoms in patients with PWS are EDS and hyperphagia. Other features include low muscle tone, short stature, behavioral problems and cognitive impairment. It is estimated that approximately 15,000 to 20,000 people in the United States suffer from PWS, and over half of those suffering from PWS experience EDS. We opened an IND for pitolisant for the treatment of PWS in November 2019 and completed a Phase 1 PK clinical trial in pediatric patients with PWS in December of 2019. We then initiated a long-term, open-label safety trial in those patients who participated in the Phase 1 PK trial. In December 2020, we initiated a Phase 2 proof-of-concept clinical trial to evaluate pitolisant for the treatment of EDS and other key symptoms in patients with PWS, including behavioral symptoms and cognitive impairment. Topline results from this clinical proof-of-concept trial were reported in November 2022 and showed a positive signal on improvement in the primary outcome related to EDS.

PWS poses a heavy burden for both patients and caregivers and there are few therapeutic options available and no FDA-approved treatments for EDS in patients with PWS. Clinical development programs in PWS have focused on hyperphagia, with no other programs focusing on EDS or cognitive function. EDS is thought to have a negative effect on behavior and cognitive function and could exacerbate these symptoms in patients with PWS. In addition, impaired histamine

signaling in the brain can contribute to behavioral symptoms and impaired cognition. We believe there is a compelling opportunity for the mechanism of action of pitolisant to impact the EDS component of this disorder as well as other symptoms, such as behavioral issues and cognitive impairment.

We have collaborated with the Foundation for Prader-Willi Research (the "FPWR") to advance our clinical program and underscore our commitment to this patient population. We are members of the FPWR Clinical Trials Consortium and are working with members of its Scientific Advisory Board to gain their insights for our development program. Progress to date includes (i) opening an IND for PWS in October 2019, (ii) completion of a Phase 1 PK trial in patients with PWS in December 2019, with patients actively rolling over into an open-label, long-term safety trial, and (iii) completion of a Phase 2 proof-of-concept signal detection clinical trial with topline data reported in November 2022.

The Phase 2 proof-of-concept clinical trial was a randomized, double-blind, placebo-controlled study designed to assess the safety and efficacy of pitolisant in patients with PWS. This proof-of-concept study was not powered to demonstrate statistical significance and was designed for signal detection. The study included patients ages 6 to 65 years and patients were randomized 1:1:1 to low dose pitolisant, high dose pitolisant, or placebo treatment groups. Pitolisant dosing was based on three age cohorts (children 6 to < 12; adolescents 12 to < 18; and adults 18 to 65) and another objective of the study was to evaluate for a dose-response to pitolisant in patients with PWS. The primary endpoint of this study was the evaluation of EDS as measured by change from baseline to end of treatment (EOT) on the Epworth Sleepiness Scale for Children and Adolescents (ESS-CHAD) Parent/Caregiver Version.

Topline study results include:

- 65 patients were enrolled in the trial, 91% of which completed treatment and all but one patient opted to continue into the open-label extension.
- Mean baseline ESS-CHAD (Parent/Caregiver Version) ranged from 14.7 to 15.7.
- Mean change from baseline to EOT on the ESS-CHAD Parent/Caregiver Version scores ranged from -3.7 to -5.5 across all age groups and treatment groups, representing a clinically meaningful change (which is defined as a ≥ 2-point improvement on this scale).
- In two of the three age groups (children and adults), there was a clinically meaningful difference (minimum of 2 points) between pitolisant and placebo, driven by the high dose pitolisant treatment group.
- In the adolescent age group, there was a high placebo response of a magnitude three times that seen in the other two age groups, which resulted in no clinically meaningful difference between pitolisant and placebo in this age group.
- A responder analysis (defined as an improvement on the ESS-CHAD Parent/Caregiver Version of ≥ 3-points or a score at EOT of ≤10 for this analysis) showed response rates of 70% in the high dose pitolisant group, 55.6% in the low dose pitolisant group, and 52.6% in the placebo group.
- Overall safety/tolerability profile was consistent with the known safety/tolerability profile of pitolisant.
- Adverse events were reported in 57% of patients on pitolisant and 65% of patients on placebo.
- Treatment-related adverse events were reported in 26% of patients on pitolisant and 30% of patients on placebo.
- There was one serious adverse event in a patient in the placebo treatment group.

These results represent the initial topline data.

Based on the positive signals from the data from our Phase 2 proof-of-concept signal detection clinical trial to evaluate pitolisant for the treatment of EDS and other key symptoms in patients with PWS, an end-of-phase 2 meeting with the FDA was held in June 2023. We believe we aligned with the FDA on the proposed Phase 3 study design to support

further investigation of pitolisant as a potential treatment to address the unmet medical need for children, adolescents and adults with PWS experiencing EDS, for which there is currently no approved treatment. In October 2023, we received FDA alignment regarding the protocol for the Phase 3 TEMPO study in patients with PWS, which we believe will satisfy the requirements for both the registrational trial and one of the requirements for pediatric exclusivity, with the other being data in pediatric narcolepsy. In February 2024, the FDA granted Orphan Drug designation to pitolisant for the treatment of PWS. We expect to initiate the Phase 3 TEMPO study in the first quarter of 2024.

Myotonic Dystrophy

DM is a rare, multi-system genetic disease that affects the neuromuscular system as well as several other systems. The primary symptom in patients with DM is myotonia, which is an impairment in the ability of muscles to relax; progressive muscle weakness is another prominent symptom of the disorder. It is inherited in an autosomal dominant pattern and there are two main types: DM1 and DM2. The underlying cause of DM1 is a mutation in the DMPK gene on chromosome 19. DM1 is the most common form of adult-onset muscular dystrophy and affects as many as 160,000 patients in the United States. EDS and fatigue are hallmark clinical characteristics in the majority of patients with DM1 and are referred to as the most frequent non-muscular symptoms in patients with DM1. EDS and fatigue occur in approximately 80% to 90% of patients with DM1. Cognitive impairment is also a prominent symptom in patients with DM1 and all of these symptoms are thought to be mediated through H_3 receptors and histaminergic pathways located throughout the CNS. DM2 is not as common as DM1 with an estimated prevalence of between 3,000 and 29,000 patients in the United States. The underlying cause of DM2 is a mutation in the CBNP gene on chromosome 3. Patients with DM1 and DM2 share similar phenotypes but disease onset is later in patients with DM2 and symptoms tend to be milder. There are currently no FDA-approved treatments for patients with DM, which represents a significant unmet medical need.

The therapeutic application of pitolisant may provide benefits across the key symptoms of EDS and fatigue which are often among the chief complaints of patients with DM. In a survey of 451 DM1 patients, daytime sleepiness and fatigue were second only to muscle weakness in symptom prevalence and impact. Our clinical program is designed to demonstrate effect on measures of EDS and fatigue, as well as assess performance related to cognitive function, such as attention, vigilance and working memory. Progress to date includes working with key opinion leaders to develop the scientific rationale for the investigation of pitolisant in patients with DM, development of a Phase 2 clinical protocol, submission of an IND at the end of 2020, the opening of an IND in January 2021, and initiation of a Phase 2 proof-of-concept clinical trial in adult patients with DM1 in June of 2021. We announced topline results for the Phase 2 proof-of-concept clinical trial in the fourth quarter of 2023.

The Phase 2 proof-of-concept clinical trial was a randomized, double-blind, placebo-controlled trial to assess the safety and efficacy of pitolisant in adult patients with DM1 ages 18 to 65. It was not powered to demonstrate statistical significance and was designed for signal detection. Thirty patients were enrolled at 20 sites across the United States and Canada. Patients were randomized to low-dose pitolisant, high-dose pitolisant, or placebo in a 1:1:1 treatment ratio and titrated over three weeks up to their randomized dose, followed by eight weeks of stable dosing. The primary trial objective was to assess for improvement in EDS as measured by the Daytime Sleepiness Scale (DSS). Secondary endpoints include assessments of fatigue as well as specific measures of cognitive function using validated computer-based assessments. Clinician and patient global impression of disease severity using the CGI-S and PGI-S, respectively, were measured as well as disease-specific patient assessments of overall disease burden. Plasma samples were collected to generate pharmacokinetic data and a PK/PD analysis were performed. Patients who completed the trial are eligible to participate in an open-label extension phase to assess the long-term safety and effectiveness of pitolisant in adult patients with DM1.

Topline study results include:

- Clinically meaningful improvements were demonstrated in EDS as measured by DSS, ESS, and Clinical Global Impression of Severity (CGI-S) of EDS.
    - On DSS, the mean change from baseline to end of double-blind period was -2.5 and -1.0 for the higher dose and lower dose pitolisant treatment groups, respectively, compared to -0.2 for placebo.
    - On ESS, the mean change from baseline to end of double-blind period was -4.88 for the higher dose pitolisant treatment group compared to -0.10 for placebo.

- On CGI-S, the mean change from baseline to end of double-blind period was -0.9 for the higher dose pitolisant treatment group compared to -0.1 for placebo.

- Clinically meaningful improvements were demonstrated for fatigue as measured by the Fatigue Severity Scale (FSS).

  - On FSS, the mean change from baseline to end of double-blind period was -0.86 and -0.36 for the higher dose and lower dose pitolisant treatment groups, respectively, compared to -0.13 for placebo.

- A clear and consistent dose-response was demonstrated with the higher dose pitolisant group showing a greater response than the lower dose group across the study endpoints.

- Safety and tolerability profile in adult patients with DM1 was consistent with the established safety profile of pitolisant with no new safety signals detected and no serious adverse events reported.

These results represent the initial topline data. The full dataset from this trial will include findings from other secondary outcomes, including the Myotonic Dystrophy Health Index (MDHI), cognitive functions, and the safety and effectiveness of pitolisant from the ongoing Open-Label Extension (OLE) Phase. We are currently evaluating the full data set and determining next steps.

Label Expansion of WAKIX in Narcolepsy

Pediatric Narcolepsy

Approximately 5% of diagnosed narcolepsy patients (approximately 3,600 patients in the U.S.) are under the age of 18 years. Symptoms often have a more profound effect in children, resulting in reduced function and greater psychological impact. Until the fourth quarter of 2018, no treatments were approved for pediatric patients with narcolepsy, at which time Xyrem received an expanded indication for the treatment of cataplexy or EDS in patients seven years of age or older with narcolepsy. Xywav (low sodium formulation of Xyrem) received the same indication in July 2020.

We are working with our partner Bioprojet in gaining a pediatric indication for both EDS and cataplexy. On January 26, 2023, Bioprojet received a positive opinion from the EMA's CHMP and in March 2023, the EMA granted approval for the marketing authorization of WAKIX for the treatment of narcolepsy in children 6 and older. Based on the data from the positive Phase 3 trial conducted by Bioprojet, we submitted an sNDA for pediatric narcolepsy in December 2023. On February 21, 2024, we announced that the FDA has granted priority review of our pediatric narcolepsy sNDA and has set a PDUFA, or target action date, of June 21, 2024.

Pediatric Exclusivity

We remain committed to obtaining pediatric exclusivity for WAKIX. Our recent efforts regarding pediatric exclusivity include the submission of the pediatric sNDA and the planned initiation of a Phase 3 study in PWS in the first quarter of 2024.

Expand Pitolisant Franchise Potentially up to 2040 and Beyond

In July 2022, we entered into the 2022 LCA with Bioprojet whereby we obtained exclusive rights to manufacture, use and commercialize one or more new products based on pitolisant in the United States and Latin America, with the potential to add additional indications and formulations upon the agreement of both parties (see "Strategic Agreements" below). The 2022 LCA will expand our opportunity in narcolepsy, and potentially other indications mutually agreed upon by the parties.

We have made progress in the development of two formulations, NG1 and NG2. Both formulations entered clinical studies in the fourth quarter of 2023 and we anticipate data in the first half of 2024.

Expansion and Diversification of our Product Portfolio

Zynerba

On October 10, 2023, we completed a tender offer to acquire all of the outstanding shares of common stock of Zynerba, a clinical-stage pharmaceutical company focused on innovative pharmaceutically produced transdermal cannabidiol therapies for orphan neuropsychiatric disorders, including FXS and 22q.

Zynerba's lead candidate, ZYN002, is the first-and-only pharmaceutically manufactured synthetic cannabidiol. It is a non-euphoric cannabidiol formulated as a patent-protected permeation-enhanced gel for transdermal delivery through the skin and into the circulatory system. The product is manufactured through a synthetic process in a cGMP facility and is not extracted from the cannabis plant. ZYN002 does not contain THC, the compound that causes the euphoric effect of cannabis, and has the potential to be a nonscheduled product if approved.

Cannabidiol, the active ingredient in ZYN002, has been granted orphan drug designation by the FDA and the EMA for the treatment of FXS and for the treatment of 22q deletion syndrome ("22q"). Additionally, ZYN002 has received FDA Fast Track designation for the treatment of behavioral symptoms in patients with FXS. ZYN002 has the potential to serve 80,000 U.S. patients who are diagnosed with FXS and another 80,000 diagnosed with 22q.

FXS is a rare genetic disorder that is the leading known cause of both inherited intellectual disability and autism spectrum disorder, affecting 1 in 3,600 to 4,000 males and 1 in 4,000 to 6,000 females. The disorder negatively affects synaptic function, plasticity and neuronal connections, and results in a spectrum of intellectual disabilities and behavioral symptoms, such as social avoidance and irritability. There are approximately 80,000 people in the United States and approximately 121,000 people in the European Union and UK living with FXS. There is a significant unmet medical need in patients living with FXS as there are currently no FDA approved treatments for this disorder.

FXS is caused by a mutation in FMR1, a gene which modulates a number of systems, including the endocannabinoid system, and most critically, codes for a protein called FMRP. The FMR1 mutation manifests as multiple repeats of a DNA segment, known as the CGG triplet repeat, resulting in deficiency or lack of FMRP. FMRP helps regulate the production of other proteins and plays a role in the development of synapses, which are critical for relaying nerve impulses, and in regulating synaptic plasticity. In people with full mutation of the FMR1 gene, the CGG segment is repeated more than 200 times, and in most cases causes the gene to not function. Methylation of the FMR1 gene also plays a role in determining functionality of the gene. In approximately 60% of patients with FXS, who have complete methylation of the FMR1 gene, no FMRP is produced, resulting in dysregulation of the systems modulated by FMRP.

Based on the data from the Phase 2 CONNECT study, the Phase 3 registration study, RECONNECT, was designed to evaluate the safety and efficacy of ZYN002 in FXS. This study is currently ongoing and we are evaluating the timelines for the completion of the study.

22q is a disorder caused by a small missing piece of the 22nd chromosome. The deletion occurs near the middle of the chromosome at a location designated q11.2. It is considered a mid-line condition, with physical symptoms including characteristic palate abnormalities, heart defects, immune dysfunction, and esophageal / GI issues, as well as debilitating neuropsychiatric and behavioral symptoms, including anxiety, social withdrawal, ADHD, cognitive impairment and autism spectrum disorder. It is estimated that 22q occurs in one in 4,000 live births, suggesting that there are approximately 80,000 people living with 22q in the United States and 129,000 people living with 22q in the European Union and the UK. Patients with 22q deletion syndrome are managed by multidisciplinary care providers, and there are currently no FDA approved treatments for this disorder.

An open-label phase 2 proof-of-concept study showed positive signals for ZYN002 in the treatment of behavioral symptoms associated with 22q. We are currently evaluating the data and are planning further development of ZYN002 in 22q.

HBS-102

In August 2021, we acquired HBS-102 along with intellectual property and other assets related to the development, manufacture, and commercialization of the Compound from ConSynance Therapeutics, Inc. We acquired full development and commercialization rights globally, but we have provided a grant-back license to ConSynance for the development and commercialization of the Compound in Greater China. HBS-102 is a Melanin-concentrating hormone ("MCH") receptor 1 (MCHR1) antagonist and a potential first-in-class molecule with a novel mechanism of action. MCH neurons are located in the hypothalamus and are involved in the regulation of feeding behavior, energy metabolism and control of REM sleep, among other centrally mediated functions. We are currently conducting a preclinical proof-of-concept study to assess the impact of HBS-102 on hyperphagia, weight gain, and other metabolic parameters in a knockout mouse model of PWS and are also conducting a thirteen-week toxicology study. We anticipate data from both studies in the first half of 2024.

Competition

Our industry is highly competitive and subject to rapid and significant change as research provides a deeper understanding of rare neurological disorders, including narcolepsy, and as new therapies are developed. We face potential competition from multiple sources, including large pharmaceutical, biotechnology and specialty pharmaceutical companies. The key competitive factors affecting the success of WAKIX, and any other product candidates that we develop, if approved, are likely to be efficacy, safety, convenience, price, the level of generic competition and the availability of reimbursement from government and other third-party payors.

WAKIX competes with currently FDA-approved products for the treatment of EDS or cataplexy in adult patients with narcolepsy, all of which are controlled substances. Jazz Pharmaceuticals' Xyrem (sodium oxybate) is an FDA-approved product for the treatment of cataplexy or EDS in patients 7 years of age and older with narcolepsy. Xyrem is a Schedule III controlled substance available only through a restricted access REMS program. Xywav, a lower sodium formulation of Xyrem, was approved in July 2020, (became commercially available in November 2020), and is also a Schedule III controlled substance subject to the same restricted access REMS program as Xyrem. Provigil and Nuvigil, which are Schedule IV WPAs, and stimulants such as methylphenidate and amphetamine (both Schedule II controlled substances), are approved for the treatment of EDS in narcolepsy. Anti-depressants and certain other agents are sometimes used off-label for the treatment of cataplexy in narcolepsy. Axsome's Sunosi (solriamfetol) was approved by the FDA in March 2019 and launched in July 2019. Sunosi (solriamfetol) is a Schedule IV controlled substance and is indicated to improve wakefulness in adult patients with EDS associated with narcolepsy or obstructive sleep apnea. It is not indicated for cataplexy in patients with narcolepsy. Additionally, Avadel Pharmaceuticals launched a once nightly formulation version of sodium oxybate in June 2023. As of January 2023, Xyrem is now available as generic sodium oxybate – one version is marketed by Hikma Pharmaceuticals through their agreement with Jazz Pharmaceuticals (launched in January 2023), and the other version is marketed by Amneal Pharmaceuticals through their agreement with Jazz Pharmaceuticals (launched in July 2023). Other potential future competitive products are currently in development, including Axsome Therapeutics' AXS-12 (reboxetine) product candidate, Suven Life Sciences' SUVN-G3031 H3 inverse agonist product candidate, NLS Pharmaceutics mazindol ER product candidate and orexin 2 receptor agonist product candidates from Takeda, Jazz Pharmaceuticals/Sumitomo Pharma and Alkermes among others.

We believe WAKIX offers a meaningfully differentiated product profile that is competitive with each of the products listed above, some of which are only approved for EDS while others (Xyrem, Xywav, Lumryz and generic sodium oxybate) are approved for the treatment of both EDS or cataplexy in patients with narcolepsy. It should be noted that WAKIX has not been compared with these products in head-to-head clinical trials, but its non-scheduled status represents a distinct competitive advantage relative to those same products. Additionally, WAKIX is priced lower than Xyrem, Xywav, Lumryz and generic sodium oxybate, which we believe is a competitive advantage for WAKIX and may contribute to third-party payor preferences for WAKIX relative to each version of sodium oxybate. Conversely, WAKIX is priced higher than other competitors such as Provigil (modafinil), Nuvigil, Sunosi (armodafinil) and certain generic competitors, such as methylphenidate and amphetamine, which may contribute to third-party payor preferences for those lower-priced treatment options relative to WAKIX.

Manufacturing, Supply and Distribution

We sell WAKIX to, a limited number of specialty distributors, that, in turn, distribute WAKIX to patients. We do not currently own or operate facilities for product manufacturing, storage and distribution, or testing, but we have contracted directly with third parties for each of these functions.

Manufacturing is subject to extensive regulation that imposes various procedural and documentation requirements that govern record keeping, manufacturing processes and controls, personnel, quality control and quality assurance, and more. Our systems and our contractors are required to be in compliance with these regulations, and compliance is assessed regularly through monitoring of performance and a formal audit program.

Our current supply chain for WAKIX involves several manufacturers that specialize in specific operations of the manufacturing process, specifically, intermediate and starting material manufacturing, drug substance manufacturing, and drug product manufacturing, labeling and secondary packaging, and distribution services:

- Interor S.A. manufactures our BF4 and BF6 intermediate and starting material used in the active pharmaceutical ingredient ("API").
- Corden Pharma Chenôve SAS, a full-service contract development and manufacturing organization ("CDMO") manufactures our API.
- Patheon UK Limited, a CDMO owned by Thermo Fisher Scientific Inc., manufactures our finished product tablets and fills them into unlabeled bottles.
- Pharma Packaging Solutions, LLC dba Tjoapack, LLC, handles our labeling and secondary packaging.
- Cardinal Health, Inc. is our third-party logistics provider.
- Inmar Rx Solutions, Inc., an advanced technology and data analytics company, specializes in reverse distribution of our product and manages our pharmaceutical returns and product recall, if needed.

Customers and Suppliers

For the year ended December 31, 2023, three customers accounted for 100% of gross product revenue; Caremark LLC accounted for 37% of gross product revenue; PANTHERx Specialty Pharmacy LLC accounted for 31% of gross product revenue; and Accredo Health Group, Inc. accounted for 32% of gross product revenue. We also depend on a single source supplier for our product, product candidates and active pharmaceutical ingredient.

Strategic Agreements

License and Commercialization Agreements with Bioprojet

In July 2017, we and Bioprojet entered into the Bioprojet License Agreement (the "2017 LCA"). Bioprojet granted to us an exclusive license to commercialize, in the United States and its territories, commonwealths, and protectorates, including Puerto Rico, a product containing pitolisant currently known as WAKIX for narcolepsy, obstructive sleep apnea, idiopathic hypersomnia, Parkinson's disease, and any other indication agreed upon by the parties which currently include PWS and DM ("the field"), as well as rights to related patent rights, know-how, trademarks, trade dress, regulatory filings and approvals (the "Bioprojet Assets"). Bioprojet also granted us a co-exclusive (with Bioprojet) license to Bioprojet Assets to clinically develop and register the pitolisant product in the field in the United States. Bioprojet retains the right to manufacture the product in the United States, and to develop outside the United States and commercialize other products that contain pitolisant as an active ingredient anywhere in the world. Bioprojet also granted us an exclusive license to use certain trademarks and trade names in connection with the commercialization of the product under the Bioprojet License Agreement. Under the 2017 LCA, we cannot, directly or indirectly, develop, market, sell, promote or file an NDA with respect to any product that would compete with pitolisant in the field.

We paid Bioprojet an initial license fee of $150.0 million, a milestone payment of $50.0 million upon FDA acceptance of the NDA in February 2019, and a milestone payment of $75.0 million plus an additional $2.0 million fee for approval of the NDA in November 2019. In October 2020, we paid $2.0 million to Bioprojet to extend the Cataplexy Milestone Payment due date to within 90 days. In January 2021, we made the $100.0 million Cataplexy Milestone Payment in full to Bioprojet. The final milestone payment of $40.0 million was paid to Bioprojet in March 2022 upon the attainment of aggregate net sales of WAKIX in the United States of $500.0 million subsequent to the date of NDA approval by the FDA. Pursuant to the 2017 LCA, we agreed to pay royalties on the net sales of the product at tiered royalty rates of 13% to 24% based on annual

total net sales during the period commencing on first commercial sale of the product and ending on the latest of 10 years from first commercial sale of the product, expiration of all regulatory exclusivity, or expiration of the last Bioprojet patent covering the product. Such royalty payments are subject to reductions based on royalties paid to any third party in order for us to commercialize the product. We also agreed to pay royalties on net sales of the product at a rate of 3% in consideration for a trademark license based on net sales for 20 years after first commercial sale of the product. We further agreed to pay minimum royalties during the third through tenth year of the Bioprojet License Agreement if the product is approved for narcolepsy to the extent such minimum royalties exceed the royalties payable as described above, which minimum amounts were calculated based on sales materially below our sales forecast.

The 2017 LCA will continue until the expiration of the obligation to pay royalties with respect to the product. We and Bioprojet may each terminate the 2017 LCA for a material breach by the other party that remains uncured for 90 days. Bioprojet may terminate the 2017 LCA in its entirety if we or our sublicensees challenge the licensed patents. In addition, we and Bioprojet have the right to terminate the 2017 LCA upon the other party's insolvency.

In July 2022, we entered into the 2022 LCA with Bioprojet whereby we obtained exclusive rights to manufacture, use and commercialize one or more new products based on pitolisant in the United States and Latin America, with the potential to add additional indications and formulations upon the agreement of both parties. We paid an initial, non-refundable $30.0 million licensing fee in October 2022 and additional payments of up to $155.0 million are potentially due under the 2022 LCA upon the achievement of certain development and sales-based milestones. In addition, certain payments will become due upon the achievement of development milestones for new indications and formulations as agreed upon by both parties. The 2022 LCA also requires a fixed trademark royalty and a tiered royalty based on net sales upon commercialization, payable to Bioprojet on a quarterly basis for 10 years from the first commercial sale of each new product, the expiration of all regulatory exclusivity, or the expiration of the last Bioprojet patent covering the products. The $30.0 million licensing fee was recorded in research and development expenses within the consolidated statement of operations and comprehensive income (loss) for the year ended December 31, 2022.

The 2022 LCA will continue until the expiration of the obligation to pay royalties with respect to each new product. Either party may terminate the 2022 LCA for a material breach by the other party that remains uncured for 90 days. Bioprojet may terminate the 2022 LCA in its entirety if we or our sublicensees challenge the licensed patents. In addition, we and Bioprojet have the right to terminate the 2022 LCA upon the other party's insolvency.

Intellectual Property

Intellectual property, including patents, trade secrets, trademarks and copyrights, is important to our business. Our commercial success depends in part on our ability to obtain and maintain proprietary intellectual property protection for our WAKIX product and potential future pitolisant-based products, as well as for future product candidates and novel discoveries, product development technologies, and know-how. Our commercial success also depends in part on our ability to operate without infringing on the proprietary rights of others and to prevent others from infringing our proprietary rights. Our policy is to develop and maintain protection of our proprietary position by, among other methods, licensing or filing U.S. and foreign patents and applications relating to our technology, inventions, and improvements that are important to the development and implementation of our business.

Our WAKIX patent portfolio is comprised of three U.S. patents exclusively licensed to us from Bioprojet under the 2017 LCA. One U.S. patent, No. 8,207,197, which has claims directed to a polymorph, i.e., a specific crystalline form, of pitolisant and, methods for preparing that polymorph of pitolisant, is expected to expire in March 2030 based on a granted request for patent term extension, but without taking into consideration any possible pediatric exclusivity. A second U.S. patent, No. 8,486,947, has claims directed to methods of treating excessive daytime sleepiness by administering pitolisant, which is expected to expire in September 2029 without taking into consideration any possible patent term extension.

Our HBS-102 patent portfolio comprises three patent families. The first patent family includes U.S. patent No. 8,637,501, which has claims to certain melanin-concentrating hormone (MCH-1) receptor antagonists as compositions of matter, as well as methods of making the antagonists, and methods of use for treating obesity, anxiety, and depression, and processes for preparing the compounds, which is expected to expire in April 2031 without taking into consideration any possible patent term extension. A second patent family comprises three issued U.S. patents (Nos. 8,716,308; 9,296,743; and 9,650,378), and issued patents in Australia, Brazil, Canada, China, Germany, Spain, France, the United Kingdom, Israel, Italy, Japan, Korea, Mexico, and New Zealand. The patents have claims directed to certain human MCH-1 receptor-selective antagonists as compositions of matter, and are expected to expire in June 2031 (the '308 patent) and January

2029 (the ‘743 and ‘378 patents, as well as the international patents). The third patent family comprises a pending PCT application (WO2021/142395) and has claims directed to therapeutic combinations or formulations of drugs comprising triple monoamine reuptake inhibitors, MCH receptor 1 (MCHR1) antagonists, and diazoxide, and methods of treating or preventing hyperphagia, moderate to severe binge eating disorder, bulimia nervosa, and management of obesity.

The term of individual patents in our portfolio depends upon the legal term of patents in the countries in which they are obtained. In the United States, the patent term is 20 years from the earliest date of filing a non-provisional patent application. The term of a U.S. patent may be eligible for patent term adjustment, which permits patent term restoration as compensation for delays incurred at the U.S. Patent and Trademark Office (the “USPTO”) during the patent prosecution process. In addition, for patents that cover an FDA-approved drug, the Drug Price Competition and Patent Term Restoration Act of 1984 (the “Hatch-Waxman Act”) permits a patent term extension of up to five years beyond the expiration of the patent. While the length of the patent term extension is related to the length of time the drug is under regulatory review, patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, and only one patent per approved drug may be extended under the Hatch-Waxman Act.

The patent positions of companies like ours are generally uncertain and involve complex legal and factual questions. Changes in either the patent laws or their interpretation in the United States may diminish our ability to protect our technology or product candidates and could affect the value of such intellectual property. In particular, our ability to stop third parties from making, using, selling, offering to sell or importing products that infringe our intellectual property will depend in part on our success in enforcing patent claims that cover our technology, inventions and improvements. We cannot guarantee that patents will be granted with respect to any patent applications we may file in the future, nor can we be sure that any patents that may be granted to us in the future will be commercially useful in protecting our products, the methods of use or manufacture of those products. Moreover, issued patents do not guarantee the right to practice our technology in relation to the commercialization of our products. Issued patents only allow us to block potential competitors from practicing the claimed inventions of the issued patents.

Further, patents and other intellectual property rights in the pharmaceutical and biotechnology space are evolving and involve many risks and uncertainties. For example, third parties may have blocking patents that could be used to prevent us from commercializing our product candidates and practicing our proprietary technology, and our issued patents may be challenged, invalidated or circumvented, which could limit our ability to stop competitors from marketing related products or could limit the term of patent protection that otherwise may exist for our product candidates. In addition, the scope of the rights granted under any issued patents may not provide us with protection or competitive advantages against competitors with similar technology. Furthermore, our competitors may independently develop similar technologies that are outside the scope of the rights granted under any issued patents. For these reasons, we may face competition with respect to our product candidates. Moreover, because of the extensive time required for development, testing and regulatory review of a potential product, it is possible that, before any particular product candidate can be commercialized, any patent protection for such product may expire or remain in force for only a short period following commercialization, thereby reducing the commercial advantage the patent provides.

We and/or our licensor also rely on protections under trade secret laws, and seek to protect and maintain the confidentiality of proprietary information to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection. Our trade secrets include, for example, certain program specific synthesis, formulations, patient selection strategies and certain aspects of our research. It is our policy to require our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to execute confidentiality agreements upon the commencement of employment or consulting relationships with us, and for employees and consultants to enter into invention assignment agreements with us. These agreements provide that all confidential information developed or made known to the individual during the course of the individual’s relationship with us is to be kept confidential and not disclosed to third parties except in specific circumstances. Where applicable, the agreements provide that all inventions to which the individual contributed as an inventor shall be assigned to us, and as such, will become our property. There can be no assurance, however, that these agreements will provide meaningful protection or adequate remedies for our trade secrets in the event of unauthorized use or disclosure of such information.

Further, we have in-licensed from Bioprojet the registered trademark product name “WAKIX” in the United States. We also have registered trademark protection in the United States for “KNOW NARCOLEPSY,” “REM AT THE WRONG TIME” and “NON-REM AT THE WRONG TIME,” as well as our brand and logo “HB,” “HB HARMONY BIOSCIENCES” and “HARMONY BIOSCIENCES.”

Government Regulation

The FDA and comparable regulatory authorities in state and local jurisdictions and in other countries impose substantial and burdensome requirements upon companies involved in the clinical development, manufacture, marketing and distribution of drugs, such as those we are developing. These agencies and other federal, state and local entities regulate, among other things, the research and development, testing, manufacture, quality control, safety, effectiveness, labeling, storage, record keeping, approval, advertising and promotion, distribution, post-approval monitoring and reporting, sampling and export and import of our products and product candidates.

U.S. Drug Development Process

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act (the "FDCA") and its implementing regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant to a variety of administrative or judicial sanctions, such as the FDA's refusal to approve pending NDAs, withdrawal of an approval, imposition of a clinical hold, issuance of warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties.

The process required by the FDA before a drug may be marketed in the United States generally involves the following:

- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's Good Laboratory Practice ("GLP") regulations and other applicable regulations;
- submission to the FDA of an IND which must become effective before human clinical trials may begin;
- approval by an independent institutional review board ("IRB") or ethics committee at each clinical site before each trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with Good Clinical Practice ("GCP") regulations to evaluate the safety and efficacy of the proposed drug product for each indication;
- submission to the FDA of an NDA after completion of all pivotal trials;
- satisfactory completion of an FDA advisory committee review, if applicable;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product is produced to assess compliance with current good manufacturing practice ("cGMP") requirements and to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity;
- satisfactory completion of potential inspection of selected clinical investigation sites to assess compliance with GCPs; and
- FDA review and approval of the NDA to permit commercial marketing of the product for particular indications for use in the United States.

Preclinical Studies

Once a product candidate is identified for development, it enters the preclinical testing stage. Preclinical tests include laboratory evaluation of product chemistry, toxicity and formulation, as well as animal studies. An IND sponsor must submit the results of the preclinical tests, together with manufacturing information and analytical data, among other things, to the FDA as part of an IND. An IND is a request for authorization from the FDA to administer an investigational drug product to humans. An IND will also include a protocol detailing, among other things, the objectives of the clinical trial, the parameters to be used in monitoring safety, and the effectiveness criteria to be evaluated, if the trial includes an efficacy

evaluation. Some preclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, raises concerns or questions related to one or more proposed clinical trials and places the clinical trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. Clinical holds also may be imposed by the FDA at any time before or during clinical trials due to safety concerns about on-going or proposed clinical trials or non-compliance with specific FDA requirements, and the trials may not begin or continue until the FDA notifies the sponsor that the hold has been lifted. As a result, submission of an IND may not result in the FDA allowing clinical trials to commence.

Clinical Trials

All clinical trials must be conducted under the supervision of one or more qualified investigators in accordance with GCPs, which include, among other things, the requirement that all research subjects provide their informed consent in writing for their participation in any clinical trial. Clinical trials must be conducted under protocols detailing the objectives of the trial, dosing procedures, subject selection and exclusion criteria and the safety and effectiveness criteria to be evaluated. the parameters to be used in monitoring safety, and the effectiveness criteria to be evaluated. Each protocol must be submitted to the FDA as part of the IND, and a separate submission to the existing IND must be made for each successive clinical trial conducted during product development and for any subsequent protocol amendments. While the IND is active, progress reports summarizing the results of the clinical trials and nonclinical studies performed since the last progress report, among other information, must be submitted at least annually to the FDA, and written IND safety reports must be submitted to the FDA and investigators for serious and unexpected serious adverse events, findings from other studies suggesting a significant risk to humans exposed to the same or similar drugs, findings from animal or in vitro testing suggesting a significant risk to humans, and any clinically important increased incidence of a serious suspected adverse reaction compared to that listed in the protocol or investigator brochure.

Furthermore, an independent IRB at each institution participating in the clinical trial must review and approve each protocol before a clinical trial commences at that institution and must also approve the information regarding the trial and the consent form that must be provided to each trial subject or his or her legal representative, monitor the study until completed and otherwise comply with IRB regulations. The FDA or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients. In addition, some clinical trials are overseen by an independent group of qualified experts organized by the sponsor, known as a data safety monitoring board or committee. Depending on its charter, this group may determine whether a trial may move forward at designated check points based on access to certain data from the trial. Information about certain clinical trials must be submitted within specific timeframes to the NIH for public dissemination on their www.clinicaltrials.gov website.clinicaltrials.gov.

Human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- Phase 1: The product candidate is initially introduced into healthy human subjects or patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early indication of its effectiveness.

- Phase 2: The product candidate is administered to a limited patient population with a specified disease or condition to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and appropriate dosage.

- Phase 3: The product candidate is administered to an expanded patient population to further evaluate dosage, to provide substantial evidence of efficacy and to further test for safety, generally at geographically dispersed clinical trial sites, in well-controlled clinical trials to generate enough data to statistically evaluate the efficacy and safety of the product for approval, to establish the overall risk-benefit profile of the product, and to provide adequate information for the labeling of the product.

Post-approval trials, sometimes referred to as Phase 4 studies, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval of an NDA.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the drug and finalize a process for manufacturing the product in commercial quantities in accordance with cGMPs. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the manufacturer must develop methods for testing the identity, strength, quality and purity of the final drug. In addition, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

Marketing Approval

Assuming successful completion of the required clinical testing, the results of the preclinical and clinical studies, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the product for one or more indications. In most cases, the submission of an NDA is subject to a substantial application user fee. Specifically, the FDA conducts a preliminary review of all NDAs within the first 60 days after submission, before accepting them for filing, to determine whether they are sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective for its intended use and whether its manufacturing is cGMP-compliant to assure and preserve the product's identity, strength, quality and purity. Under the PDUFA guidelines that are currently in effect, the FDA has a goal of ten months from the date of "filing" of a standard NDA for a new molecular entity to review and act on the submission. This review typically takes twelve months from the date the NDA is submitted to FDA because the FDA has approximately two months to make a "filing" decision after it the application is submitted.

The FDA also may require submission of a REMS to ensure that the benefits of the drug outweigh its risks. The REMS could include medication guides, physician communication plans, assessment plans, and/or elements to assure safe use, such as restricted distribution methods, patient registries, or other risk minimization tools. The FDA will not approve the NDA without an approved REMS, if required.

The FDA may refer an application for a novel drug to an advisory committee. An advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA may inspect one or more clinical trial sites to assure compliance with GCP requirements.

After evaluating the NDA and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and clinical trial sites, the FDA may issue an approval letter, or, in some cases, a complete response letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. A complete response letter usually describes the specific deficiencies in the NDA identified by the FDA and may require additional clinical data, such as an additional clinical trial or other significant and time-consuming requirements related to clinical trials, nonclinical studies or manufacturing. Even with the resubmission of the application with this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

Even if the FDA approves a product, it may limit the approved indications for use of the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs.

FDA Expedited Development and Review Programs

The FDA has a number of programs intended to expedite the development or review of a marketing application for an investigational drug. For example, the fast-track designation program is intended to expedite or facilitate the process for developing and reviewing product candidates that meet certain criteria. Specifically, investigational drugs are eligible for fast-track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. The sponsor of a fast-track product candidate has opportunities for more frequent interactions with the applicable FDA review team during product development and, once an NDA is submitted, the application may be eligible for priority review. With regard to a fast track product candidate, the FDA may consider for review sections of the NDA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA, the FDA agrees to accept sections of the NDA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the NDA.

A product candidate intended to treat a serious or life-threatening disease or condition may also be eligible for breakthrough therapy designation to expedite its development and review. A product candidate can receive breakthrough therapy designation if preliminary clinical evidence indicates that the product candidate, alone or in combination with one or more other drugs or biologics, may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes all of the fast-track program features, as well as more intensive FDA interaction and guidance beginning as early as Phase 1 and an organizational commitment to expedite the development and review of the product candidate, including involvement of senior managers.

Any product candidate submitted to the FDA for approval, including a product candidate with a fast-track designation or breakthrough designation, may also be eligible for other types of FDA programs intended to expedite development and review, such as priority review. An NDA is eligible for priority review if the product candidate is designed to treat a serious condition, and if approved, would provide a significant improvement in safety or efficacy compared to available therapies. The FDA will attempt to direct additional resources to the evaluation of an NDA designated for priority review in an effort to facilitate the review. The FDA endeavors to review applications with priority review designations within six months of the filing date as compared to ten months for review of new molecular entity NDAs under its current PDUFA review goals.

In addition, depending on the design of the applicable clinical trials, a product candidate may be eligible for accelerated approval. Specifically, drugs intended to treat serious or life-threatening diseases or conditions may be eligible for accelerated approval upon a determination that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA generally requires that a sponsor of a drug receiving accelerated approval perform adequate and well-controlled confirmatory clinical trials and may require that such confirmatory trials be underway prior to granting accelerated approval. Drugs receiving accelerated approval may be subject to expedited withdrawal procedures if the sponsor fails to conduct the required confirmatory trials in a timely manner or if such trials fail to verify the predicted clinical benefit. In addition, the FDA currently requires as a condition of accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

Fast-track designation, breakthrough therapy designation, priority review, and accelerated approval do not change the standards for approval but may expedite the development or approval process. Even if a product candidate qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

Post-Approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, and advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications, certain manufacturing changes and additional labeling claims are subject to further FDA review and approval. There also are continuing, annual program user fee requirements for any marketed products, as well as new application fees for supplemental applications with clinical data.

Drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and certain state agencies and are subject to periodic unannounced inspections by the FDA and these certain state agencies for compliance with cGMPs and other laws and regulations. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP requirements and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance.

Once an approval is granted, the FDA may withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in mandatory revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs;
- mandated modification of promotional materials and labeling and the issuance of corrective information, safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warning or other safety information about the product;
- fines, warning letters, untitled letters, or holds on post-approval clinical trials;
- refusal of the FDA to approve pending NDAs or supplements to approved NDAs, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe legally available products for uses that are not described in the product's labeling and that differ from those tested by us and approved by the FDA. Such off-label uses are common across medical specialties. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer's communications on the subject of off-label use of their products.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act ("PDMA"), which regulates the distribution of drugs and drug samples at the federal level, and sets minimum standards for the registration and regulation of drug distributors by the states. Both the PDMA and state laws limit the distribution of prescription pharmaceutical product samples and impose requirements to ensure accountability in distribution.

Marketing Exclusivity

Market exclusivity provisions under the FDCA can delay the submission or the approval of certain marketing applications. The FDCA provides a five-year period of non-patent data exclusivity within the United States to the first applicant to obtain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not approve or even accept for review an

abbreviated new drug application ("ANDA") or an NDA submitted under Section 505(b)(2) ("505(b)(2) NDA") submitted by another company for another drug based on the same active moiety, regardless of whether the drug is intended for the same indication as the original innovative drug or for another indication, where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement to one of the patents listed with the FDA by the innovator NDA holder.

The FDCA alternatively provides three years of non-patent data exclusivity for an NDA, or supplement to an existing NDA, if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the modification for which the drug received approval on the basis of the new clinical investigations and does not prohibit the FDA from approving ANDAs or 505(b)(2) NDAs for drugs containing the active agent for the original indication or condition of use. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Pediatric exclusivity is another type of marketing exclusivity available in the United States. Pediatric exclusivity provides for an additional six months of marketing exclusivity attached to another period of exclusivity if a sponsor conducts clinical trials in children in response to a written request from the FDA. The issuance of a written request does not require the sponsor to undertake the described clinical trials.

Orphan Drug Designation and Exclusivity

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug intended to treat a rare disease or condition, which is a disease or condition that (i) affects fewer than 200,000 individuals in the United States, or (ii) if it affects more than 200,000 individuals in the United States, there is no reasonable expectation that the cost of developing and making the product available in the United States for the disease or condition will be recovered from sales of the product. Orphan designation must be requested before submitting an NDA. After the FDA grants an Orphan Drug Designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Among other benefits of an Orphan Drug Designation are tax credits for certain research and a waiver of the user fee for the NDA. Orphan designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to a seven-year period of marketing exclusivity during which the FDA may not approve any other applications to market the same therapeutic agent for the same disease or condition, except in limited circumstances, such as a subsequent product's showing of clinical superiority over the product with orphan exclusivity or where the original applicant cannot produce sufficient quantities of product. Competitors, however, may receive approval of different therapeutic agents for the disease or condition for which the orphan product has exclusivity. Orphan exclusivity also could block the approval of a competing product for seven years if a competitor obtains approval of the "same drug," as defined by the FDA, or if the active moiety of the product candidate is determined to be contained within the competitor's product for the same disease or condition. In addition, if an orphan designated product receives marketing approval for a disease or condition broader than what is designated, it may not be entitled to orphan exclusivity.

DEA Regulation

The Controlled Substances Act of 1970 ("CSA") establishes registration, security, recordkeeping, reporting, storage, distribution and other requirements administered by the DEA. The DEA is concerned with the control of handlers of controlled substances, and with the equipment and raw materials used in their manufacture and packaging, in order to prevent loss and diversion into illicit channels of commerce.

The DEA regulates controlled substances as Schedule I, II, III, IV or V substances. Schedule I substances by definition have no established medicinal use and may not be marketed or sold in the United States. A pharmaceutical product may be listed as Schedule II, III, IV or V, with Schedule II substances considered to present the highest risk of abuse and Schedule V substances the lowest relative risk of abuse among such substances. The FDA did not recommend that the DEA schedule WAKIX as a controlled substance, and WAKIX is therefore not scheduled as a controlled substance by the DEA.

Annual registration is required for any facility that manufactures, distributes, dispenses, imports or exports any controlled substance. The registration is specific to the particular location, activity and controlled substance schedule. For example, separate registrations are needed for import and manufacturing, and each registration will specify which schedules of controlled substances are authorized.

The DEA typically inspects a facility to review its security measures prior to issuing a registration. Security requirements vary by controlled substance schedule, with the most stringent requirements applying to Schedule I and Schedule II substances. Required security measures include background checks on employees and physical control of inventory through measures such as cages, surveillance cameras and inventory reconciliations. Records must be maintained for the handling of all controlled substances, and periodic reports made to the DEA, for example distribution reports for Schedule I and II controlled substances, Schedule III substances that are narcotics, and other designated substances. Reports must also be made for thefts or losses of any controlled substance, and to obtain authorization to destroy any controlled substance. In addition, special authorization and notification requirements apply to imports and exports.

In addition, a DEA quota system controls and limits the availability and production of controlled substances in Schedule I or II. Distributions of any Schedule I or II controlled substance must also be accompanied by special order forms, with copies provided to the DEA. The DEA may adjust aggregate production quotas and individual production and procurement quotas from time to time during the year, although the DEA has substantial discretion in whether or not to make such adjustments. To meet its responsibilities, the DEA conducts periodic inspections of registered establishments that handle controlled substances. Individual states also regulate controlled substances.

Other Healthcare Laws

In addition to FDA regulation of pharmaceutical products, pharmaceutical companies are subject to federal healthcare laws and regulations as well as regulation by the states and foreign jurisdictions in which they conduct their business that restrict business practices in the pharmaceutical industry. These laws may impact, among other things, our current and future business operations, including our clinical research activities, and proposed sales, marketing and education programs and constrain the business or financial arrangements and relationships with healthcare providers and other parties through which we market, sell and distribute our products for which we obtain marketing approval. These laws include U.S. federal and state anti-kickback and false claims laws, civil monetary penalties laws, consumer protection and transparency laws as well as similar foreign laws in the jurisdictions outside the U.S., including, without limitation, those laws described below.

The federal Anti-Kickback Statute prohibits, among other things, individuals or entities from knowingly and willfully offering, paying, soliciting or receiving remuneration, directly or indirectly, overtly or covertly, in cash or in kind to induce or in return for purchasing, leasing, ordering or arranging for or recommending the purchase, lease or order of any item or service reimbursable under Medicare, Medicaid or other federal healthcare programs. A person or entity does not need to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation.

The federal civil and criminal false claims laws, including the civil False Claims Act, prohibit, among other things, any individual or entity from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government, or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. Manufacturers can be held liable under the False Claims Act even when they do not submit claims directly to government payors if they are deemed to “cause” the submission of false or fraudulent claims. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act and the civil monetary penalties statute. The False Claims Act also permits a private individual acting as a “whistleblower” to bring actions on behalf of the federal government alleging violations of the False Claims Act and to share in any monetary recovery.

The federal Civil Monetary Penalties Law prohibits, among other things, the offering or transferring of remuneration to a Medicare or Medicaid beneficiary that the person knows or should know is likely to influence the beneficiary’s selection of a particular supplier of Medicare or Medicaid payable items or services. Federal government price reporting laws require manufacturers to calculate and report complex pricing metrics to government programs.

The federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA") created additional federal criminal statutes that prohibit, among other things, knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private third-party payors and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the healthcare fraud statute implemented under HIPAA or specific intent to violate it in order to have committed a violation.

The federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services ("CMS") information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain non-physician practitioners (physician assistants, nurse practitioners, clinical nurse specialists, certified registered nurse anesthetists, anesthesiologist assistants, and certified nurse midwives) and teaching hospitals, and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by physicians and their immediate family members.

Similar state and local laws and regulations may also restrict business practices in the pharmaceutical industry, such as state anti-kickback and false claims laws, which may apply to business practices, including but not limited to, research, distribution, sales and marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, or by patients themselves; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws and regulations that require drug manufacturers to file reports relating to pricing information and marketing expenditures or which require tracking gifts and other remuneration and items of value provided to physicians, other healthcare providers and entities; and state and local laws that require the registration of pharmaceutical sales representatives.

Violation of any of such laws or any other governmental regulations that apply may result in significant criminal, civil and administrative penalties including damages, fines, imprisonment, disgorgement, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, reputational harm, diminished profits and future earnings, disgorgement, exclusion from participation in government healthcare programs and the curtailment or restructuring of our operations. To the extent that any of our products are sold in a foreign country, we may be subject to similar foreign laws and regulations, which may include, for instance, applicable post-marketing requirements, including safety surveillance, anti-fraud and abuse laws, implementation of corporate compliance programs, and reporting of payments or transfers of value to healthcare professionals.

Data Privacy and Security Laws

Pharmaceutical companies may be subject to U.S. federal and state health information privacy, security and data breach notification laws, which may govern the collection, use, disclosure and protection of health-related and other personal information. In the U.S., HIPAA imposes privacy, security and breach reporting obligations with respect to individually identifiable health information upon "covered entities" (health plans, health care clearinghouses and certain health care providers), and their respective business associates, individuals or entities that create, received, maintain or transmit protected health information in connection with providing a service for or on behalf of a covered entity. HIPAA mandates the reporting of certain breaches of health information to the Department of Health and Human Services ("HHS"), affected individuals and if the breach is large enough, the media. Entities that are found to be in violation of HIPAA as the result of a breach of unsecured protected health information ("PHI"), a complaint about privacy practices or an audit by the HHS may be subject to significant civil, criminal and administrative fines and penalties and/or additional reporting and oversight obligations if required to enter into a resolution agreement and corrective action plan with HHS to settle allegations of HIPAA non-compliance. Even when HIPAA does not apply, according to the Federal Trade Commission or the FTC, failing to take appropriate steps to keep consumers' personal information secure constitutes unfair acts or practices in or affecting commerce in violation of Section 5(a) of the Federal Trade Commission Act ("FTCA"). The FTC expects a company's data security measures to be reasonable and appropriate in light of the sensitivity and volume of consumer information it holds, the size and complexity of its business, and the cost of available tools to improve security and reduce vulnerabilities. Individually identifiable health information is considered sensitive data that merits stronger safeguards.

In addition, certain state laws govern the privacy and security of health information in certain circumstances, some of which may be more stringent, broader in scope or offer greater individual rights with respect to PHI than HIPAA, many of which may differ from each other, thus, complicating compliance efforts. Failure to comply with these laws, where applicable, can result in the imposition of significant civil and/or criminal penalties and private litigation. For example, California recently enacted legislation, the California Consumer Privacy Act ("CCPA"), which went into effect January 1, 2020. The CCPA, among other things, creates new data privacy obligations for covered companies and provides new privacy rights to California residents, including the right to opt out of certain disclosures of their information. The CCPA also creates a private right of action with statutory damages for certain data breaches, thereby potentially increasing risks associated with a data breach. Although the law includes limited exceptions, including for "protected health information" maintained by a covered entity or business associate, it may regulate or impact our processing of personal information depending on the context. Further, the California Privacy Rights Act (the "CPRA") recently passed in California. The CPRA will impose additional data protection obligations on covered businesses, including additional consumer rights processes, limitations on data uses, new audit requirements for higher risk data, and opt outs for certain uses of sensitive data. It will also create a new California data protection agency authorized to issue substantive regulations and could result in increased privacy and information security enforcement. The majority of the provisions will go into effect on January 1, 2023, and additional compliance investment and potential business process changes may be required.

In Europe, the General Data Protection Regulation ("GDPR"), went into effect in May 2018 and imposes stringent data protection requirements for controllers and processors of personal data of persons within the European Economic Area ("EEA"). The GDPR applies to any company established in the EEA as well as to those outside the EEA if they collect and use personal data in connection with the offering of goods or services to individuals in the EEA or the monitoring of their behavior. The GDPR, together with national legislation, regulations and guidelines of the EEA member states and the United Kingdom governing the processing of personal data, impose strict obligations and restrictions on the ability to collect, analyze and transfer personal data, including health data from clinical trials and adverse event reporting. In particular, these obligations and restrictions concern the consent of the individuals to whom the personal data relates, the information provided to the individuals, the transfer of personal data out of the EEA or the United Kingdom, security breach notifications, security and confidentiality of the personal data and imposition of substantial potential fines for breaches of the data protection obligations. Companies that must comply with the GDPR face increased compliance obligations and risk, including more robust regulatory enforcement of data protection requirements and potential fines for noncompliance of up to €20 million or 4% of the annual global revenue of the noncompliant company, whichever is greater. Further, from January 1, 2021, companies have to comply with the GDPR and also the United Kingdom GDPR ("UK GDPR") which, together with the amended UK Data Protection Act 2018, retains the GDPR in UK national law. The UK GDPR mirrors the fines under the GDPR, e.g. fines up to the greater of €20 million (£17.5 million) or 4% of global turnover. The relationship between the United Kingdom and the European Union in relation to certain aspects of data protection law remains unclear, and it is unclear how United Kingdom data protection laws and regulations will develop in the medium to longer term, and how data transfers to and from the United Kingdom will be regulated in the long term. The European Commission has adopted an adequacy decision in favor of the United Kingdom, enabling data transfers from EU member states to the United Kingdom without additional safeguards. However, the UK adequacy decision will automatically expire in June 2025 unless the European Commission re-assesses and renews or extends that decision. European data protection authorities may interpret the GDPR, UK GDPR and national laws differently and impose additional requirements, which add to the complexity of processing personal data in or from the EEA or United Kingdom. Guidance on implementation and compliance practices are often updated or otherwise revised.

Coverage and Reimbursement

Sales of any pharmaceutical product depend, in part, on the extent to which such product will be covered by third-party payors, such as federal, state and foreign government healthcare programs, commercial insurance and managed healthcare organizations, and the level of reimbursement for such product by third-party payors. In the United States, no uniform policy exists for coverage and reimbursement for pharmaceutical products among third-party payors. Therefore, decisions regarding the extent of coverage and amount of reimbursement to be provided are made on a plan-by-plan basis. The process for determining whether a third-party payor will provide coverage for a product typically is separate from the process for setting the price of such product or for establishing the reimbursement rate that the payor will pay for the product once coverage is approved. Third-party payors may limit coverage to specific products on an approved list, also known as a formulary, which might not include all of the FDA-approved products for a particular indication, or place products at certain formulary levels that result in lower reimbursement levels and higher cost-sharing obligation imposed on patients. One third-party payor's decision to cover a particular medical product or service does not ensure that other payors will also provide coverage for the medical product or service and the level of coverage and reimbursement can differ significantly from payor

to payor. As a result, the coverage determination process will often require us to provide scientific and clinical support for the use of our products to each payor separately and can be a time-consuming process, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. Additionally, a third-party payor's decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved.

In international markets, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies. For example, the European Union provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. Pharmaceutical products may face competition from lower-priced products in foreign countries that have placed price controls on pharmaceutical products. Furthermore, there can be no assurance that a product will be considered medically reasonable and necessary for a specific indication, that a product will be considered cost-effective by third-party payors, that an adequate level of reimbursement will be established even if coverage is available or that the third-party payors' reimbursement policies will not adversely affect the ability to sell a product profitably.

Healthcare Reform

In the United States and certain foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes to the healthcare system. In March 2010, the ACA was signed into law, which substantially changed the way healthcare is financed by both governmental and private insurers in the United States and significantly affected the pharmaceutical industry. The ACA contained a number of provisions, including those governing enrollment in federal healthcare programs, reimbursement adjustments and fraud and abuse changes. Additionally, the ACA increased the minimum level of Medicaid rebates payable by manufacturers of brand name drugs; required collection of rebates for drugs paid by Medicaid managed care organizations; required manufacturers to participate in a coverage gap discount program, under which they must agree to offer point-of-sale discounts (increased to 70 percent pursuant to the Bipartisan Budget Act of 2018, effective as of January 1, 2019) off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D; imposed a non-deductible annual fee on pharmaceutical manufacturers or importers who sell certain "branded prescription drugs" to specified federal government programs, implemented a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted, or injected expanded the types of entities eligible for the 340B drug discount program; expanded eligibility criteria for Medicaid programs; created a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; and established a Center for Medicare and Medicaid Innovation at CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending.

Since its enactment, there have been judicial, executive and Congressional legislative challenges to certain aspects of the ACA. On June 17, 2021 the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. Thus, the ACA will remain in effect in its current form. Further, prior to the U.S. Supreme Court ruling, President Biden issued an executive order to initiate a special enrollment period from February 15, 2021 through August 15, 2021 for purposes of obtaining health insurance coverage through the ACA marketplace. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA. Other legislative changes have also been proposed and adopted since the ACA was enacted, including aggregate reductions of Medicare payments to providers, which was temporarily suspended from May 1, 2020 through March 31, 2022 under the Coronavirus Aid, Relief and Economic Security Act ("CARES Act"), and further reduced payments to several types of Medicare providers. In March 2021, the American Rescue Plan Act of 2021 was signed into law, which eliminated the statutory Medicaid drug rebate cap for single source and innovator multiple source drugs, beginning January 1, 2024. The rebate was previously capped at 100% of a drug's average manufacturer price.

Moreover, there has recently been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted legislation designed, among other things, to bring more transparency to product pricing, review the relationship between

pricing and manufacturer patient programs and reform government program reimbursement methodologies for pharmaceutical products in an attempt to control drug costs. Individual states in the United States have also become increasingly active in implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures and, in some cases, mechanisms to encourage importation from other countries and bulk purchasing. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine which drugs and suppliers will be included in their healthcare programs. Furthermore, there has been increased interest by third-party payors and governmental authorities in reference pricing systems and publication of discounts and list prices.

The Inflation Reduction Act of 2022, or IRA, which was signed into law on August 16, 2022, included several provisions to reduce drug spending by the federal government and reduce out-of-pocket costs for patients on Medicare. This legislation requires that the federal government negotiate prices for some drugs covered under Medicare Part B and Part D with the highest total spending, beginning in 2026. It also requires manufacturers to pay rebates back to the federal government if the price of their medications for Medicare beneficiaries rises faster than inflation. The legislation also placed a cap on out-of-pocket costs for Medicare Part D enrollees and shifted the cost of supplying these medications to manufacturers and plans. Eligibility for the Medicare Part D Low-Income Subsidy Program was also expanded under the legislation, and it further delayed the implementation of the Trump Administration's drug rebate rule to 2027. The IRA permits the Secretary of the Department of Health and Human Services, or HHS, to implement many of these provisions through guidance, as opposed to regulation, for the initial years. On August 29, 2023, HHS announced the list of the first ten drugs that will be subject to price negotiations, although the drug price negotiation program is currently subject to legal challenges. For that and other reasons, it is currently unclear how the IRA will be effectuated, or the impact of the IRA on our business.

We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our products or additional pricing pressures.

Facilities

Our corporate headquarters are located at 630 W. Germantown Pike Plymouth Meeting, Pennsylvania. In December 2020, we leased additional office space at this same location, which increased our footprint to approximately 35,781 square feet of office space. As of December 31, 2023, 85 of our employees are located at our corporate headquarters. Pursuant to our Right of Use Agreement with Paragon, we also utilize office space at 330 N. Wabash Ave, Suite 3500, Chicago, Illinois 60611, where 8 of our employees are located.

Human Capital Management

As of December 31, 2023, we have 246 full-time employees, 127 who are dedicated to commercial functions, which includes sales, marketing, market access, commercial operations, and insights, and 65 who are dedicated to research and development. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

We believe that our future success largely depends upon our continued ability to attract and retain highly skilled employees. We provide our employees with competitive salaries and bonuses, opportunities for equity ownership, development programs that enable continued learning and growth and a robust employment package that promotes well-being across all aspects of their lives, including health care, retirement planning and paid time off.

We believe that much of our success is rooted in the diversity of our teams and our commitment to inclusion. We value diversity at all levels and focus on extending our diversity and inclusion initiatives across our entire workforce.

Corporate Social Responsibility

Social responsibility has always been integral to our core values. We are committed to doing business with integrity and ethics. We focus on providing our employees safe and healthy working conditions, and actively participate in the communities where we are located. In addition, we support philanthropic organizations, patient-focused associations, local and national charitable organizations, and areas of need across the country by providing monetary donations or supplying

food, medical supplies and other resources. We collaborate with patient advocacy organizations to understand the needs of patients living with rare, neurological disorders including narcolepsy, and are committed to addressing those needs.

Our environmental, social and governance ("ESG") and social impact strategy is directed by our Board and overseen by management and a cross-functional executive team comprised of Legal, Compliance, Human Resources, Corporate Affairs and Investor Relations. Our Board of Directors, through our Nominations and Governance Committee, has oversight over our strategic process. We conducted a materiality assessment, including engaging internal stakeholders and reviewing external standards, to inform the creation of an ESG framework, and set our goals and targets to address industry trends, laws and regulations, and stakeholder expectations. We will regularly evaluate our performance in meeting those goals. We may also engage with external organizations and experts to benchmark performance and identify best practices.

Corporate Information

Our principal executive offices are located at 630 W. Germantown Pike, Plymouth Meeting, PA 19462, and our telephone number is (484) 539-9800. Our internet website is www.harmonybiosciences.com. We routinely make important information available on our website free of charge, including copies of our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act, as soon as reasonably practicable after such reports are electronically filed with, or furnished to, the SEC. We recognize our website as a key channel of distribution to reach public investors and as a means of disclosing material non-public information to comply with our disclosure obligations under Regulation FD. Information contained on our website shall not be deemed incorporated into, or to be part of this Annual Report on Form 10-K, and any website references are not intended to be made through active hyperlinks.

Item 1A. Risk Factors.

Investing in our common stock involves a high degree of risk. You should carefully consider the risks and uncertainties described below and the other information included or incorporated by reference in this Annual Report on Form 10-K before making an investment in our common stock. Our business, financial condition, results of operations, or prospects could be materially and adversely affected if any of these risks occurs, and as a result, the market price of our common stock could decline and you could lose all or part of your investment. This Annual Report on Form 10-K also contains forward-looking statements that involve risks and uncertainties. See "Part I—Cautionary Note Regarding Forward-Looking Statements." Our actual results could differ materially and adversely from those anticipated in these forward-looking statements as a result of certain factors, including those set forth below.

Risks Related to Our Business

We are substantially dependent on the commercial success of our only approved product, WAKIX. If we are unable to maintain or increase sales of WAKIX, our ability to generate revenue and our financial condition will be adversely affected.

Historically, our business has been substantially dependent on WAKIX and our financial results have been significantly influenced by sales of WAKIX, which was approved for the treatment of EDS in adult patients with narcolepsy in August 2019 and for the treatment of cataplexy in adult patients with narcolepsy in October 2020. In addition to the risks discussed elsewhere in this section, our ability to generate revenue from sales of WAKIX depends on a number of factors, including, but not limited to:

- the effectiveness of our sales and marketing strategy and operations, and obtaining market acceptance of WAKIX, including garnering market share from existing and future treatment alternatives;
- maintaining compliance with all regulatory requirements applicable to WAKIX and our commercial activities, including the post-marketing requirements and post-marketing commitments required by the FDA;
- increased pricing pressure from, changes in policies by, or restrictions on reimbursement imposed by, third-party payors, including our ability to maintain adequate coverage and reimbursement for WAKIX;

- the continued acceptability of the safety profile of WAKIX and the occurrence of any unexpected side effects, adverse reactions or misuse, including the potential business impact of withdrawing the product (either voluntarily or as mandated by the FDA), loss of support by the advocacy communities or loss of positive corporate reputation resulting in unfavorable media coverage;

- successfully managing third-party service providers involved in the manufacturing and development of pitolisant;

- successfully completing the development of pitolisant in other indications by demonstrating safety, tolerability and efficacy profiles that are satisfactory to the FDA;

- obtaining regulatory approvals to market pitolisant for other indications;

- complying with the terms of the license agreement with Bioprojet;

- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter;

- maintaining, protecting and expanding the portfolio of intellectual property rights, including patents, trade secrets and knowhow, especially in light of potential competition from generic versions of pitolisant; and

- attracting, hiring and retaining qualified personnel.

In our efforts to market WAKIX for the treatment of EDS in adult patients with narcolepsy and cataplexy in adult patients with narcolepsy, our revenue is dependent, in part, on the size of the markets in the United States, or in other territories where we may seek and obtain regulatory approval, the number of competitors in such markets, the acceptance of the price of WAKIX in those markets and the ability to obtain reimbursement at any price. If the number of our addressable patients is not as large as we estimate or the reasonably accepted population for treatment is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of WAKIX. If we are not able to generate substantial revenue from the sale of WAKIX, we may not remain profitable.

The continued commercial success of WAKIX and any other product candidates we develop will depend on the degree of their market acceptance.

Even with the requisite approvals from the FDA and other regulatory authorities, the continued commercial success of WAKIX for the treatment of EDS in adult patients with narcolepsy and cataplexy in adult patients with narcolepsy, and any other indications and product candidates we may develop, will depend on the degree of their acceptance by physicians, patients, third-party payors and others in the medical community. If WAKIX or any other product candidates we develop do not achieve an adequate level of market acceptance, we may not generate significant product revenue or any profits from operations. The degree of market acceptance of WAKIX or any other product candidates we develop, if approved for commercial sale, will depend on a number of factors, some of which are beyond our control, including:

- the safety and efficacy of the product as demonstrated in clinical trials;

- the perception of physicians, patients, third-party payors and others in the medical community of the relative safety, efficacy, convenience, effect on quality-of-life and cost-effectiveness of the product, compared to those of other available treatments;

- the product's approved labeling, including the description of the product's approved indications, the description of its efficacy, including the endpoints in which it showed an improvement, and the prevalence and severity of any side effects, including any associated limitations or warnings;

- the cost of treatment in relation to alternative treatments, including any similar generic treatments;

- our ability to differentiate WAKIX or other approved products from other treatments in the same space;

- the adoption of WAKIX as a first-line therapy for EDS in adult patients with narcolepsy and cataplexy in adult patients with narcolepsy;

- the prevalence and severity of any side effects, including those that may be discovered following approval and commercialization;

- the willingness of the target patient population to try new treatments and of physicians to prescribe these treatments;

- the strength of marketing and distribution support and timing of market introduction of competitive products;

- the publicity concerning our products or competing products and treatments;

- product liability litigation alleging injuries relating to our products or similar classes of drugs;

- any post-approval study requirements for our products and the results thereof; and

- sufficient third-party insurance coverage and reimbursement.

Our continuing efforts to educate physicians, patients, third-party payors and others in the medical community on the benefits and risks of WAKIX may require significant resources and may never be successful. The adoption of WAKIX could be limited if physicians prescribe it only as a second line therapy. Physicians may opt to prescribe the products of our competitors for a variety of reasons. For example, WAKIX did not demonstrate non-inferiority to modafinil and, as such, physicians and patients may choose modafinil rather than WAKIX. Furthermore, because the clinical response to WAKIX may take several weeks before addressing EDS and cataplexy symptoms, patients and physicians may choose other fast acting, stimulant and wake promoting agents over WAKIX. If WAKIX fails to achieve an adequate level of acceptance by physicians, patients, third-party payors and others in the medical community, we will not be able to generate sufficient revenue to become or remain profitable.

We cannot guarantee that WAKIX or any other product candidates we may seek to develop will ever be commercially successful, and to the extent they are not commercially successful, such product candidates would incur significant expense with no corresponding revenue. Because we expect the sales of WAKIX to generate substantially all of our revenue for the foreseeable future, the failure of WAKIX to find market acceptance would substantially harm our business and could require us to seek additional financing.

The market opportunity for WAKIX or any future product candidate we develop may be smaller than we estimate.

The potential market opportunity for WAKIX and any future product candidate is difficult to precisely estimate. Our estimates of the potential market opportunity for our product candidates include several key assumptions of the current market size and current pricing for commercially available products and are based on industry and market data obtained from industry publications, studies conducted by us, our industry knowledge, third-party research reports and other surveys. While we believe our estimates are reasonable and reliable, they may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of diseases and disorders. The number of patients may turn out to be lower than expected. Additionally, the potentially addressable patient population for WAKIX or any future product candidate we develop may be limited or may not be amenable to treatment with WAKIX or such future product candidate, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business.

We rely on our license agreements with Bioprojet to provide rights to the core intellectual property relating to pitolisant, and any termination or loss of significant rights under the agreement would adversely affect our development and/or commercialization of pitolisant.

We have licensed our core intellectual property relating to pitolisant from Bioprojet. If, for any reason, our license and commercialization agreements with Bioprojet are terminated or we otherwise lose those rights, it would materially adversely affect our business. Pursuant to our license and commercialization agreements, we obtained intellectual property rights in connection with the commercialization of pitolisant in the United States and its territories, commonwealths and

protectorates, including Puerto Rico, which includes an exclusive license to use certain intellectual property owned by Bioprojet related to clinically developing and commercializing the pitolisant product candidate for narcolepsy, obstructive sleep apnea, idiopathic hypersomnia and Parkinson's Disease.

Under the license agreements, Bioprojet is responsible for conducting all preclinical studies and clinical trials necessary for achieving and maintaining regulatory approval in the United States for narcolepsy and cataplexy indications, including all costs and expenses. We are responsible for all other costs associated with other development and regulatory activities, unless Bioprojet otherwise agrees to participate in funding such activities. We must obtain consent from Bioprojet before commencing any clinical trials related to pitolisant. Our ability to pursue indications other than the ones specifically enumerated in the license agreement is also contingent on mutual agreement of Bioprojet and us as to those indications and such agreement may be withheld at Bioprojet's discretion. If Bioprojet denies consent for us to conduct clinical trials or pursue any such other indication for any reason, we will not have the right under our license and commercialization agreement to commercialize our product for such indication. In such event, Bioprojet may pursue commercialization of such indication for itself in our territory, or it may license the right to commercialize such indication in our territory to third parties, including our competitors.

Our license and commercialization agreements also impose on us obligations relating to exclusivity, territorial rights, development, commercialization, funding, payment, diligence, sublicensing, insurance, intellectual property protection and other matters. For example, under the 2017 LCA and 2022 LCA, we cannot, directly or indirectly, develop, market, sell, promote or file an NDA with respect to any product that would compete with pitolisant in the field. If we breach any material obligations, or use the intellectual property licensed to us in an unauthorized manner, we may be required to pay damages to Bioprojet, and Bioprojet may have the right to terminate our license, which would result in us being unable to develop, manufacture and sell pitolisant and would materially adversely affect our business.

We may not be successful in our efforts to identify, in-license or acquire, discover, develop or commercialize additional product candidates, or identify other indications for pitolisant beyond EDS or cataplexy in adult patients with narcolepsy.

Although a substantial amount of our effort is focused on the commercialization of WAKIX for the treatment of EDS in adult patients with narcolepsy and cataplexy in adult patients with narcolepsy, we also may seek to identify, in-license or acquire, discover, develop and commercialize additional product candidates in the rare neurological disorders field, such as our recent acquisition of Zynerba's pipeline, and to identify other indications for pitolisant beyond the treatment of EDS in adult patients with narcolepsy and cataplexy in adult patients with narcolepsy. We cannot assure you that our efforts to do so will be successful. Even if we are successful at in-licensing or acquiring additional product candidates, their requisite development activities may require substantial resources, and we cannot assure you that these development activities will result in regulatory approvals. We also cannot assure you that our efforts to develop and commercialize pitolisant for other indications beyond the treatment of EDS in adult patients with narcolepsy and cataplexy in adult patients with narcolepsy will be successful.

Our business, products or product pricing could be subject to negative publicity, which could have a material adverse effect on our reputation, business, financial position, results of operations, liquidity and cash flows.

In recent years, the pharmaceutical industry has been the subject of public complaints and significant publicity regarding the pricing of pharmaceutical products, including publicity and pressure resulting from prices charged by competitors and peer companies for new products as well as price increases by competitors and peer companies on older products that the public has deemed excessive. We may experience downward pricing pressure on the price of WAKIX and any other future approved products due to social or political pressure to lower the cost of drugs, which could reduce our revenue and future profitability. Orphan drugs in particular have received recent negative publicity for the perceived high prices charged for them by their manufacturers, and as a result orphan drug developers such as us may be negatively impacted by such publicity and any U.S. or other government regulatory response. Due to these factors, we may suffer public criticism and negative publicity in media coverage, by industry trade associations and legislators.

Any of the events or developments described above could result in reputational harm and reduced market acceptance and demand for our products, could harm our ability to market our products in the future, could cause us to incur significant expense, could cause our senior management to be distracted from execution of our business strategy, and could have a material adverse effect on our business, reputation, financial condition, results of operations, liquidity, cash flows and/or share price.

Third-party relationships are important to our business. If we are unable to enter into and maintain strategic collaborations or if these relationships are not successful, our business could be adversely affected.

We have limited product development and distribution capabilities and we do not yet have any product manufacturing capabilities. In addition, we may enter into collaborations for the development and commercialization of certain of our product candidates. If we enter into such collaborations, we will have limited control over the amount and timing of resources that our collaborators will dedicate to the development or commercialization of our product candidates. Our ability to generate revenue from these arrangements will depend on any future collaborators' abilities to successfully perform the functions assigned to them in these arrangements. In addition, any future collaborators may have the right to abandon research or development projects and terminate applicable agreements, including funding obligations, prior to or upon the expiration of the agreed upon terms. Relationships we enter into may pose a number of risks, including the following:

- current or future third parties have, and future third-party collaborators may have, significant discretion in determining the efforts and resources that they will apply;
- third parties may not perform their obligations as expected;
- third parties may not pursue development and commercialization of any product candidates that we decide to develop as drugs and that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical study or trial results, changes in the third parties' strategic focus or available funding, or external factors, such as a strategic transaction that may divert resources or create competing priorities;
- third parties may delay preclinical studies or clinical trials, provide insufficient funding for a preclinical study or clinical trial, stop a preclinical study or clinical trial or abandon one of our product candidates, repeat or conduct clinical studies or new clinical trials or require a new formulation of a product candidate for clinical testing;
- third parties could independently develop, or develop with other third parties, products that compete directly or indirectly with our products and product candidates if the third parties believe that the competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- product candidates discovered in collaboration with us may be viewed by our current or future collaborators as competitive with their own product candidates or products, which may cause such third parties to cease to devote resources to the commercialization of our product candidates;
- third parties may fail to comply with applicable regulatory requirements regarding the development, manufacture, packaging, labeling, holding, distribution and/or marketing of a product candidate or product;
- third parties with marketing and distribution rights to pitolisant or any future product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with third parties, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or terminations of the research, development or commercialization of pitolisant or any future product candidates, might lead to additional responsibilities for us with respect to pitolisant or any future product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- third parties may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- third parties may infringe the intellectual property rights of other third parties, which may expose us to litigation and potential liability;

- if one of our third parties is involved in a business combination, the collaborator might deemphasize or terminate the development or commercialization of any product candidate licensed to it by us; and

- relationships may be terminated by the collaborator, and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

If our relationships do not result in the successful discovery, development and commercialization of products or if a third party terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under any third-party agreements we enter into, our development of pitolisant or any future product candidates could be delayed and we may need additional resources. Additionally, if any third party terminates its agreement with us, we may find it more difficult to attract new collaborators and our perception in the business and financial communities could be adversely affected.

Relationships are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of future collaborators. We face significant competition in seeking appropriate collaborators. Our ability to reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. If we are unable to reach agreements with suitable third parties on a timely basis, on acceptable terms, or at all, we may have to curtail the development of a product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms, or at all. If we fail to enter into relationships or do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our product candidates, bring them to market and generate revenue from sales of drugs or continue to develop our technology, and our business may be materially and adversely affected.

We expect to rely on third parties to conduct our clinical trials for pitolisant and other product candidates that we decide to develop. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or comply with regulatory requirements, we may not be able to obtain regulatory approval of or commercialize any potential product candidates on a timely basis or at all.

We will continue to rely upon third parties, including independent investigators, to conduct preclinical studies or clinical trials under agreements with universities, medical institutions, CROs, strategic partners and others. We expect to have to negotiate budgets and contracts with CROs and study or trial sites, which may result in delays to our development timelines and increased costs.

We will have to rely heavily on third parties over the course of our preclinical studies and clinical trials and, as a result, will have limited control over the clinical investigators and limited visibility into their day-to-day activities, including with respect to their compliance with the approved clinical protocol and regulatory requirements. Nevertheless, we are responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards, and our reliance on third parties does not relieve us of our regulatory responsibilities. We and these third parties are required to comply with GCP requirements for clinical trials, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for product candidates in clinical development.

Regulatory authorities enforce these GCP requirements through periodic inspections of study or trial sponsors, clinical investigators and trial sites. If we or any of these third parties fail to comply with applicable GCP requirements, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to suspend or terminate these clinical trials or perform additional clinical trials before approving our marketing applications. We cannot be certain that, upon inspection, such regulatory authorities will determine that any of our clinical trials comply with the GCP or other applicable requirements. In addition, our clinical trials must be conducted with drug products produced under cGMP requirements and may require a large number of patients. Our failure or any failure by these third parties to comply with these regulations, which would delay the regulatory approval or

commercialization process. Moreover, our business may be implicated if any of these third parties violates federal or state laws or regulations including fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

Any parties conducting our future clinical trials, if any, generally will not be our employees and, except for remedies that may be available to us under our agreements with the third parties conducting such clinical trials, if any, we cannot control whether or not they devote sufficient time and resources to our ongoing preclinical and clinical programs. These third parties may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other product development activities, which could affect their performance on our behalf. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to complete development of, obtain regulatory approval of or successfully commercialize our current and future product candidates. As a result, our financial results and the commercial prospects for our current and future product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed.

If any of our relationships with these third-party CROs or others terminate, we may not be able to enter into contractual and other arrangements with alternative CROs or other third parties in a timely manner to meet projected clinical development deadlines or to do so on commercially reasonable terms. Switching or adding additional CROs involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new CRO begins work. As a result, delays may occur, which can materially affect our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

If we experience delays in meeting or fail to meet the regulatory requirements for commercialization of our current or future potential product candidates, the commercial prospects for our product candidates may be harmed and our ability to generate revenue will be materially impaired.

We rely completely on third parties to manufacture and distribute our supply of WAKIX and our product candidates, including certain sole-source suppliers and manufacturers, and intend to rely on third parties to manufacture and distribute any future product candidates.

We do not currently have, nor do we plan to acquire, the infrastructure or capability to manufacture or distribute commercial quantities of WAKIX or our product candidates. Our ability to commercially supply WAKIX and our ability to obtain adequate supplies of our product candidates for clinical studies depends, in part, on the ability of third-party manufacturers to supply and manufacture the raw materials, API and other important components related to the manufacture of WAKIX and our product candidates. We also rely on third parties to package the finished product. These third-party manufacturers have limited experience manufacturing the raw materials and API for WAKIX and our product candidates to be supplied to patients in the United States. Prior to the approval of WAKIX, we experienced minor issues related to product specifications and other minor delays in supply related to our third-party suppliers and manufacturers. We cannot guarantee that even minor changes in the process will result in products that are safe and, where applicable, effective. If we fail to develop and maintain supply relationships with these third parties, we may be unable to continue to successfully commercialize WAKIX and any product candidates that may receive approval.

We rely and will continue to rely on certain third parties as the sole source of the materials they supply or the finished products they manufacture. For example, we rely on Interor S.A., Corden Pharma Chenôve SAS and Patheon UK Limited to provide intermediate supply ingredients, API and finished products, respectively.

Additionally, we rely on our suppliers and manufacturers to purchase materials from other third parties. Any of our existing suppliers or manufacturers may:

- fail to supply us with product on a timely basis or in the requested amount due to unexpected damage to or destruction of facilities or equipment or otherwise;

- fail to increase manufacturing capacity and produce drug product and components in larger quantities and at higher yields in a timely or cost-effective manner, or at all, to sufficiently meet our commercial needs;

- be unable to meet our production demands due to issues related to their reliance on sole-source suppliers and manufacturers;

- supply us with product that fails to meet regulatory requirements;

- become unavailable through business interruption or financial insolvency;

- lose regulatory status as an approved source;

- be unable or unwilling to (i) honor current supply agreements or (ii) renew current supply agreements when such agreements expire on a timely basis, on acceptable terms or at all; or

- discontinue production or manufacturing of necessary drug substances or products.

In the event of any of the foregoing, if we do not have an alternative supplier or manufacturer in place, we would be required to expend substantial management time and expense to identify, qualify and transfer technical processes to alternative suppliers or manufacturers. Transferring technology to other sites may require additional processes, technologies and validation studies, which are costly, may take considerable amounts of time, may not be successful and, in most cases, require prior review and approval by the FDA. Any need to find and qualify new suppliers or manufacturers could significantly delay production of WAKIX, adversely impact our ability to market WAKIX, adversely impact the conduct of our clinical trials and adversely affect our business. There can be no assurance that replacements would be available to us on a timely basis, on acceptable terms or at all. While we currently maintain a robust supply of raw material inventory and an 18 month supply of finished goods inventory to ensure product availability, any interruption in the supply of a drug substance or other material or in the manufacture of WAKIX or our product candidates could have a material adverse effect on our business, financial condition, operating results and prospects.

Additionally, although we are ultimately responsible for ensuring compliance with regulatory requirements such as cGMPs, we are dependent on our contract suppliers and manufacturers for day-to-day compliance with cGMP for production of both drug substances and finished products. Facilities used by our contract suppliers and manufacturers to produce the drug substances and materials or finished products for commercial sale must undergo inspection and be approved by the FDA and other relevant regulatory authorities for the manufacture of our products. A number of our contract suppliers and manufacturers must comply with cGMP requirements enforced by the FDA through its facilities inspection program and review of submitted technical information. If the safety of WAKIX is compromised due to a failure to adhere to applicable laws or for other reasons, we may not be able to successfully commercialize our product and we may be held liable for injuries sustained as a result. In addition, the manufacturing facilities of certain of our suppliers are located outside of the United States. This may give rise to difficulties in importing our product into the United States or other countries as a result of, among other things, regulatory agency approval requirements, taxes, tariffs, local import requirements such as import duties or inspections, incomplete or inaccurate import documentation or defective packaging.

Because a number of companies compete with us, many of which have greater resources than we do, and because we face rapid changes in science in our industry, we cannot be certain that our products will be accepted in the marketplace or capture market share.

Competition from other biotechnology and pharmaceutical companies is intense and is expected to increase. There may be a number of companies pursuing the development of pharmaceuticals in rare neurological disorders, our area of focus. These companies may be very large, and may have financial, technical, sales and distribution and other resources substantially greater than ours. The greater resources of these competitors may enable them to develop, obtain regulatory approval for or market competing products more quickly or effectively, making it extremely difficult for us to capture a share of the market for our product. We also face competition, and may in the future face additional competition, from manufacturers of generic drugs. Certain U.S. state laws allow for, and in some instances in the absence of specific instructions from the prescribing physician mandate, the dispensing of generic products rather than branded products when a generic version is available.

Generic competition often results in decreases in the prices at which branded products can be sold. The commercial potential of our current products and any future products may be reduced or eliminated if our competitors develop or acquire and commercialize generic or branded products that are safer or more effective, have fewer side effects, are easier to administer or are less expensive than our products. We also face competition from off-label uses of approved drugs. Additionally, the biotechnology and pharmaceutical industries are subject to rapid changes in science, and our competitors may develop and market products with improved therapeutic profiles relative to pitolisant or any future product candidates that would render pitolisant or any future product candidates noncompetitive.

We may need to increase the size and capabilities of our organization based on business need, and we may experience difficulties in managing our growth.

We commenced operations in 2017 and, as of December 31, 2023, had approximately 250 employees. As we advance the development of pitolisant in other indications and commercialize WAKIX as a treatment for EDS in adult patients with narcolepsy and cataplexy in adult patients with narcolepsy, we must continue to grow the size of the organization. Future growth will impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, retaining and motivating additional employees;
- effectively managing our development efforts, including the clinical development and FDA or other regulatory authority review processes for pitolisant or any future product candidates;
- effectively managing any third-party service providers involved in the development and manufacture of pitolisant or any future product candidates; and
- improving our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to successfully develop and commercialize WAKIX or any future product candidates will depend, in part, on our ability to effectively manage any future growth. Our management will have to dedicate a significant amount of its attention to managing these growth activities. In addition, we expect to incur additional costs in hiring, training and retaining such additional personnel.

If we are not able to effectively expand our organization, we may not be able to successfully execute the tasks necessary to further develop and commercialize pitolisant or any future product candidates and, accordingly, may not achieve our research, development and commercialization goals.

Our future success depends on our ability to retain our key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on the principal members of our management and scientific teams. We do not maintain “key person” insurance for any of our executives or other employees. The loss of the services of any of these persons could impede the achievement of our research, development and commercialization objectives.

To encourage valuable employees to remain at our company, in addition to salary and cash incentives, we have provided equity award grants that vest over time. The value to employees of these equity grants that vest over time may be significantly affected by changes in the price of our common stock that are beyond our control, and may at any time be insufficient to retain employees who receive more lucrative offers from other companies. Any of our employees could leave our employment at any time, with or without notice.

Recruiting and retaining qualified operations, finance and accounting, quality and compliance, scientific, clinical, manufacturing and sales and marketing personnel or consultants is also critical to our success. We may not be able to attract and retain these personnel on acceptable terms, given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. If we are unable to attract, retain and motivate qualified and experienced personnel, it could harm our business, results of operations and financial condition. Even if we are successful in attracting and retaining such personnel, competition for such employees may significantly increase our compensation costs and adversely affect our business, results of operations and financial condition.

The loss of the services of any of our executive officers, key employees or consultants could seriously harm our ability to successfully implement our business strategy. Replacing executive officers, key employees or consultants may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel or consultants on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel.

We may hire part-time employees or use consultants. As a result, certain of our employees, officers, directors or consultants may not devote all of their time to our business, and may from time to time serve as employees, officers, directors and consultants of other companies.

Public health pandemics, including the COVID-19 pandemic, may result in disruptions to our commercialization, clinical trials, manufacturing and other business operations, which could have a material adverse effect on our business, financial condition, operating results, cash flows and prospects.

A public health epidemic, including COVID-19, poses the risk that we or our employees, contractors, suppliers, distributors and other partners, as well as physicians treating narcolepsy patients, may be prevented from conducting business and patient-care activities for an indefinite period of time, including due to shutdowns and quarantines that may be requested or mandated by governmental authorities. Beginning in March 2020, we had increased reliance on personnel working from home which may negatively impact productivity or disrupt, delay or otherwise adversely impact our business. In addition, remote working could increase our cyber security risk.

General protective measures put into place at various governmental levels, including quarantines, travel restrictions and business shutdowns, may also negatively affect our operations. For example, the COVID-19 pandemic affected our ability to access HCPs, and caused fewer patients to visit their HCP, resulting in fewer prescriptions being written. Additionally, the effects of COVID-19 disrupted the supply chain and could disrupt the manufacturing or shipment of WAKIX and of drug substance and finished drug product. For example, we may face supply chain and manufacturing limitations or difficulties as resources are shifted toward vaccine manufacturing and distribution. Any delays or interruptions in the manufacture and supply of WAKIX could result in delays for our planned clinical trials, impair our ability to meet demand for new WAKIX prescriptions and impede our clinical trial recruitment, testing, monitoring, data collection and analysis and other related activities.

Any of the foregoing factors could have a material adverse impact on our business, financial condition, operating results, cash flows and prospects. The extent to which COVID-19 or any future pandemics impact our operations and those of our third-party partners will depend on future developments, which are highly uncertain and cannot be predicted with confidence, including the duration of the pandemic, additional or modified government actions, the severity of the pandemic, speed of the spread of a virus, and the actions taken to contain the virus or treat its impact, among others.

We or the third parties upon whom we depend may be adversely affected by natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Any unplanned event, such as flood, fire, explosion, earthquake, extreme weather condition, medical epidemics, power shortage, telecommunication failure or other natural or manmade accidents or incidents that result in us being unable to fully utilize our facilities, the manufacturing facilities of our third-party contract manufacturers or our or their distribution networks, may have a material and adverse effect on our ability to operate our business, particularly on a daily basis, and have significant negative consequences on our financial and operating conditions. Loss of access to these facilities may result in increased costs, or interruptions in the commercialization of WAKIX or our business operations. Natural disasters could further disrupt our operations and have a material and adverse effect on our business, financial condition, results of operations and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as our research facilities, the manufacturing facilities of our third-party contract manufacturers or our or their distribution networks, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible, for us to continue our business for a substantial period of time.

The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business. As part of our risk management

policy, we maintain insurance coverage at levels that we believe are appropriate for our business. However, in the event of an accident or incident at these facilities, we cannot assure our investors that the amounts of insurance will be sufficient to satisfy any damages and losses. If our facilities or the manufacturing facilities of our third-party contract manufacturers are unable to operate because of an accident or incident or for any other reason, even for a short period of time, any or all of our research and development programs may be harmed. Any business interruption may have a material and adverse effect on our business, financial condition, results of operations and prospects.

We depend on our information technology systems, and any failure of these systems could harm our business. Any real or perceived security breaches, loss of data, and other disruptions or incidents could compromise the privacy, security, integrity or confidentiality of sensitive information related to our business or prevent us from accessing critical information and expose us to liability and reputational harm, which could adversely affect our business, results of operations and financial condition.

We collect and maintain data and information that is necessary to conduct our business, and we are increasingly dependent on information technology systems and infrastructure to operate our business, including systems infrastructure operated and maintained by our third-party suppliers or providers. In the ordinary course of our business, we collect, store and transmit large amounts of confidential information, including intellectual property, proprietary business information and personal information. It is critical that we do so in a secure manner to maintain the privacy, security, confidentiality and integrity of such confidential information. We have established physical, electronic and organizational measures to safeguard and secure our systems and facilities to prevent an information compromise, and rely on commercially available systems, software, tools and monitoring to provide security for our information technology systems and the processing, transmission and storage of digital information. We have also outsourced elements of our information technology infrastructure, and as a result, a number of third-party vendors may or could have access to our confidential information. Our internal information technology systems and infrastructure, and those of our current and any future collaborators, contractors and consultants and other third parties on which we rely, are vulnerable to damage or unauthorized access or use resulting from computer viruses, malware, natural disasters, terrorism, war, telecommunication and electrical failures, denial-of-service attacks, cyber- attacks or cyber-intrusions over the Internet, hacking, phishing and other social engineering attacks, attachments to emails, persons inside our organization (including employees or contractors), lost or stolen devices, or persons with access to systems inside our organization.

The risk of a security breach or disruption or data loss, particularly through social engineering attacks, cyber-attacks or cyber intrusion, including by computer hackers, foreign governments and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. In addition, the prevalent use of mobile devices that access confidential information increases the risk of data security breaches, which could lead to the loss of confidential information or other intellectual property. As a result of the COVID-19 pandemic, we may also face increased cybersecurity risks due to our reliance on internet technology and the number of our employees who are working remotely, which may create additional opportunities for cybercriminals to exploit vulnerabilities. Furthermore, because the techniques used to obtain unauthorized access to, or to sabotage, systems change frequently and often are not recognized until launched against a target, we may be unable to anticipate these techniques or implement adequate preventative measures. There can be no assurance that our cybersecurity risk management program and processes, including our policies, controls or procedures, will be fully implemented, complied with or effective in protecting our systems and information.

We may also experience security breaches that may remain undetected for an extended period. The costs to us to mitigate, investigate and respond to potential security incidents, breaches, disruptions, network security problems, bugs, viruses, worms, malicious software programs and security vulnerabilities could be significant, and while we have implemented security measures to protect our data security and information technology systems, our efforts to address these problems may not be successful, and these problems could result in unexpected interruptions, delays, cessation of service and other harm to our business and our competitive position. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our product development programs. For example, the loss of clinical trial data from completed or ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Moreover, if a real or perceived security breach affects our systems (or those of our third-party providers or suppliers) or results in the loss of or accidental, unlawful or unauthorized access to, use of, release of or other processing of personally identifiable information or clinical trial data, our reputation could be materially damaged. In addition, such a breach may require notification to governmental agencies, the media or individuals pursuant to applicable data privacy and security laws. We would also be exposed to a risk of loss, negative publicity, harm to our reputation, governmental investigation and/or enforcement actions, claims or litigation and potential

liability, which could materially adversely affect our business, results of operations and financial condition. The global data protection landscape is rapidly evolving, and we may be affected by or subject to new, amended or existing laws and regulations in the future, including as our operations continue to expand or if we begin to operate in foreign jurisdictions.

Our employees and independent contractors, including principal investigators, consultants, commercial collaborators, service providers and other vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could have an adverse effect on our results of operations.

We are exposed to the risk that our employees and independent contractors, including principal investigators, consultants, any future commercial collaborators, service providers and other vendors may engage in misconduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or other unauthorized activities that violate the laws and regulations of the FDA and other similar regulatory bodies, including those laws that require the reporting of true, complete and accurate information to such regulatory bodies; manufacturing standards; U.S. federal and state healthcare fraud and abuse laws, data privacy and security laws and other similar non-U.S. laws; or laws that require the true, complete and accurate reporting of financial information or data. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs, and other business arrangements. Such misconduct also could involve the improper use or misrepresentation of individually identifiable information, including, without limitation, information obtained in the course of clinical trials, the creation of fraudulent data in our preclinical studies or clinical trials, or illegal misappropriation of product, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter misconduct by employees and other third-parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. In addition, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and financial results, including, without limitation, the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgement, possible exclusion from participation in Medicare, Medicaid and other U.S. federal healthcare programs or healthcare programs in other jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, imprisonment, other sanctions, contractual damages, reputational harm, diminished profits and future earnings and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

A variety of risks associated with operating internationally could materially adversely affect our business.

We have conducted clinical studies in the Czech Republic, Romania, and Poland in the past, and currently conduct studies in Ireland, Australia, and the United Kingdom. We may conduct future clinical studies in other countries as well. Doing business internationally involves a number of risks, including but not limited to:

- multiple, conflicting and changing laws and regulations, such as privacy regulations, tax laws, export and import restrictions, employment laws, regulatory requirements and other governmental approvals, permits and licenses;
- failure by us to obtain and maintain regulatory approvals for the use of our products in various countries;
- additional potentially relevant third-party patent rights;
- complexities and difficulties in obtaining protection and enforcing our intellectual property;
- difficulties in staffing and managing foreign operations;
- complexities associated with managing multiple payor reimbursement regimes, government payors or patient self-pay systems;

- limits in our ability to penetrate international markets;

- global macroeconomic conditions, including an increase in inflation rates or interest rates, labor shortages, supply chain shortages, disruptions and instability in the banking industry and other parts of the financial services sector, or other economic, political, or legal uncertainties or adverse developments;

- financial risks, such as longer payment cycles, difficulty collecting accounts receivable, the impact of local and regional financial crises on demand and payment for our products and exposure to foreign currency exchange rate fluctuations;

- terrorism and/or political instability, unrest and wars, such as the conflicts involving Ukraine and Russia or Israel and its surrounding regions, which could delay or disrupt our business, and if such political unrest escalates or spills over to or otherwise impacts additional regions it could heighten many of the other risk factors included in this Item 1A;

- natural disasters (including as a result of climate change), which could cause significant damage to the infrastructure upon which our business operations rely, and the timing, nature or severity of which we may be unable to prepare for;

- economic instability, outbreak of disease or epidemics such as the COVID-19 pandemic, boycotts, curtailment of trade and other business restrictions;

- certain expenses including, among others, expenses for travel, translation and insurance; and

- regulatory and compliance risks that relate to maintaining accurate information and control over sales and activities that may fall within the purview of the U.S. Foreign Corrupt Practices Act, its books and records provisions, or its anti-bribery provisions.

Any of these factors could significantly harm our future international expansion and operations and, consequently, our results of operations.

***Failure to keep up with evolving laws, regulations, trends and shareholder expectations relating to environmental, social and governance, or ESG, practices or reporting could adversely impact our reputation, share price and access to and cost of capital or otherwise adversely impact our business*.**

Certain institutional investors, investor advocacy groups, investment funds, creditors and other influential financial market participants, as well as governments, regulators, customers, patients, employees and other stakeholders, have become increasingly focused on companies' ESG practices, including the impact of business on the environment and diversity, equity and inclusion matters. Certain organizations also provide ESG ratings, scores and benchmarking studies that assess companies' ESG practices. Although there are no universal standards for such ratings, scores or benchmarking studies, they are used by some investors to inform their investment and voting decisions. It is possible that our future stockholders or organizations that report on, rate or score ESG practices will not be satisfied with our ESG strategy or performance. Unfavorable press about or ratings or assessments of our ESG strategies or practices, regardless of whether or not we comply with applicable legal requirements, may lead to negative investor sentiment toward us, which may hinder the Company's access to capital.

Our reputation could be damaged if we do not, or are perceived not to, meet stakeholder demand with respect to ESG matters, which could adversely affect our business, financial condition, profitability and cash flows. We may be criticized for our lack of ESG initiatives or goals or perceived as not taking sufficient action in connection with any of these matters. In turn, we may take certain actions, including the establishment of ESG-related goals or targets, to improve our ESG profile and/or respond to stakeholder demand; however, such actions may be costly or be subject to numerous conditions that are outside our control, and we cannot guarantee that we will meet these goals or targets or that such actions will have the desired effect even if met.

Additionally, we and/or other parties in our value chain are subject to or are expected to be subject to additional climate and other ESG-related obligations arising from legislation and regulation in the United States, the European Union

and other jurisdictions, including new reporting requirements, even as the availability and quality of the information that may be required to comply with such laws and regulations remains limited. We expect for our compliance costs with these laws and regulations to increase in the future, and any failure, or perceived failure, by us to adhere to such laws and regulations, or meet evolving and varied stakeholder expectations and standards, could harm our business, reputation, financial condition, and operating results.

Risks Related to Our Financial Condition and Capital Requirements

We have a limited operating history and history of commercializing drugs, which may make it difficult for you to evaluate the success of our business to date and assess our future viability.

We commenced operations in 2017, and our operations to date have been largely focused on staffing our company, business planning, raising capital, acquiring the rights to pitolisant, seeking registration in the United States for our product WAKIX, which is approved for the treatment of EDS in adult patients with narcolepsy and cataplexy in adult patients with narcolepsy, the commercialization of WAKIX, manufacturing WAKIX on a commercial scale, and preparing to develop pitolisant for other potential indications. This has included preparing the application for regulatory approval and other activities that were required for us to obtain approval of our NDA, and activities related to commercializing WAKIX. WAKIX is our only drug candidate for which we have obtained regulatory approval. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a longer history of successfully developing and commercializing drugs.

We expect our financial condition and operating results to continue to fluctuate from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. We may encounter unforeseen expenses, difficulties, complications, delays and other known or unknown factors.

We have made and may be required in the future to make significant payments to Bioprojet under our licensing and collaboration agreements for pitolisant.

Under our agreements with Bioprojet, we are subject to significant obligations, including payment obligations upon the achievement of specified milestones and payments based on product sales, as well as other material obligations. Certain of the milestone payments payable by us under these agreements were paid prior to our commercialization of WAKIX.

There can be no assurance that we will have the funds necessary to make such payments in the future, or be able to raise such funds when needed, on terms acceptable to us, or at all. If we fail to comply with our payment obligations, Bioprojet has the right to terminate the license agreement, in which event we would not be able to develop, manufacture or market WAKIX or any other pitolisant-based product candidate. Furthermore, if we are forced to raise additional funds to make such payments, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts.

Our TLA Credit Agreement contains restrictive and financial covenants that may limit our operating flexibility.

Our TLA Credit Agreement (as defined below) contains certain restrictive covenants that either limit our ability to, or require a mandatory prepayment in the event that, we or our subsidiaries engage in new lines of business, incur additional indebtedness or liens, make certain investments, make certain payments, pay cash dividends, merge with other companies or consummate certain changes of control, make certain acquisitions, transfer or dispose of certain assets, liquidate or dissolve, amend certain material agreements, enter into sale and leaseback transactions, enter into various other specified transactions, or change our name, location, or executive office without notice. We, therefore, may not be able to engage in any of the foregoing transactions unless we obtain the consent of the lenders, or in certain cases JP Morgan on their behalf, or prepay the outstanding amount under the TLA Credit Agreement.

Our failure to comply with the covenants or other terms of the TLA Credit Agreement, including as a result of events beyond our control, could result in a default under the TLA Credit Agreement that could materially and adversely affect the ongoing viability of our business.

JPMorgan Chase Bank, N.A. and the other Lenders may elect to accelerate the repayment of all unpaid principal of the Loans, accrued interest and other amounts owed under the TLA Credit Agreement upon consummation of a specified

change of control transaction or the occurrence of certain events of default (as specified in the TLA Credit Agreement), including, among other things:

- our default in a payment obligation under the TLA Credit Agreement;
- our breach of the covenants or other terms of the TLA Credit Agreement;
- our failure to properly maintain the collateral;
- the occurrence of a specified change of control transaction occurring;
- one or more judgments resulting in liability greater than $20.0 million; and
- certain specified insolvency and bankruptcy-related events.

Subject to any applicable cure period set forth in the TLA Credit Agreement, all amounts outstanding with respect to the Loans (principal and accrued interest), as well as any applicable prepayment premiums or interest "make-whole" payments, would become due and payable. Our assets or cash flow may not be sufficient to fully repay our obligations under the Loans if the obligations thereunder are accelerated upon any events of default. Further, if we are unable to repay, refinance or restructure our obligations under the Loans, the Administrative Agent on behalf of the Lenders could proceed to protect and enforce their rights under the TLA Credit Agreement and other loan documents by exercising such remedies (including foreclosure on the assets securing our obligations under the TLA Credit Agreement and the other loan documents) as are available to the Administrative Agent and the Lenders and in respect thereof under applicable law, either by suit in equity or by action at law, or both, whether for specific performance of any covenant or other agreement contained in the TLA Credit Agreement or other loan documents or in aid of the exercise of any power granted in the TLA Credit Agreement or other loan documents. Such repayment could have a material adverse effect on our business, operating results and financial condition.

Our obligations under the TLA Credit Agreement are secured by all of our assets, with certain exceptions. We may not be able to generate sufficient cash flow or sales to meet the financial covenants or pay the principal and interest under the TLA Credit Agreement. Furthermore, our future working capital, borrowings or equity financing could be unavailable to repay or refinance the amounts outstanding under the TLA Credit Agreement. In the event of a liquidation, the lenders under the facility would be repaid all outstanding principal and interest prior to distribution of assets to unsecured creditors, and the holders of our common stock would receive a portion of any liquidation proceeds only if all of our creditors then existing, including the lenders under the TLA Credit Agreement, were first repaid in full.

We have never paid dividends on our common stock, and we do not intend to pay dividends for the foreseeable future. Consequently, any gains from an investment in our common stock will likely depend on whether the price of our common stock increases.

We have never declared or paid any dividends on our common stock and do not intend to pay any dividends in the foreseeable future. We anticipate that we will retain all of our future earnings for use in the operation of our business and for general corporate purposes. Any determination to pay dividends in the future will be at the discretion of our board of directors. Accordingly, investors must rely on sales of their common stock after price appreciation, which may never occur, as the only way to realize any future gains on their investments.

Raising additional funds by issuing securities may cause dilution to existing shareholders, raising additional funds through debt financings may involve restrictive covenants, and raising funds through lending and licensing arrangements may restrict our operations or require us to relinquish proprietary rights to our technologies or product candidates.

To the extent that we raise additional capital by issuing equity securities, our existing shareholders' ownership may experience substantial dilution, and the terms of these securities may include liquidation or other preferences that could adversely affect the rights of a common shareholder. Additionally, any agreements for future debt or preferred equity financings, if available, may involve covenants limiting or restricting our ability to take specific actions, such as incurring

additional debt, making capital expenditures or declaring dividends, which could adversely affect our ability to conduct our business.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates, or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts, or grant rights to develop and market product candidates that we would otherwise develop and market ourselves.

Our ability to utilize our net operating loss carryforwards may be limited.

As of December 31, 2023, we had U.S. federal net operating loss carryforwards of $179.4 million and we had state net operating loss carryforwards of approximately $137.3 million. Utilization of the federal and state net operating loss carryforwards may be subject to a substantial limitation due to federal and state provisions. If we have experienced an ownership change at any time since our incorporation, we may be subject to limitations on our ability to utilize our existing net operating loss carryforwards to offset taxable income. In addition, future changes in our stock ownership, which may be outside of our control, may trigger an ownership change and, consequently, the limitations under Section 382 of the Code. As a result, if or when we earn net taxable income, our ability to use our pre-change net operating loss carryforwards to offset such taxable income may be subject to limitations, which could adversely affect our future cash flows.

Changes in tax laws or regulations could adversely affect our results of operations, business and financial condition.

New tax laws or regulations could be enacted at any time, and existing tax laws or regulations could be interpreted, modified or applied in a manner that is adverse to us or our customers, each of which could adversely affect our results of operations, business and financial condition. Beginning in 2022, the Tax Cuts and Jobs Act of 2017 eliminated the option to deduct research and development expenditures for tax purposes in the year incurred and instead requires taxpayers to capitalize and subsequently amortize such expenditures over five years for research activities conducted in the United States and over 15 years for research activities conducted outside the United States. This tax law change will continue to have an adverse effect on our income tax liability, which could be material. In August 2022, the Inflation Reduction Act of 2022 was enacted, which, among other things, imposes a new 15% alternative minimum tax on the adjusted financial statement income of certain large corporations for tax years beginning after December 31, 2022. Changes in tax laws or regulations could materially increase our tax provision, cash tax liabilities, and effective tax rate, which may adversely affect our results of operations, business and financial condition.

Risks Related to Development, Regulatory Approval and Commercialization

The regulatory approval process of the FDA is costly, lengthy and inherently unpredictable, and our business may be substantially harmed if we experience insufficient results in the course of our clinical development or are ultimately unable to obtain regulatory approval for any of our ongoing development programs in other potential indications.

Although the commercialization of WAKIX is our primary focus, as part of our longer-term growth strategy, we plan to evaluate pitolisant in other indications and develop other product candidates. The research, testing, manufacturing, labeling, approval, selling, import, export, pricing and reimbursement marketing and distribution of drug products are subject to extensive regulation by the FDA and other regulatory agencies in the United States. Although we have obtained regulatory approval for WAKIX in the United States for the treatment of EDS or cataplexy in adult patients with narcolepsy, it is possible that we may not obtain regulatory approval for pitolisant for other indications, or for any other product candidates we may seek to develop in the future.

The FDA or comparable regulatory authorities can delay, limit or deny approval of a drug candidate for many reasons or require us to conduct additional preclinical or clinical testing, including, but not limited to, the following:

- a drug candidate may not be deemed safe or effective, or the clinical and other benefits may be deemed to not outweigh the candidate’s risks;

- regulatory authorities may disagree with the design or execution of our clinical trials plan;
- the population studied in clinical trials may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- regulatory authorities may not find the data from nonclinical and clinical studies and trials sufficient or may disagree with our interpretation of data from nonclinical or clinical studies;
- serious and unexpected drug-related side effects experienced by participants in our clinical trials or by individuals using drugs similar to our product candidates, or other products containing the active ingredient in our product candidates;
- regulatory authorities may disagree with us regarding the formulation, labeling and/or the product specifications of our product candidates;
- clinical trial site inspection(s) by the FDA or other regulatory authorities may result in unacceptable findings that could negatively impact approval of pitolisant;
- regulatory authorities might not accept or deem acceptable a third-party manufacturers' processes or facilities; or
- regulatory authorities may change their approval policies or adopt new regulations.

Prior to obtaining approval to commercialize a drug candidate in the United States, we or our collaborators must demonstrate with substantial evidence from well-controlled clinical trials, and to the satisfaction of the FDA, that such drug candidates are safe and effective for their intended uses. The number of nonclinical and clinical studies and trials that will be required for FDA approval varies depending on the drug candidate, the disease or condition that the drug candidate is designed to address, and the regulations applicable to any particular drug candidate. In addition, data obtained from preclinical trials and clinical trials are susceptible to varying interpretations, and regulatory authorities may not interpret our data as favorably as we do, which may further delay, limit or prevent development efforts, clinical trials or marketing approval. Furthermore, as more competing drug candidates within a particular class of drugs proceed through clinical development to regulatory review and approval, the amount and type of clinical data that may be required by regulatory authorities may increase or change. Even if we believe the preclinical or clinical data for our product candidates are promising, such data may not be sufficient to support approval by the FDA and other regulatory authorities. If pitolisant fails to demonstrate safety and efficacy in clinical trials or does not gain regulatory approval for other indications, our business and results of operations will be materially and adversely harmed. Additionally, if the FDA requires that we conduct additional clinical trials, places limitations on pitolisant in our label, delays approval to market pitolisant or limits the use of pitolisant, our business and results of operations may be harmed.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of one or more of our product candidates.

If we fail to obtain and sustain an adequate level of coverage and reimbursement for WAKIX and other product candidates by third-party payors, sales would be adversely affected.

Successful sales of WAKIX and any other product candidates that may receive regulatory approval depend on the availability of coverage and adequate reimbursement from third-party payors. Patients who are prescribed medications for

the treatment of their conditions generally rely on third- party payors to reimburse all or part of the costs associated with their prescription drugs. There is significant uncertainty related to third-party payor coverage and reimbursement of newly approved drugs. Regulatory approvals, pricing and reimbursement for new drug products vary widely from country to country. In the United States, the principal decisions about reimbursement for new medicines are typically made by CMS, an agency within HHS. CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare. Private payors tend to follow CMS to a substantial degree. Commercial third-party payors, such as private health insurers and health maintenance organizations, also decide which medications they will pay for and establish reimbursement levels, though commercial third-party payors often follow CMS' reimbursement determinations. The availability of coverage and the extent of reimbursement by governmental and private payors is essential for most patients to be able to afford treatments. Sales of WAKIX or other product candidates that we may identify will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, private health coverage insurers and other third-party payors.

Coverage and reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

We cannot be sure that reimbursement will be available for WAKIX and, if coverage and reimbursement are available, what the level of reimbursement will be. Our inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition. If coverage and adequate reimbursement is not available, or is available only to limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

Obtaining coverage and reimbursement approval for a product from a government or other third- party payor can be an expensive and time-consuming process that could require us to provide supporting scientific, clinical and cost effectiveness data for the use of our products to the payor. The industry competition to be included in third-party payors' drug formularies, or lists of medications for which third- party payors provide coverage and reimbursement, often leads to downward pricing pressures on pharmaceutical products. In addition, third-party payors may refuse to include a particular branded drug in their formularies or otherwise restrict patient access through formulary controls or otherwise to a branded drug when a less costly generic equivalent or other alternative is available. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors, and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. In addition, many pharmaceutical manufacturers must calculate and report certain price reporting metrics to the government, such as average manufacture price and best price. Penalties may apply in some cases when such metrics are not submitted accurately and timely. We cannot be sure that reimbursement will be available for any product candidate that we commercialize and, if reimbursement is available, the level of reimbursement.

In addition, there may be significant delays in obtaining such coverage and reimbursement for newly approved products, and coverage may be more limited than the purposes for which the product is approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for coverage and reimbursement does not imply that a product will be

paid for in all cases or at a rate that covers our costs, including research, development, intellectual property, manufacture, sale and distribution expenses.

Interim reimbursement levels for new products, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the product and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost products and may be incorporated into existing payments for other services. Net prices for products may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors, by any future laws limiting drug prices and by any future relaxation of laws that presently restrict imports of product from countries where they may be sold at lower prices than in the United States.

While we have obtained coverage for WAKIX from certain third-party payors, the resulting reimbursement payment rates might not be adequate or may require co-payments that patients find unacceptably high. Patients are unlikely to use WAKIX unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of WAKIX. Therefore, coverage and adequate reimbursement is critical to new product acceptance. Coverage decisions may depend upon clinical and economic standards that disfavor new products when more established or lower cost therapeutic alternatives are already available or subsequently become available. We may suffer loss of corporate reputation due to industry-wide legislative or public scrutiny of our pricing decisions and practices within an increasingly price-sensitive environment.

Despite obtaining formulary approval from certain third-party payors, sometimes with prior authorization or other formulary restrictions and requirements, including documented failure or inadequate response to alternative treatments, we expect to experience pricing pressures in connection with the sale of WAKIX due to the trend toward cost containment, managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. Large public and private payors, managed care organizations, group purchasing organizations and similar organizations are exerting increasing influence on decisions regarding the use of, and reimbursement levels for, particular treatments. Such third-party payors, including Medicare, are questioning the coverage of, and challenging the prices charged for medical products and services, and many third-party payors limit coverage of, or reimbursement for, newly approved health care products. The downward pressure on healthcare costs in general, particularly prescription medicines, medical devices and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the successful commercialization of new products. Further, the adoption and implementation of any future governmental cost containment or other health reform initiative may result in additional downward pressure on the price that we may receive for WAKIX.

These cost-control initiatives could decrease the price we have established for WAKIX, which could result in product revenue being lower than anticipated. The pricing, coverage and reimbursement of WAKIX must be adequate to support a commercial infrastructure. If the price for WAKIX decreases or if governmental and other third-party payors do not provide adequate coverage and reimbursement levels, our revenue, gross margins and prospects for profitability will suffer.

While we have not taken any steps to attain regulatory or patent approvals in any specific markets outside of the United States, we plan to explore obtaining additional licensing rights from Bioprojet to expand into international markets with WAKIX**.** Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe and other countries will likely put pressure on the pricing and usage of medical products. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. Other countries allow companies to fix their own prices for medical products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for WAKIX. Accordingly, in markets outside the United States, the reimbursement for WAKIX may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenue and profits.

WAKIX has been approved by the FDA for the treatment of EDS in adult patients with narcolepsy, and cataplexy in adult patients with narcolepsy. Regulatory approval is limited by the FDA to the specific indication for which approval has been granted and, unless we seek regulatory approval for additional indications, we will be prohibited from marketing pitolisant for other indications. We may be subject to fines, penalties or injunctions if we are determined to have promoted or be promoting the use of pitolisant for unapproved or "off-label" uses, resulting in damage to our reputation and business.

While we received approval for the indications of the treatment of EDS in adult patients with narcolepsy and cataplexy in adult patients with narcolepsy, WAKIX is not indicated to treat any other conditions. We are prohibited from promoting WAKIX for any other indication unless we are granted FDA approval for such indication. The FDA strictly regulates the promotional claims that may be made about prescription products, and WAKIX may not be promoted for uses that are not approved by the FDA as reflected in its approved labeling. If we are not able to obtain FDA approval for any desired future indications for our products and product candidates, our ability to effectively market and sell our products may be reduced and our business may be adversely affected.

While physicians may choose to prescribe products for uses that are not described in the product's labeling and for uses that differ from those tested in clinical trials and approved by the regulatory authorities, we are prohibited from marketing and promoting the products for indications that are not specifically approved by the FDA. These "off-label" uses are common across medical specialties and may constitute an appropriate treatment for some patients in varied circumstances. Regulatory authorities in the United States generally do not restrict or regulate the behavior of physicians in their choice of treatment within the practice of medicine. Regulatory authorities do, however, restrict communications by biotechnology or pharmaceutical companies on off-label use. If the FDA determines that our promotional activities constitute promotion of an off-label use, it could request that we modify our promotional materials and subject us to FDA regulatory or enforcement actions as well as actions by other agencies, including issuance of warning letters or untitled letters, suspension or withdrawal of an approved product from the market, mandatory or voluntary recalls, civil fines, disgorgement of money, operating restrictions, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement, injunctions or criminal prosecution, any of which could significantly harm our business.

WAKIX or any of our future product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, reduce the commercial attractiveness of a prescribing label or result in significant negative consequences following regulatory approval, if approved.

Clinical trials of WAKIX or other product candidates we may develop could reveal a high and unacceptable incidence and severity of undesirable side effects. Undesirable side effects could adversely affect patient enrollment in clinical studies, cause us or regulatory authorities to interrupt, delay or halt clinical studies or result in the delay, denial or withdrawal of regulatory approval by the FDA or other regulatory authorities. Undesirable or adverse side effects also could result in regulatory authorities mandating a more restrictive prescribing label for the product, which, in turn, could limit the market acceptance of the product even if approved for marketing and commercialization.

Drug-related side effects could result in potential product liability claims. We believe our product liability insurance coverage is sufficient in light of our clinical programs; however, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts or maintain coverage at all to protect us against losses due to liability. A successful product liability claim or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations, business and financial condition. In addition, regardless of merit or eventual outcome, product liability claims may result in impairment of our business reputation, significant negative media attention, withdrawal of clinical study participants, costs due to related litigation, distraction of management's attention from our primary business, initiation of investigations by regulators, substantial monetary awards to patients or other claimants, the inability to commercialize our current product candidate or any future product candidate, product recalls, restrictions on labeling, marketing or promotion, decreased demand for our product candidates, if approved for marketing, and loss of revenue.

Additionally, if we or others later identify undesirable side effects caused by WAKIX, either in the post-marketing setting or in clinical trials in other potential indications for which we develop pitolisant, or in clinical trials for other product candidates, a number of potentially significant negative consequences could result, including but not limited to:

- the delay, prevention or withdrawal of approvals by regulatory authorities;

- the requirement to suspend marketing of a product or withdraw it from the marketplace;
- the requirement of additional warnings on the prescribing label or limitations on product access;
- requirements to conduct costly post-marketing studies;
- requirements to change the manner in which a product is administered;
- the requirement of a REMS plan, which could include a medication guide outlining the risks of such side effects for distribution to patients, a communication plan for healthcare providers and/or other elements to assure safe use;
- designation as a controlled substance by the DEA;
- a product may become less competitive
- fines, injunctions or civil and criminal penalties;
- litigation and the potential to be held liable for harm caused to patients; and
- an adverse effect on our reputation.

Any of these events could prevent us from achieving or maintaining market acceptance of pitolisant and could significantly harm our business, results of operations, financial condition and prospects.

If the FDA or comparable foreign regulatory authorities approve generic versions of any of our products that receive marketing approval, or such authorities do not grant our products appropriate periods of exclusivity before approving generic versions of our products, the sales of our products could be adversely affected.

Once an NDA is approved, the product covered thereby becomes a "reference listed drug" in the FDA's publication, "Approved Drug Products with Therapeutic Equivalence Evaluations," commonly known as the Orange Book. Manufacturers may seek approval of generic versions of reference listed drugs through submission of ANDAs in the United States. In support of an ANDA, a generic manufacturer need not conduct clinical trials. Rather, the applicant generally must show that its product has the same active ingredient(s), dosage form, strength, route of administration and conditions of use or labeling as the reference listed drug and that the generic version is bioequivalent to the reference listed drug, meaning it is absorbed in the body at the same rate and to the same extent. Generic products may be significantly less costly to bring to market than the reference listed drug and companies that produce generic products are generally able to offer them at lower prices. Thus, following the introduction of a generic drug, a significant percentage of the sales of any branded product or reference listed drug is typically lost to the generic product.

The FDA may not approve an ANDA for a generic product until any applicable period of non-patent exclusivity for the reference listed drug has expired. The FDCA provides a period of five years of non-patent exclusivity for a new drug containing a new chemical entity ("NCE"). Specifically, in cases where such exclusivity has been granted, an ANDA may not be submitted to the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification that a patent covering the reference listed drug is either invalid or will not be infringed by the generic product, in which case the applicant may submit its application four years following approval of the reference listed drug.

While we have received five years of NCE exclusivity for WAKIX, manufacturers may seek to launch generic products following the expiration of the applicable exclusivity period we obtain, even if we still have patent protection for our product.

Competition that our products may face from generic versions of our products could materially and adversely affect our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on the investments we have made in those product candidates.

We may incur unexpected costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.

To obtain the requisite regulatory approvals to commercialize any of our product candidates, we must demonstrate through extensive preclinical studies and clinical trials that our product candidates are safe and effective in humans. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process and our future clinical trial results may not be successful.

We may experience delays in completing our clinical trials or preclinical studies and initiating or completing additional clinical trials. We may also experience numerous unforeseen events during our clinical trials that could delay or prevent our ability to receive marketing approval or commercialize the product candidates we develop, including:

- we may be unable to generate sufficient preclinical, toxicology, or other in vivo or in vitro data to support the initiation or continuation of clinical trials;
- regulators, IRBs or other reviewing bodies may not authorize us or our investigators to commence a clinical trial, or to conduct or continue a clinical trial at a prospective or specific trial site;
- the FDA or comparable foreign regulatory authorities may not agree as to the design or implementation of our clinical trials;
- we may not reach agreement on acceptable terms with prospective CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- we may be unable to identify, recruit and train suitable clinical investigators;
- the number of subjects or patients required for clinical trials of pitolisant in additional indications or any other product candidate may be larger than we anticipate, enrollment in these clinical trials may be insufficient or slower than we anticipate, and the number of clinical trials being conducted at any given time may be high and result in fewer available patients for any given clinical trial, or patients may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors, including those manufacturing our product candidates or conducting clinical trials on our behalf, may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we may have to amend clinical trial protocols submitted to regulatory authorities or conduct additional studies to reflect changes in regulatory requirements or guidance, which we may be required to resubmit to an IRB and regulatory authorities for re-examination;
- regulators, IRBs or other reviewing bodies may fail to approve or subsequently find fault with the manufacturing processes or facilities of third-party manufacturers with which we enter into agreement for clinical and commercial supplies;
- the supply or quality of pitolisant or any other product candidate or other materials necessary to conduct clinical trials of our product candidates may be insufficient, inadequate or not available at an acceptable cost, or we may experience interruptions in supply; and
- the potential for approval policies or regulations of the FDA or the applicable foreign regulatory agencies to significantly change in a manner rendering our clinical data insufficient for approval.

Regulators, IRBs of the institutions in which clinical trials are being conducted or data monitoring committees may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects,

failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Negative or inconclusive results from our ongoing clinical trials of pitolisant for the treatment of narcolepsy, or any other clinical trial or preclinical studies in animals that we conduct, could mandate repeated or additional clinical trials and could result in changes to or delays in clinical trials in other indications. We do not know whether any clinical trials that we may conduct will demonstrate adequate efficacy and safety to result in regulatory approval to market pitolisant for our initial or potential additional indications, or any other product candidate. If later stage clinical trials do not produce favorable results, our ability to obtain regulatory approval for pitolisant for initial or potential additional indications, or any other product candidate, may be adversely impacted.

Our failure to successfully initiate and complete clinical trials of pitolisant for potential additional indications or any other product candidate and to demonstrate the efficacy and safety necessary to obtain regulatory approval to market pitolisant or any other product candidate would significantly harm our business. Our product candidate development costs will also increase if we experience delays in testing or regulatory approvals and we may be required to obtain additional funds to complete clinical trials. We cannot assure you that our clinical trials will begin as planned or be completed on schedule, if at all, or that we will not need to restructure our trials after they have begun. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our product candidates, which may harm our business and results of operations. In addition, many of the factors that cause, or lead to, delays of clinical trials may ultimately lead to the denial of regulatory approval of pitolisant or any other product candidate.

In addition, prior to our acquisition of the rights to pitolisant, we had no involvement with or control over the nonclinical or clinical development of pitolisant. Additionally, pursuant to our collaboration agreement with Bioprojet, we will rely on data generated by Bioprojet in connection with seeking regulatory approval of pitolisant in the territories in which we have rights to develop and commercialize pitolisant. We are dependent on Bioprojet having conducted such research and development in accordance with the applicable protocols and legal, regulatory and scientific standards, having accurately reported the results of all clinical trials and other research they conducted prior to our acquisition of the rights to pitolisant, having correctly collected and interpreted the data from these trials and other research, and having supplied us with complete information, data sets and reports required to adequately demonstrate the results reported through the date of our acquisition of these assets. Problems related to predecessors could result in increased costs and delays in the development of pitolisant for additional indications, which could adversely affect our ability to generate any future revenue from sales of pitolisant, if approved for additional indications.

A Fast Track Designation from the FDA, even if granted for any of our product candidates, may not lead to a faster development or regulatory review or approval process, and does not increase the likelihood that our product candidates will receive regulatory approval.

During the development of certain of our product candidates, we received Fast Track Designation from the FDA and we may also seek further designations for some or all of our other product candidates. The Fast Track program is intended to expedite or facilitate the process for reviewing new product candidates that meet certain criteria. Specifically, drugs are eligible for Fast Track designation if they are intended, alone or in combination with one or more drugs or biologics, to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. The sponsor of a Fast Track product candidate has opportunities for more frequent interactions with the applicable FDA review team during product development and, once an NDA is submitted, the application may be eligible for priority review. An NDA submitted for a Fast Track product candidate may also be eligible for rolling review, where the FDA may consider for review sections of the NDA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA, the FDA agrees to accept sections of the NDA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the application.

The FDA has broad discretion whether or not to grant this designation. Even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. Even if we do receive Fast Track Designation for any of our product candidates, such product candidates may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may also withdraw Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development program.

Furthermore, such a designation does not increase the likelihood that ZYN002 or any other product candidate that may be granted Fast Track designation will receive regulatory approval in the U.S. Many product candidates that have received Fast Track Designation have ultimately failed to obtain approval.

Interim, "topline" and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose interim, "topline" or preliminary data from our clinical trials, which is based on a preliminary analysis of then-available topline data, and the results and related findings and conclusions are subject to change following completion of the study or a full analyses of all data related to the particular trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the interim, "topline" or preliminary results that we report may differ from future results of the same trials, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. "Topline" data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, "topline" data should be viewed with caution until the final data are available. We may also disclose interim data from our clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Adverse differences between "topline," preliminary or interim data and final data could significantly harm our business prospects.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our business in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular drug, product candidate or our business. If the interim, "topline" or preliminary data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for and commercialize our product candidates, our business, operating results, prospects or financial condition may be harmed.

Enrollment and retention of patients in clinical trials is an expensive and time-consuming process and could be made more difficult or rendered impossible by multiple factors outside our control.

We may encounter delays in enrolling, or be unable to enroll, a sufficient number of patients to complete any of our clinical trials on our current timelines, or at all, and even once enrolled we may be unable to retain a sufficient number of patients to complete any of our trials. Enrollment in our clinical trials may be slower than we anticipate, leading to delays in our development timelines. Patient enrollment and retention in clinical trials depends on many factors, including the size of the patient population, the nature of the trial protocol, our ability to recruit clinical trial investigators with the appropriate competencies and experience, the existing body of safety and efficacy data with respect to the study drug, the number and nature of competing treatments and ongoing clinical trials of competing drugs for the same indication, the proximity of patients to clinical sites, the eligibility criteria for the trial and the proportion of patients screened that meets those criteria, our ability to obtain and maintain patient consents, and the risk that patients enrolled in clinical trials will drop out of the trials before completion.

Furthermore, any negative results or new safety signals we or third parties may report in clinical trials of our product candidates may make it difficult or impossible to recruit and retain patients in our clinical trials. Similarly, negative results reported by our competitors about their drug candidates may negatively affect patient recruitment in our clinical trials. In addition, marketing authorization of competitors in this same class of drugs may impair our ability to enroll patients into our clinical trials, delaying or potentially preventing us from completing recruitment of one or more of our trials. Delays or failures in planned patient enrollment or retention may result in increased costs, program delays or both, which could have a harmful effect on our ability to develop pitolisant or any future product candidates, or could render further development impossible. In addition, we expect to rely on CROs and clinical trial sites to ensure proper and timely conduct of our future clinical trials, and, while we intend to enter into agreements governing their services, we will be limited in our ability to compel their actual performance.

Even if we receive orphan drug designation for our product candidates, we may not be able to maintain the benefits associated with orphan drug designation, including marketing exclusivity, which may cause our product revenue to be reduced.

Regulatory authorities in some jurisdictions, including the United States, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States, or a patient population greater than 200,000 in the United States, where there is no reasonable expectation that the cost of developing the drug or biologic will be recovered from sales in the United States. For example, the FDA granted orphan drug designation to pitolisant for the treatment of narcolepsy in 2010, and for the treatment of IH in 2023.

In the United States, orphan designation entitles a party to financial incentives such as opportunities for grant funding for clinical trial costs, tax advantages and user-fee waivers. In addition, generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the disease or condition for which it has such designation, the drug is entitled to a seven-year period of marketing exclusivity, which precludes the FDA from approving another marketing application for the same drug for the same disease or condition for that time period. Under the FDA's regulations, the FDA will deny orphan drug exclusivity to a designated drug upon approval if the FDA has already approved another drug with the same active ingredient for the same indication, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or where the manufacturer is unable to assure sufficient product quantity. In connection with WAKIX's approvals, we received orphan drug exclusivities for the treatment of excessive daytime sleepiness in adult patients with narcolepsy, and for the treatment of cataplexy in adult patients with narcolepsy.

Orphan drug exclusivity in the United States may be unavailable where the indication for which the product candidate is approved is broader than the orphan-designated indication or is otherwise different from the orphan-designated disease or condition.

Even if we obtain orphan drug exclusivity for a drug candidate, that exclusivity may not effectively protect the candidate from competition. WAKIX may face additional competition because different drugs with a different active moiety can still be approved for the same condition. Even after an approved drug is granted orphan exclusivity, exclusivity may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to assure sufficient quantities of the drug to meet the needs of patients with the rare disease or condition following approval. In addition, the FDA can subsequently approve products with the same active moiety for the same condition if the FDA concludes that the later drug is safer, more effective, or makes a major contribution to patient care. Orphan drug designation neither shortens the development or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

We are subject to ongoing regulatory obligations and continued regulatory review with respect to WAKIX, which will result in significant additional expense. Additionally, WAKIX could be subject to labeling and other restrictions and market withdrawal, and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with WAKIX.

WAKIX is subject to extensive and ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, distribution, import, export, record keeping and submission of safety and other post-market information, including both federal and state requirements in the United States. In addition, manufacturers and manufacturers' facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to cGMP. As such, we and our contract manufacturers are subject to continual review and periodic inspections to assess compliance with cGMP. Accordingly, we and others with whom we work must continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production and quality control.

Our regulatory approval for WAKIX for the treatment of EDS or cataplexy in adult patients with narcolepsy, and any other regulatory approvals we may receive for pitolisant or any future product candidates may be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, which must comply with applicable GCP regulations. We could also be asked to conduct post marketing clinical studies to verify the safety and efficacy of future product candidates in general or in specific patient subsets. For example, as a part of the regulatory approval for WAKIX for the treatment of EDS in adult

patients with narcolepsy, we are required to conduct post-marketing studies in women exposed to pitolisant in pregnancy, including a registry-based observational cohort study to assess maternal, fetal, and infant outcomes of women exposed to pitolisant during pregnancy, and another study of a different design such as a case control study or a retrospective cohort study using electronic medical record data, and a lactation study.

We are also required to report certain adverse events and production problems, if any, to the FDA, and to comply with requirements concerning advertising and promotion for WAKIX. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved label. As such, we may not promote WAKIX for indications or uses for which it does not have FDA approval. The holder of an approved NDA must submit new or supplemental applications and obtain approval for certain changes to the approved product, product labeling or manufacturing process.

If a regulatory agency discovers previously unknown problems with WAKIX, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, or disagrees with the promotion, marketing, or labeling of a product, the regulatory agency may impose restrictions on the product or us, including requiring withdrawal of the product from the market. If we fail to comply with applicable regulatory requirements, a regulatory agency or enforcement authority may, among other things:

- issue warning or untitled letters;
- impose civil or criminal penalties, including product seizures and injunctions;
- limit or suspend regulatory approval;
- place clinical holds on any of our proposed or ongoing clinical trials;
- refuse to approve pending applications or supplements to approved applications;
- impose restrictions on our operations, including closing our contract manufacturers' facilities, on the manufacturing of our products, or on the labeling or marketing of our products; or
- seize or detain products, refuse to import or export products, or require or request a product recall or withdrawal of the products from the market.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. Any failure to comply with ongoing regulatory requirements may significantly and adversely affect our ability to commercialize and generate revenue from WAKIX or future product candidates. If regulatory sanctions are applied or if regulatory approval is withdrawn, the value of our company and our operating results will be adversely affected.

The regulatory requirements and policies may change, and additional government regulations may be enacted for which we may also be required to comply. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or in other countries. If we or any future collaboration partner are not able to maintain regulatory compliance, we or such collaboration partner, as applicable, may face government enforcement action and our business will suffer.

Our business operations and current and future relationships with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers are subject to applicable healthcare regulatory laws, which could expose us to penalties.

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers, may expose us to broadly applicable federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market,

sell and distribute our product candidates, if approved. The laws that affect our current and future operations include, but are not limited to:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or certain rebate), directly or indirectly, overtly or covertly, in cash or in kind, in exchange for, or to induce or reward, or in return for, either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item, or service for which payment may be made, in whole or in part, under any U.S. federal healthcare programs, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers and formulary managers, among others, on the other. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

- the U.S. federal civil and criminal false claims laws, such as the False Claims Act ("FCA"), which imposes significant penalties and can be enforced by private citizens through civil qui tam actions, and prohibits individuals or entities from, among other things, knowingly presenting, or causing to be presented, to the U.S. federal government, false, fictitious or fraudulent claims for payment of federal funds, and knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim, or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the U.S. federal government. For example, pharmaceutical companies have been prosecuted under the FCA in connection with their alleged off-label promotion of drugs, purportedly concealing price concessions in the pricing information submitted to the government for government price reporting purposes, and allegedly providing free product to customers with the expectation that the customers would bill federal health care programs for the product. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the FCA;

- the federal civil monetary penalties laws, which impose civil fines for, among other things, the offering or transfer of remuneration to a Medicare or state healthcare program beneficiary if the person knows or should know it is likely to influence the beneficiary's selection of a particular provider, practitioner, or supplier of services reimbursable by Medicare or a state healthcare program, unless an exception applies;

- HIPAA, which imposes criminal and civil liability for, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement, in connection with the delivery of, or payment for, healthcare benefits, items or services. Similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the healthcare fraud statute implemented under HIPAA or specific intent to violate it in order to have committed a violation;

- the FDCA, which prohibits, among other things, the adulteration or misbranding of drugs, biologics and medical devices;

- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers;

- federal price reporting laws, which require manufacturers to calculate and report complex pricing metrics to government programs, where such reported prices may be used in the calculation of reimbursement and/or discounts on approved products;

- state law equivalents of each of the above federal laws, such as state anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers, and may be broader in scope than their federal equivalents;

- federal transparency requirements detailing interactions with and payments to healthcare providers, such as the federal reporting requirements under the Physician Payments Sunshine Act, which requires, among other things, certain manufacturers of drugs, devices, biologics and medical supplies reimbursed under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to the HHS

information related to payments and other transfers of value provided to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other health care professionals (physician assistants, nurse practitioners, clinical nurse specialists, certified registered nurse anesthetists, anesthesiologist assistants, and certified nurse midwives), and teaching hospitals and physician ownership and investment interests, including such ownership and investment interests held by a physician's immediate family members. Failure to submit required information may result in civil monetary penalties;

- state laws that require pharmaceutical companies to implement compliance programs, comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, or to track and report gifts, compensation and other remuneration provided to physicians and other health care providers and other potential referral sources, state laws that require drug manufacturers to file reports relating to pricing information and marketing expenditures, state and local laws requiring the registration of pharmaceutical sales representatives;

- the U.S. Foreign Corrupt Practices Act of 1977, as amended, which prohibits, among other things, U.S. companies and their employees and agents from authorizing, promising, offering, or providing, directly or indirectly, corrupt or improper payments or anything else of value to foreign government officials, employees of public international organizations and foreign government owned or affiliated entities, candidates for foreign political office, and foreign political parties or officials thereof; and

- similar healthcare laws in the European Union and EEA and other jurisdictions, including reporting requirements detailing interactions with and payments to healthcare providers.

Ensuring that our business operations and current and future arrangements with third parties comply with applicable healthcare laws and regulations will likely be costly. It is possible that governmental authorities will conclude that our business practices, including, without limitation, our patient support and financial assistance programs, do not comply with current or future statutes, regulations, agency guidance or case law involving applicable fraud and abuse or other healthcare laws. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that apply to us, we may be subject to penalties, including civil, administrative and criminal penalties, damages, fines, the curtailment or restructuring of our operations, contractual damages, disgorgement, reputational harm, additional oversight and reporting obligations if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, the exclusion from participation in federal and state healthcare programs and individual imprisonment, any of which could adversely affect our ability to market pitolisant, if approved, and adversely impact our financial results. Further, defending against any such actions can be costly, time-consuming and may require significant personnel resources.

Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired. The risk of our being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the applicable regulatory agencies or the courts, and their provisions are open to a variety of interpretations.

Actual or perceived failure to comply with applicable data protection, privacy and security laws, regulations, standards and other requirements could adversely affect our business, results of operations, and financial condition.

The global data protection landscape is rapidly evolving, and we are or may become subject to numerous state, federal and foreign laws, requirements and regulations governing the collection, use, disclosure, retention, and security of personal data, such as information that we may collect in connection with clinical trials in the U.S. and abroad. Implementation standards and enforcement practices are likely to remain uncertain for the foreseeable future, and we cannot yet determine the impact future laws, regulations, standards, or perception of their requirements may have on our business. This evolution may create uncertainty in our business, affect our ability to operate in certain jurisdictions or to collect, store, transfer use and share personal information, necessitate the acceptance of more onerous obligations in our contracts, result in liability or impose additional costs on us. The cost of compliance with these laws, regulations and standards is high and is likely to increase in the future. Any failure or perceived failure by us to comply with federal, state or foreign laws or regulation, our internal policies and procedures or our contracts governing our processing of personal information could result in negative publicity, government investigations and enforcement actions, claims by third parties

and damage to our reputation, any of which could have a material adverse effect on our operations, financial performance and business.

Certain states have also adopted comparable privacy and security laws and regulations, some of which may be more stringent than HIPAA. Such laws and regulations will be subject to interpretation by various courts and other governmental authorities, thus creating potentially complex compliance issues for us and our future customers and strategic partners. In addition, the California Consumer Privacy Act of 2018 (the "CCPA") went into effect on January 1, 2020. The CCPA creates individual privacy rights for California consumers and increases the privacy and security obligations of entities handling certain personal information. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. The CCPA may increase our compliance costs and potential liability, and many similar laws have been proposed at the federal level and in other states. Further, the California Privacy Rights Act (the "CPRA") recently passed in California. The CPRA will impose additional data protection obligations on covered businesses, including additional consumer rights processes, limitations on data uses, new audit requirements for higher risk data, and opt outs for certain uses of sensitive data. It will also create a new California data protection agency authorized to issue substantive regulations and could result in increased privacy and information security enforcement. The majority of the provisions went into effect on January 1, 2023, and additional compliance investment and potential business process changes may be required. In the event that we are subject to or affected by HIPAA, the CCPA, the CPRA or other domestic privacy and data protection laws, any liability from failure to comply with the requirements of these laws could adversely affect our financial condition.

As our operations and business grow, we may become subject to or affected by new or additional data protection laws and regulations and face increased scrutiny or attention from regulatory authorities. In the U.S., HIPAA imposes, among other things, certain standards relating to the privacy, security, transmission and breach reporting of individually identifiable health information. Most healthcare providers, including research institutions from which we obtain patient health information, are subject to privacy and security regulations promulgated under HIPAA, as amended by HITECH. While we do not believe that we are currently acting as a covered entity or business associate under HIPAA and thus are not directly subject to its requirements or penalties. However, any person may be prosecuted under HIPAA's criminal provisions either directly or under aiding-and-abetting or conspiracy principles. Consequently, depending on the facts and circumstances, we could face substantial criminal penalties if we knowingly receive individually identifiable health information from a HIPAA-covered healthcare provider or research institution that has not satisfied HIPAA's requirements for disclosure of individually identifiable health information. In addition, we may maintain sensitive personally identifiable information, including health information, that we receive throughout the clinical trial process, in the course of our research collaborations, and directly from individuals (or their healthcare providers) who enroll in our patient assistance programs. As such, we may be subject to state laws requiring notification of affected individuals and state regulators in the event of a breach of personal information, which is a broader class of information than the health information protected by HIPAA.

Furthermore, certain health privacy laws, data breach notification laws, consumer protection laws and genetic testing laws may apply directly to our operations and/or those of our collaborators and may impose restrictions on our collection, use and dissemination of individuals' health information. Patients about whom we or our collaborators obtain health information, as well as the providers who share this information with us, may have statutory or contractual rights that limit our ability to use and disclose the information. We may be required to expend significant capital and other resources to ensure ongoing compliance with applicable privacy and data security laws. Claims that we have violated individuals' privacy rights or breached our contractual obligations, even if we are not found liable, could be expensive and time consuming to defend and could result in adverse publicity that could harm our business.

In Europe, the GDPR went into effect in May 2018 and imposes strict requirements for processing the personal data of individuals within the EEA. Companies that must comply with the GDPR face increased compliance obligations and risk, including more robust regulatory enforcement of data protection requirements and potential fines for noncompliance of up to €20 million or 4% of the annual global revenue of the noncompliant company, whichever is greater. Among other requirements, the GDPR regulates transfers of personal data subject to the GDPR to third countries that have not been found to provide adequate protection to such personal data, including the United States, and the efficacy and longevity of current transfer mechanisms between the EU and the United States remains uncertain. For example, in 2016, the EU and United States agreed to a transfer framework for data transferred from the EU to the United States, called the Privacy Shield. In July 2020, the Court of Justice of the European Union ("CJEU") invalidated the Privacy Shield for purposes of international transfers and imposed further restrictions on the use of standard contractual clauses ("SCCs"). The European Commission issued revised SCCs in June 2021 to account for the decision of the CJEU and recommendations made by the European Data Protection Board. The revised SCCs must be used for relevant new data transfers from September 27, 2021; existing

standard contractual clauses arrangements must be migrated to the revised clauses by December 27, 2022. The new SCCs apply only to the transfer of personal data outside of the EEA and not the UK; the UK's Information Commissioner's Office launched a public consultation on its draft revised data transfers mechanisms in August 2021. There is some uncertainty around whether the revised clauses can be used for all types of data transfers, particularly whether they can be relied on for data transfers to non-EEA entities subject to the GDPR. As supervisory authorities issue further guidance on personal data export mechanisms, including circumstances where the SCCs cannot be used, and/or start taking enforcement action, we could suffer additional costs, complaints and/or regulatory investigations or fines, and/or if we are otherwise unable to transfer personal data between and among countries and regions in which we operate, it could affect the manner in which we provide our services, the geographical location or segregation of our relevant systems and operations, and could adversely affect our financial results.

Further, from January 2021, companies have to comply with the GDPR and also the UK GDPR, which, together with the amended UK Data Protection Act 2018, retains the GDPR in UK national law. The UK GDPR mirrors the fines under the GDPR, i.e., fines up to the greater of €20 million (£17.5 million) or 4% of global turnover. The relationship between the United Kingdom and the European Union in relation to certain aspects of data protection law remains unclear, and it is unclear how United Kingdom data protection laws and regulations will develop in the medium to longer term, and how data transfers to and from the United Kingdom will be regulated in the long term. The European Commission has adopted an adequacy decision in favor of the United Kingdom, enabling data transfers from EU member states to the United Kingdom without additional safeguards. However, the UK adequacy decision will automatically expire in June 2025 unless the European Commission re-assesses and renews or extends that decision.

Although we work to comply with applicable laws, regulations and standards, our contractual obligations and other legal obligations, these requirements are evolving and may be modified, interpreted and applied in an inconsistent manner from one jurisdiction to another, and may conflict with one another or other legal obligations with which we must comply. If we or third-party CMOs, CROs or other contractors or consultants fail to comply with applicable federal, state or local regulatory requirements, we could be subject to a range of regulatory actions that could affect our or our contractors' ability to develop and commercialize our product candidates and could harm or prevent sales of any affected products that we are able to commercialize, or could substantially increase the costs and expenses of developing, commercializing and marketing our products. Any threatened or actual government enforcement action could also generate adverse publicity and require that we devote substantial resources that could otherwise be used in other aspects of our business. Increasing use of social media could give rise to liability, breaches of data security or reputational damage.

Clinical practice guidelines and recommendations published by various organizations could have significant influence on the use of WAKIX.

Professional societies, practice management groups, private health and science foundations and organizations involved in various diseases from time to time may publish guidelines or recommendations to the healthcare and patient communities. The recommendations of these groups or organizations may relate to such matters as usage, dosage, route of administration and use of concomitant therapies. Recommendations or guidelines suggesting the reduced use of WAKIX or the use of competitive or alternative products as the standard of care to be followed by patients and healthcare providers could result in decreased use of WAKIX.

Product candidates we develop in the future may be classified as controlled substances, the making, use, sale, importation, exportation and distribution of which are subject to regulation by state, federal and foreign law enforcement and other regulatory agencies.

Product candidates we develop in the future may be classified as controlled substances, which are subject to state, federal and foreign laws and regulations regarding their manufacture, use, sale, importation, exportation and distribution. Controlled substances are regulated under CSA and regulations of the DEA.

The DEA regulates controlled substances as Schedule I, II, III, IV or V substances. Schedule I substances by definition have no established medicinal use and may not be marketed or sold in the United States. A pharmaceutical product may be listed as Schedule II, III, IV or V, with Schedule II substances considered to present the highest risk of abuse and Schedule V substances the lowest relative risk of abuse among such substances.

Various states also independently regulate controlled substances. Though state-controlled substances laws often mirror federal law, because the states are separate jurisdictions, they may separately schedule drugs as well. While some

states automatically schedule a drug when the DEA does so, in other states there must be rulemaking or a legislative action. State scheduling may delay commercial sale of any controlled substance drug product for which we obtain federal regulatory approval and adverse scheduling could impair the commercial attractiveness of such product. We or our collaborators must also obtain separate state registrations in order to be able to obtain, handle and distribute controlled substances for clinical trials or commercial sale, and failure to meet applicable regulatory requirements could lead to enforcement and sanctions from the states in addition to those from the DEA or otherwise arising under federal law.

For any of our products or product candidates classified as controlled substances, we and our suppliers, manufacturers, contractors, customers and distributors are required to obtain and maintain applicable registrations from state, federal and foreign law enforcement and regulatory agencies and comply with state, federal and foreign laws and regulations regarding the manufacture, use, sale, importation, exportation and distribution of controlled substances. There is a risk that DEA regulations may limit the supply of the compounds used in clinical trials for our product candidates, and, in the case of our approved products, the ability to produce and distribute our products in the volume needed to meet commercial demand.

Regulations associated with controlled substances govern manufacturing, labeling, packaging, testing, dispensing, production and procurement quotas, recordkeeping, reporting, handling, shipment and disposal. These regulations increase the personnel needs and the expense associated with development and commercialization of product candidates including controlled substances. The DEA, and some states, conduct periodic inspections of registered establishments that handle controlled substances. Failure to obtain and maintain required registrations or comply with any applicable regulations could delay or preclude us from developing and commercializing our product candidates containing controlled substances and subject us to enforcement action. The DEA may seek civil penalties, refuse to renew necessary registrations or initiate proceedings to revoke those registrations. In some circumstances, violations could lead to criminal proceedings. Because of their restrictive nature, these regulations could limit commercialization of any of our approved products or product candidates that are classified as controlled substances.

Enacted and future healthcare legislative changes may increase the difficulty and cost for us to obtain marketing approval for and commercialize our product candidates and affect the prices we may obtain.

In the United States, the European Union and other some foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities, and affect our ability to profitably sell any products for which we obtain marketing approval. In particular, there have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, collectively the ACA, was enacted to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the healthcare industry, and impose additional healthcare policy reforms. The law has continued the downward pressure on pharmaceutical pricing, especially under the Medicare program, and increased the industry's regulatory burdens and operating costs. Among the provisions of the ACA of importance to the pharmaceutical industry and our potential product candidates are the following:

- an annual, non-deductible fee payable by any entity that manufactures or imports specified branded prescription drugs and biologic agents (other than those designated as orphan drugs), which is apportioned among these entities according to their market share in certain government healthcare programs;

- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program for branded and generic drugs;

- a methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;

- the Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 70% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries under their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D;

- extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;

- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to certain individuals with income at or below 133% of the federal poverty level, thereby potentially increasing a manufacturer's Medicaid rebate liability;

- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;

- establishment of a Center for Medicare and Medicaid Innovation at the CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending; and

- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

Since its enactment, there have been numerous judicial, executive and legislative challenges to certain aspects of the ACA. On June 17, 2021 the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. Thus, the ACA will remain in effect in its current form. Further, prior to the U.S. Supreme Court ruling, President Biden issued an executive order to initiate a special enrollment period from February 15, 2021 through August 15, 2021 for purposes of obtaining health insurance coverage through the ACA marketplace. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA.

Other legislative changes have been proposed and adopted in the United States since the ACA was enacted. For example, in August 2011, the Budget Control Act of 2011, among other things, led to aggregate reductions to Medicare payments to providers, which went into effect in April 2013 and, due to subsequent legislative amendments to the statute, will stay in effect through 2032, with the exception of a temporary suspension from May 1, 2020 through March 31, 2022, unless additional Congressional action is taken. Additionally, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several types of providers, including hospitals and cancer treatment centers, and increased the statute of limitations period in which the government may recover overpayments to providers from three to five years. The Inflation Reduction Act of 2022, or IRA, which was signed into law on August 16, 2022, included several provisions to reduce drug spending by the federal government and reduce out-of-pocket costs for patients on Medicare. This legislation requires that the federal government negotiate prices for some drugs covered under Medicare Part B and Part D with the highest total spending, beginning in 2026. It also requires manufacturers to pay rebates back to the federal government if the price of their medications for Medicare beneficiaries rises faster than inflation. The legislation also placed a cap on out-of-pocket costs for Medicare Part D enrollees and shifted the cost of supplying these medications to manufacturers and plans. Eligibility for the Medicare Part D Low-Income Subsidy Program was also expanded under the legislation, and it further delayed the implementation of the Trump Administration's drug rebate rule to 2027. On August 29, 2023, the Secretary of Health and Human Services, or HHS, announced the list of the first ten drugs that will be subject to price negotiations, although the drug price negotiation program is currently subject to legal challenges. For that and other reasons, it is currently unclear how the IRA will be effectuated, or the impact of the IRA on our business.

Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, CMS may develop new payment and delivery models, such as bundled payment models. In addition, recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under government payor programs, and review the relationship between pricing and manufacturer patient programs. While any proposed measures will require authorization through additional legislation to become effective, the probability of their success is uncertain.

Individual states in the United States have also increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third- party payors or other restrictions could harm our business, results of operations, financial condition and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. Furthermore, there has been increased interest by third-party payors and governmental authorities in reference pricing systems and publication of discounts and list prices. These reforms could reduce the ultimate demand for our commercial products and product candidates, once approved, or put pressure on our product pricing.

In the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our product candidates, if approved. In addition to continuing pressure on prices and cost containment measures, legislative developments at the European Union or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the European Union, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than European Union, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most European Union member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing European Union and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize our product candidates, if approved. In markets outside of the United States and European Union, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action in the United States, the European Union or any other jurisdiction. If we or any third parties we may engage are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, we may be subject to enforcement action and we may not achieve or sustain profitability.

If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs that we participate in, we could be subject to additional reimbursement requirements, penalties, sanctions and fines, which could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

We participate in and have certain price reporting obligations to the Medicaid Drug Rebate Program. Under the Medicaid Drug Rebate Program, we are required to pay a rebate to each state Medicaid program for our covered outpatient drugs that are dispensed to Medicaid beneficiaries and paid for by a state Medicaid program as a condition of having federal funds being made available to the states for our drugs under Medicaid and Medicare Part B. Those rebates are based on pricing data we have to report on a monthly and quarterly basis to the CMS, the federal agency that administers the Medicaid Drug Rebate Program. These data include, among other things, the average manufacturer price (“AMP”) and, in the case of innovator products, the best price (“BP”) for each drug which, in general, represents the lowest price available from the manufacturer to any entity in the U.S. in any pricing structure, calculated to include all sales and associated rebates, discounts and other price concessions. We are liable for errors associated with our submission of pricing data and for any overcharging of government payors. For example, failure to submit monthly and/or quarterly AMP and BP data on a timely basis could result in a civil monetary penalty for each day the submission is late beyond the due date. Failure to make necessary disclosures and/or to identify overpayments could result in allegations against us under the Federal False Claims Act and other laws and regulations. Any required refunds to the U.S. government or responding to a government investigation or enforcement action would be expensive and time consuming and could have a material adverse effect on our business, results of operations and financial condition.

Federal law requires that any company that participates in the Medicaid Drug Rebate Program also participate in the 340B program in order for federal funds to be available for the manufacturer’s drugs under Medicaid and Medicare Part B. The 340B program requires participating manufacturers to agree to charge statutorily defined covered entities no more than the 340B “ceiling price” for the manufacturer’s covered outpatient drugs. These 340B covered entities include a

variety of community health clinics and other entities that receive health services grants from the Public Health Service, as well as hospitals that serve a disproportionate share of low-income patients. The ACA expanded the list of covered entities to include certain free-standing cancer hospitals, critical access hospitals, rural referral centers and sole community hospitals, but exempts "orphan drugs" from the ceiling price requirements for these covered entities. The 340B ceiling price is calculated using a statutory formula based on the average manufacturer price and rebate amount for the covered outpatient drug as calculated under the Medicaid Drug Rebate Program, and in general, products subject to Medicaid price reporting and rebate liability are also subject to the 340B ceiling price calculation and discount requirement. Any additional future changes to the definition of average manufacturer price and the Medicaid rebate amount under the ACA or other legislation or regulation could affect our 340B ceiling price calculations and negatively impact our results of operations commercializing pitolisant. In addition, legislation may be introduced that, if passed, would further expand the 340B program to additional covered entities or would require participating manufacturers to agree to provide 340B discounted pricing on drugs used in an inpatient setting.

To be eligible to have our products that we successfully commercialize paid for with federal funds under the Medicaid program and purchased by certain federal agencies and grantees, we have to participate in the U.S. Department of Veterans Affairs ("VA"), Federal Supply Schedule ("FSS") pricing program. As part of this program, we are obligated to make our products available for procurement on an FSS contract under which we must comply with standard government terms and conditions and charge a price that is no higher than the statutory Federal Ceiling Price ("FCP") to four federal agencies (VA, U.S. Department of Defense ("DOD"), Public Health Service, and U.S. Coast Guard).

We are subject to U.S. and certain foreign export and import controls, sanctions, embargoes, anti-corruption laws, and antimony laundering laws and regulations. Compliance with these legal standards could impair our ability to compete in domestic markets. We can face criminal liability and other serious consequences for violations, which can harm our business.

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations, various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls, the U.S. Foreign Corrupt Practices Act of 1977, as amended, the United States domestic bribery statute contained in 18 U.S.C. § 201, the United States Travel Act, the USA PATRIOT Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, contractors and other partners from authorizing, promising, offering, or providing, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. We may have direct or indirect interactions with officials and employees of government agencies or government- affiliated hospitals, universities, and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, contractors and other partners, even if we do not explicitly authorize or have actual knowledge of such activities. Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm and other consequences.

Disruptions at the FDA and other government agencies caused by funding shortages or global health concerns could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new or modified products from being developed, approved or commercialized in a timely manner or at all, or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA and foreign regulatory authorities to review and approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory, and policy changes, the FDA's or foreign regulatory authorities' ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's or foreign regulatory authorities' ability to perform routine functions. Average review times at the FDA and foreign regulatory authorities have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable. Disruptions at the FDA and other agencies may also slow the time necessary for new [drugs, and biologics] or modifications to approved [drugs and biologics] to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities.

Separately, in response to the COVID-19 pandemic, the FDA postponed most inspections at domestic and foreign manufacturing facilities at various points. Even though the FDA has since resumed standard inspection operations, the FDA has continued to monitor and implement changes to its inspectional activities, and any resurgence of the virus may lead to other inspectional or administrative delays. If a prolonged government shutdown occurs, or if global health concerns hinder or prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

We will need to obtain FDA approval of any proposed product names, and any failure or delay associated with such approval may adversely affect our business.

Any proprietary name we intend to use for our product candidates will require approval from the FDA regardless of whether we have secured a formal trademark registration from the USPTO. The FDA reviews proposed product names, considering both the potential for the name to lead to medical errors due to confusion with other product names and whether the proposed name is overly fanciful, misleadingly implies unique effectiveness or composition, or contributes to overstatement of product efficacy, minimization of risk, broadening of product indications or unsubstantiated superiority. If the FDA objects to any of our proposed product names, we may be required to adopt an alternative name for our product candidates. If we adopt an alternative name, we would lose the benefit of our existing trademark applications for such product candidate, and may be required to expend significant additional resources in an effort to identify a suitable product name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA. We may be unable to build a successful brand identity for a new trademark in a timely manner or at all, which would limit our ability to commercialize our product candidates.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.

We rely, and will continue to rely, on a combination of patents, trademarks and confidentiality agreements with employees, consultants, collaborators, advisors and other third parties to protect the intellectual property related to our current and future product candidates. Our success depends in large part on our licensor's ability to obtain and maintain patent protection in the United States with respect to WAKIX and our ability to obtain and maintain patent protection in the United States and any other relevant foreign jurisdictions with respect to any future product candidates that we develop. We seek to ensure that our current and future licensors obtain appropriate patent protection to all product candidates that we license from them. The patent prosecution process is expensive and time- consuming, and we and our licensors may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

Our patent portfolio comprises three U.S. patents exclusively licensed to us from Bioprojet. One U.S. patent, No. 8,207,197 has claims directed to a polymorph, i.e., a specific crystalline form, of pitolisant and, methods for preparing that polymorph of pitolisant, which is expected to expire in February 2029 without taking into consideration any possible patent term extension. A second U.S. patent, No. 8,486,947, has claims directed to methods of treating excessive daytime sleepiness by administering pitolisant, which is expected to expire in September 2029 without taking into consideration any possible patent term extension.

The patents that we in-license now or the patents and patent applications that we own or in-license in the future may not have patentable claims that protect our current and future product candidates in the relevant jurisdictions where we intend to commercialize such products. There is no assurance that we and our licensor are aware of all potentially relevant prior art relating to future patent applications. As such, the patent examiner may find prior art that can prevent a patent from issuing from a pending patent application. During the patent examination process, we or our licensor may be required to narrow the pending claims to overcome prior art, a process that may limit the scope of patent protection. Even if patents do successfully issue based on our future patent applications, and even if the issued patents cover our current and future product candidates, including their compositions formulation, method of manufacture, and method of use, third parties may challenge our issued patents' validity, enforceability or scope, which may result in such patents being narrowed, invalidated or held unenforceable. Any successful opposition to these patents or any other patents owned by or licensed to us in the future could deprive us of rights necessary for the successful commercialization of any of our current or future product candidates, if approved. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced.

If the patent applications we may own or in-license in the future with respect to our current and future product candidates fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for any of our current or future product candidates, it could dissuade other companies from collaborating with us to develop future product candidates, and threaten our ability to commercialize our current and future product candidates. Notably, pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Any such outcome could have an adverse effect on our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions, and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than U.S. law does.

Publications of discoveries in scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our future patent applications may not result in patents being issued which protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. On September 16, 2011, the Leahy-Smith America Invents Act (the “Leahy-Smith Act”) was signed into law. The Leahy-Smith Act made a number of significant changes to United States patent laws. These include provisions that affect the way patent applications are prosecuted and challenged at the USPTO and may also affect patent litigation. The USPTO has developed and continues to develop new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective on March 16, 2013. Accordingly, it remains unclear what impact the Leahy-Smith Act, subsequent rulemaking, and judicial interpretation of the Leahy-Smith Act and regulations will have on the operation of our business. However, the Leahy- Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have an adverse effect on our business and financial condition. Moreover, future changes to the patent laws of the United States and foreign jurisdictions may adversely affect the term, scope, validity and enforceability of our or our licensor’s patent rights. For example, a new bill (Terminating the Extension of Rights Misappropriated Act, or TERM Act, H.R. 3199) percolating through the United States Congress aims to reduce the term of certain drug patents in order to ease generic entry and increase competition.

The inventorship and ownership rights for patents that we in-license or may own or in-license in the future may be challenged by third parties. Such challenges could result in loss of exclusive rights to such patents, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or require us to obtain a license from such third parties on commercially reasonable terms to secure exclusive rights. If any such challenges to inventorship or ownership were asserted, there is no assurance that a court would find in our favor or that, if we choose to seek a license, such license would be available to us on acceptable terms or at all.

Moreover, we may be subject to a third-party pre-issuance submission of prior art to the USPTO or become involved in pre- and post-issuance opposition, derivation, re-examination, inter partes review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications, whether owned or in-licensed now or in the future, is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our licensed patents may be challenged in the courts or patent offices in the United States. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part,

which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Moreover, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after the filing of the earliest non-provisional application to which the patent claims priority. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. We may be required to disclaim a portion of patent term in order to overcome double patenting rejections from the patent office, thus potentially shortening our exclusivity period. Without patent protection for our current or future product candidates, we may be open to competition from generic versions of such products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If we fail to comply with our obligations under any license, collaboration or other agreements, we may be required to pay damages and could lose intellectual property rights that are necessary for developing and protecting our current and future product candidates.

We have licensed certain intellectual property rights covering pitolisant from Bioprojet, and we may license intellectual property rights from others in the future. If, for any reason, our license agreement with Bioprojet or any future licensor is terminated or we otherwise lose the rights associated with a license, it could adversely affect our business. Our license agreement with Bioprojet imposes, and any future collaboration agreements or license agreements we enter into are likely to impose various development, commercialization, funding, milestone, royalty, diligence, sublicensing, insurance, patent prosecution and enforcement or other obligations on us. If we breach any material obligations, or use the intellectual property licensed to us in an unauthorized manner, we may be required to pay damages and the licensor may have the right to terminate the license, which could result in us being unable to develop, manufacture and sell products that are covered by the licensed technology, or having to negotiate new or reinstated licenses on less favorable terms, or enable a competitor to gain access to the licensed technology.

If we do not obtain protection under the Hatch-Waxman Amendments by extending the patent term for our current and future product candidates, our business may be harmed.

Our commercial success will largely depend on our licensor's ability to obtain and maintain patent and other intellectual property in the United States for pitolisant, and our target indications, and our ability to maintain obtain and maintain patent and other intellectual property in the United States for any product candidates. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting product candidates might expire before or shortly after such candidates begin to be commercialized. We expect to seek extensions of patent terms in the United States.

Depending upon the timing, duration and specifics of FDA marketing approval of our current and future product candidates, one or more of our U.S. patents may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years beyond the normal expiration of the patent as compensation for patent term lost during drug development and the FDA regulatory review process, which is limited to the approved indication (or any additional indications approved during the period of extension). This extension is limited to only one patent that covers the approved product, the approved use of the product, or a method of manufacturing the product. However, the applicable authorities, including the FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. We may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time-period or the scope of patent protection afforded could be less than we request.

If we or our licensor are unable to extend the expiration date of our or their existing patents or obtain new patents with longer expiry dates, as applicable, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data to obtain approval of competing products following our patent expiration and launch their product earlier than might otherwise be the case.

The validity, scope and enforceability of any patents listed in the Orange Book that cover our current and future product candidates can be challenged by third parties.

One or more third parties may challenge the current patents, or future patents within our portfolio, which could result in the invalidation of, or render unenforceable, some or all of the relevant patent claims or a finding of non-infringement. For example, if a third party files an ANDA for a generic drug containing pitolisant, and relies in whole or in part on studies conducted by or for us, the third party will be required to certify to the FDA that either: (1) there is no patent information listed in the FDA's Orange Book with respect to our NDA for the applicable approved product candidate; (2) the patents listed in the Orange Book have expired; (3) the listed patents have not expired, but will expire on a particular date and approval is sought after patent expiration; or (4) the listed patents are invalid or will not be infringed by the manufacture, use or sale of the third party's generic drug. A certification that the new drug will not infringe the Orange Book-listed patents for the applicable approved product candidate, or that such patents are invalid, is called a paragraph IV certification. If the third party submits a paragraph IV certification to the FDA, a notice of the paragraph IV certification must also be sent to us once the third party's ANDA is accepted for filing by the FDA. We may then initiate a lawsuit to defend the patents identified in the notice. The filing of a patent infringement lawsuit within 45 days of receipt of the notice automatically prevents the FDA from approving the third party's ANDA until the earliest of 30 months or the date on which the patent expires, the lawsuit is settled, or the court reaches a decision in the infringement lawsuit in favor of the third party. If we do not file a patent infringement lawsuit within the required 45-day period, the third party's ANDA will not be subject to the 30-month stay of FDA approval.

Moreover, a third party may challenge the current patents, or future patents within our portfolio, which could result in the invalidation of some or all of the patents that might otherwise be eligible for listing in the Orange Book for one of our products. If a third party successfully challenges all of the patents that might otherwise be eligible for listing in the Orange Book for one of our products, we will not be entitled to the 30-month stay of FDA approval upon the filing of an ANDA for a generic drug containing, for example, pitolisant, and relies in whole or in part on studies conducted by or for us.

Litigation or other proceedings to enforce or defend intellectual property rights are often very complex in nature, may be very expensive and time-consuming, may divert our management's attention from our core business, and may result in unfavorable results that could limit our ability to prevent third parties from competing with our current and future product candidates.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for noncompliance with these requirements.

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and other foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign national or international patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Noncompliance events that could result in abandonment or lapse of patent rights include, but are not limited to, failure to timely file national and regional stage patent applications based on our international patent application, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain patents and patent applications, whether owned or in-licensed now or in the future, covering any of our current or future product candidates, our competitors might be able to enter the market, which would have an adverse effect on our business.

We may need to acquire or license intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.

A third party may hold intellectual property, including patent rights that are important or necessary to the development of our current and future product candidates. It may be necessary for us to use the patented or proprietary technology of one or more third parties to commercialize our current and future product candidates. If we are unable to acquire such intellectual property outright, or obtain licenses to such intellectual property from such third parties when needed or on commercially reasonable terms, our ability to commercialize our current and future product candidates, if approved, would likely be delayed.

The risks described elsewhere pertaining to our intellectual property rights also apply to the intellectual property rights that we in-license, and any failure by us or our licensors to obtain, maintain, defend and enforce these rights could have an adverse effect on our business. In some cases we may not have control over the prosecution, maintenance or enforcement of the patents that we license, and may not have sufficient ability to provide input into the patent prosecution, maintenance and defense process with respect to such patents, and our licensors may fail to take the steps that we believe are necessary or desirable in order to obtain, maintain, defend and enforce the licensed patents.

Third-party claims or litigation alleging infringement of patents or other proprietary rights, or seeking to invalidate patents or other proprietary rights, may delay or prevent the development and commercialization of any of our current or future product candidates.

Our commercial success depends in part on our avoiding infringement and other violations of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the pharmaceutical and biotechnology industries, including patent infringement lawsuits, interferences, derivation and administrative law proceedings, inter partes review and post-grant review before the USPTO, as well as oppositions and similar processes in foreign jurisdictions. Numerous United States and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are or may in the future be developing product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, and as we gain greater visibility and market exposure as a public company, the risk increases that our product candidates or other business activities may be subject to claims of infringement of the patent and other proprietary rights of third parties.

Third parties may assert that we are infringing their patents or employing their proprietary technology without authorization.

There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our current and future product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our current and future product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our current and future product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire. Similarly, if any third-party patent was to be held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patent may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all. In addition, we may be subject to claims that we are infringing other intellectual property rights, such as trademarks or copyrights, or misappropriating the trade secrets of others, and to the extent that our employees, consultants or contractors use intellectual property or proprietary information owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our current and future product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful infringement or other intellectual property claim against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our affected products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our current and future product candidates, and we have done so from time to time. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize our current and future product candidates, which could harm our business significantly. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

We cannot provide any assurances that third-party patents do not exist which might be enforced against our current and future product candidates, resulting in either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties or other forms of compensation to third parties.

We may not identify relevant third-party patents or may incorrectly interpret the relevance, scope or expiration of a third-party patent, which might adversely affect our ability to develop and market our products.

We cannot guarantee that any of our patent searches or analyses, including the identification of relevant patents, the scope of patent claims or the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States and abroad that is relevant to or necessary for the commercialization of our current product candidate in any jurisdiction.

It is possible that we and our current and future licensors will fail to identify patentable aspects of research and development output before it is too late to obtain patent protection. The patent applications that we may own or in-license in the future may fail to result in issued patents with claims that cover our current and future product candidates. We and our current and future licensors may also inadvertently make statements to regulatory agencies during the regulatory approval process that may be inconsistent with positions that have been taken during prosecution of the patent applications, which may result in such patents being narrowed, invalidated or held unenforceable.

The scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history. Our interpretation of the relevance or the scope of a patent or a pending application may be incorrect, which may negatively affect our ability to market our products. We may incorrectly determine that our products are not covered by a third-party patent or may incorrectly predict whether a third party's pending application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States or abroad that we consider relevant may be incorrect, and our failure to identify and correctly interpret relevant patents may negatively affect our ability to develop and market our products.

We may become involved in lawsuits to protect or enforce our patents, the patents of our licensors or our other intellectual property rights, which could be expensive, time consuming and unsuccessful.

Competitors may infringe or otherwise violate the patents of our licensor or our other intellectual property rights. To counter infringement or unauthorized use, we may be required to file legal claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that an asserted patent is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that the asserted patent does not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of asserted patents at risk of being invalidated or interpreted narrowly and could put a related patent application at risk of not issuing. The initiation of a claim against a third party may also cause the third party to bring counter claims against us such as claims asserting that our patents are invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, non-enablement or lack of statutory subject matter. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant material information from the USPTO, or made a materially misleading statement, during prosecution. Third parties may also raise similar validity claims before the USPTO in post-grant proceedings such as ex parte re-examinations, inter partes review, or post-grant review, or oppositions or similar proceedings outside the United States, in parallel with litigation or even outside the context of litigation. The outcome following legal assertions of invalidity and unenforceability is unpredictable. We cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. For the patents and patent applications that we may license in the future, we may have limited or no right to participate in the defense of any licensed patents against challenge by a third party. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of any future patent protection on our current or future product candidates. Such a loss of patent protection could harm our business.

We may not be able to detect or prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States. Our business could be harmed if in litigation the prevailing party does not offer us a license on commercially reasonable terms. Any litigation or other proceedings to enforce our intellectual property rights may fail, and even if successful, may result in substantial costs and distract our management and other employees.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have an adverse effect on the price of our common shares.

Because of the expense and uncertainty of litigation, we may not be in a position to enforce our intellectual property rights against third parties.

Because of the expense and uncertainty of litigation, we may conclude that even if a third party is infringing our in-licensed patents, any patents that may be issued as a result of our future patent applications, or other intellectual property rights, the risk-adjusted cost of bringing and enforcing such a claim or action may be too high or not in the best interest of our company or our shareholders. In such cases, we may decide that the more prudent course of action is to simply monitor the situation or initiate or seek some other non-litigious action or solution.

Changes in U.S. patent law or the patent law of other countries or jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.

The United States has recently enacted and implemented wide-ranging patent reform legislation. The U.S. Supreme Court and the U.S. Court of Appeals for the Federal Circuit have issued numerous precedential opinions in recent years narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on actions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce patents that we have licensed or that we might obtain in the future. Similarly, changes in patent law and regulations in other countries or jurisdictions or changes in the governmental bodies that enforce them or changes in how the relevant governmental authority enforces patent laws or regulations may weaken our ability to obtain new patents or to enforce patents that we have licensed or that we may obtain in the future.

The U.S. federal government retains certain rights in inventions produced with its financial assistance under the Bayh-Dole Act. The federal government retains a “nonexclusive, non-transferable, irrevocable, paid-up license” for its own benefit. The Bayh-Dole Act also provides federal agencies with “march-in rights.” March-in rights allow the government, in specified circumstances, to require the contractor or successors in title to the patent to grant a “nonexclusive, partially exclusive, or exclusive license” to a “responsible applicant or applicants.” If the patent owner refuses to do so, the government may grant the license itself.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of their former employers or other third parties.

We employ individuals who were previously employed at other biotechnology or pharmaceutical companies. Although we seek to protect our ownership of intellectual property rights by ensuring that our agreements with our employees, collaborators and other third parties with whom we do business include provisions requiring such parties to assign rights in inventions to us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed confidential information of our employees’ former employers or other third parties. We may also be subject to claims that former employers or other third parties have an ownership interest in our future patents. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and if we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Even if we are successful, litigation could result in substantial cost and be a distraction to our management and other employees. Moreover, any such litigation or the threat thereof may adversely affect our reputation, our ability to form strategic alliances or sublicense our rights to collaborators, engage with scientific advisors or hire employees or consultants, each of which would have an adverse effect on our business, results of operations and financial condition.

In addition, while it is our policy to require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in

executing such an agreement with each party who in fact develops intellectual property that we regard as our own. Our and their assignment agreements may not be self- executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we or our licensors fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we and our licensors are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

Any trademarks we have obtained or may obtain may be infringed or be successfully challenged, resulting in harm to our business.

We expect to rely on trademarks as one means to distinguish any of our current and future product candidates that are approved for marketing from the products of our competitors. For example, we are marketing pitolisant for the treatment of EDS or cataplexy in adult patients with narcolepsy under the brand name WAKIX, which we have licensed from Bioprojet. We may design or create new trademarks and apply to register them, our trademark applications may not be approved in the United States or any relevant foreign jurisdiction. Third parties may oppose or attempt to cancel our trademark applications or trademarks, or otherwise challenge our use of the trademarks. In the event that our trademarks are successfully challenged, we could be forced to rebrand our drugs, which could result in loss of brand recognition and could require us to devote resources to advertising and marketing new brands. Our competitors may infringe our trademarks and we may not have adequate resources to enforce our trademarks. If we attempt to enforce our trademarks and assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Risks Related to Ownership of our Common Stock

Our directors, officers and principal stockholders beneficially own a significant percentage of our stock and are able to exert significant control over matters subject to stockholder approval.

As of December 31, 2023, our directors, officers, five percent or greater stockholders, and their respective affiliates beneficially owned in the aggregate approximately 64% of our outstanding voting stock. As a result, these stockholders have the ability to influence us through this ownership position. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents, and approval of any merger, sale of assets or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

Future sales of our common stock in the public market, including sales by our directors, officers, or significant shareholders, could cause our share price to fall.

Sales of a substantial number of shares of our common stock in the public market or the perception that these sales might occur could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity securities. Certain holders of our common stock are entitled to rights with respect to registration of such shares under the Securities Act pursuant to a registration rights agreement between such holders and us. If such holders, by exercising their registration rights, sell a large number of shares, they could adversely affect the market price for our common stock. Subject to compliance with applicable securities laws, our officers, directors and other shareholders and their respective affiliates may sell some or all of their common shares in the future. No prediction can be made as to the effect, if any, such future sales will have on the market price of the common shares prevailing from time to time.

If securities or industry analysts do not publish research or publish unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock is influenced by the research and reports that industry or securities analysts publish about us or our business. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline. Moreover, if our operating results do not meet the expectations of the investor community, one

or more of the analysts who cover our company may change their recommendations regarding our company, and our stock price could decline.

Our quarterly operating results may fluctuate significantly.

Our operating results are subject to quarterly fluctuations. Our net income and other operating results are affected by numerous factors, including:

- variations in the level of expenses related to our development programs;
- addition or termination of clinical trials;
- any intellectual property infringement lawsuit in which we may become involved;
- regulatory developments affecting pitolisant;
- execution of any collaborative, licensing or similar arrangements, and the timing of payments we may make or receive under these arrangements;
- the achievement and timing of milestone payments under our existing collaboration and license agreements; and
- the level of underlying demand for WAKIX and customers' buying patterns.

If our quarterly operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any quarterly fluctuations in our operating results may, in turn, cause the price of our stock to fluctuate substantially.

Future sales and issuances of our common stock or rights to purchase our common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause the stock price of our common stock to decline.

In the future, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. We also expect to issue common stock to employees, consultants and directors pursuant to our equity incentive plans. If we sell common stock, convertible securities or other equity securities in subsequent transactions, or common stock is issued pursuant to equity incentive plans, investors may be materially diluted. New investors in such subsequent transactions could gain rights, preferences and privileges senior to those of holders of our common stock.

Our charter documents and Delaware law could prevent a takeover that stockholders consider favorable and could also reduce the market price of our stock.

Our amended and restated certificate of incorporation (the "Certificate of Incorporation") and our amended and restated bylaws (the "Bylaws") contain provisions that could delay or prevent a change in control of the Company. These provisions could also make it more difficult for stockholders to elect directors and take other corporate actions. These provisions include:

- providing for a classified board of directors with staggered, three-year terms;
- authorizing our board of directors to issue preferred stock with voting or other rights or preferences that could discourage a takeover attempt or delay changes in control;
- prohibiting cumulative voting in the election of directors;
- providing that vacancies on our board of directors may be filled only by a majority of directors then in office, even though less than a quorum;

- prohibiting the adoption, amendment or repeal of our Bylaws or Certificate of Incorporation regarding the election and removal of directors without the required approval of at least 66.67% of the shares entitled to vote at an election of directors;

- prohibiting stockholder action by written consent;

- limiting the persons who may call special meetings of stockholders; and

- requiring advance notification of stockholder nominations and proposals.

These provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors, which is responsible for appointing the members of our management.

In addition, we are subject to the anti-takeover provisions contained in Section 203 of the Delaware General Corporation Law (the "DGCL"). Under Section 203 of the DGCL, a corporation may not, in general, engage in a business combination with any holder of 15% or more of its capital stock unless the holder has held the stock for three years or, among other exceptions, the board of directors has approved the transaction.

These and other provisions in our Certificate of Incorporation, Bylaws and under Delaware law could discourage potential takeover attempts, reduce the price investors are willing to pay for shares of our common stock and result in the market price of our common stock being lower than it would be without these provisions.

Our Certificate of Incorporation provides that the Court of Chancery of the State of Delaware will be the exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our Certificate of Incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for:

- any derivative action or proceeding brought on our behalf;

- any action asserting a claim of breach of a fiduciary duty owed by, or other wrongdoing by, any of our current or former directors, officers, employees or our stockholders;

- any action asserting a claim against us arising under the DGCL, our amended and restated certificate of incorporation, or our amended and restated bylaws (as either may be amended from time to time) or as to which the DGCL confers jurisdiction on the Court of Chancery of the State of Delaware; and

- any action asserting a claim against us that is governed by the internal-affairs doctrine.

Our stockholders are deemed to have notice of and have consented to the provisions of our amended and restated certificate of incorporation related to choice of forum. This exclusive forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers and other employees. If a court were to find the exclusive forum provision in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving the dispute in other jurisdictions, which could seriously harm our business.

Claims for indemnification by our directors and officers may reduce our available funds to satisfy successful third-party claims against us and may reduce the amount of money available to us.

Our Certificate of Incorporation and Bylaws provide that we will indemnify our directors and officers, in each case to the fullest extent permitted by Delaware law.

In addition, as permitted by Section 145 of the DGCL, our amended and restated bylaws and our indemnification agreements that we have entered into with our directors and officers provide that:

- we will indemnify our directors and officers for serving us in those capacities or for serving other business enterprises at our request, to the fullest extent permitted by Delaware law. Delaware law provides that a corporation may indemnify such person if such person acted in good faith and in a manner such person reasonably believed to be in or not opposed to the best interests of the registrant and, with respect to any criminal proceeding, had no reasonable cause to believe such person's conduct was unlawful;

- we may, in our discretion, indemnify employees and agents in those circumstances where indemnification is permitted by applicable law;

- we are required to advance expenses, as incurred, to our directors and officers in connection with defending a proceeding, except that such directors or officers shall undertake to repay such advances if it is ultimately determined that such person is not entitled to indemnification;

- we will not be obligated pursuant to our amended and restated bylaws to indemnify a person with respect to proceedings initiated by that person against us or our other indemnitees, except with respect to proceedings authorized by our board of directors or brought to enforce a right to indemnification;

- the rights conferred in our amended and restated bylaws are not exclusive, and we are authorized to enter into indemnification agreements with our directors, officers, employees and agents and to obtain insurance to indemnify such persons; and

- we may not retroactively amend our amended and restated bylaw provisions to reduce our indemnification obligations to directors, officers, employees and agents.

Item 1B. Unresolved Staff Comments.

None.

Item 1C. Cybersecurity

Introduction

The global data protection landscape is rapidly evolving, and we may be impacted by or subject to new, amended, or existing laws and regulations in the future, including as our operations continue to expand or if we begin to operate in foreign jurisdictions. The risk of a security breach or disruption or data loss, particularly through social engineering attacks, cyber-attacks, or cyber intrusion, including by computer hackers, foreign governments, and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased.

We collect and maintain data and information necessary to conduct our business, and we are increasingly dependent on information technology systems and infrastructure to operate our business, including systems infrastructure operated and maintained by third-party service providers. In the ordinary course of business, we collect, store, and transmit confidential information, including intellectual property, proprietary business information and personal information. It is critical that we do so in a secure manner to maintain the privacy, security, confidentiality, and integrity of such confidential information.

We have established physical, electronic, and organizational measures to safeguard and secure our systems and facilities to prevent an information compromise. We rely on commercially available systems, software, tools and monitoring to provide security for our information technology systems and the processing, transmission, and storage of digital information. We have also outsourced elements of our information technology infrastructure, and as a result, a number of third-party vendors may or could have access to our confidential information.

The cost to mitigate, investigate and respond to potential security incidents, breaches, disruptions, network security problems, bugs, viruses, worms, malicious software programs and security vulnerabilities could be significant, and while we have implemented security measures to protect our data and information technology systems, our efforts to address these potential risks may not be successful, and could result in unexpected interruptions, delays, cessation of service and other harm to our business and competitive position.

If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our product development programs.

For example, the loss of clinical trial data from completed or ongoing or planned clinical trials could delay our regulatory approval efforts and significantly increase our costs as a result of efforts to recover or reproduce the lost data. Moreover, if a real or perceived security breach affects our systems (or those of our third-party service providers), or result in the loss of or accidental, unlawful, or unauthorized access to, use of, release of or other processing of personally identifiable information or clinical trial data, our reputation could be materially damaged.

In addition, a breach may require notification to governmental agencies, the media, or individuals pursuant to applicable data privacy and security laws. We would also be exposed to a risk of loss, negative publicity, harm to our reputation, governmental investigation and/or enforcement actions, claims or litigation and potential liability, which could materially adversely affect our business, results of operations and financial condition.

Cyber Risk Governance

The Audit Committee is a sub-committee of our Board of Directors and is delegated to the role of cyber risk oversight for the Company.

Our management team, including the CIO, is responsible for assessing and managing our material risks from cybersecurity threats. The team has primary responsibility for our overall cybersecurity risk management program and supervises both our internal cybersecurity personnel and our retained external cybersecurity consultants. The CIO reports the progress of cyber risk reduction initiatives to the Audit Committee on a periodic basis. The CIO has extensive information technology experience in the corporate environment.

Our management team supervises efforts to prevent, detect, mitigate, and remediate cybersecurity risks and incidents through various means, which may include briefings from internal security personnel; threat intelligence and other information obtained from governmental, public or private sources, including external consultants engaged by us; and alerts and reports produced by security tools deployed in the IT environment.

In February 2024, we chartered the Information Systems, Data and Cybersecurity Governance Committee (the “Cybersecurity Committee”), which is comprised of the Company’s business unit leaders and is responsible for the management of our cyber risk exposure and monitoring the effectiveness of the cybersecurity program. The Cybersecurity Committee will report the progress of cyber risk reduction initiatives to our senior leadership and the Audit Committee on a periodic basis.

Cyber Risk Management Strategy

We have a cyber risk management policy and asset-based cyber risk management methodology for the continuous identification, assessment, and management of our cyber risk exposure. Our cyber risk managers have been trained in our cyber risk management policy and methodology. The methodology is as follows:

- Maintaining an up-to-date and accurate inventory of all assets (e.g., data, systems, hardware, software, and vendors).

- Categorization of all assets based on the criticality of the data processed and the assets criticality to the continuity of business operations.

- A profile is maintained of the most likely threats, their intent, and the impact the threat may have on the confidentiality, integrity, and availability of Company assets.

- Evaluation of relevant risk-based scenarios or vulnerability of an asset and how a threat may exploit the asset.
- Implemented security controls or “mitigating factors” are considered for each scenario.
- Considering the asset, threat, risk-based scenario or vulnerability, and the mitigating factors, a likelihood and impact determination is made to calculate the final risk level.
- Risk reduction plans are determined and used to prioritize security program initiatives.
- Risk mitigations are tracked and monitored in a risk register.

There can be no assurance that our cybersecurity risk management program and processes, including our policies, controls or procedures, will be fully implemented, complied with or effective in protecting our systems and information.

Cyber Risk Exposure

Our top cyber risks have been grouped into genericized categories to avoid disclosure of sensitive information but include sufficient detail for a reasonable investor to understand our cyber risks and maintain confidence we act in good faith to reduce our cyber risk exposure. In the fourth quarter of 2023, we performed a risk analysis of all critical assets, determining the following are our current top cyber risks, none of which are material:

- Compromise to the confidentiality of intellectual property.
- Compromise to the confidentiality and integrity of financial records.
- Compromise to the confidentiality of employee records.
- Compromise to the confidentiality, integrity, and availability of core business systems.
- Compromise to the confidentiality, integrity, and availability of critical third-party vendors supporting the continuity of business operations.

We have not identified any risks from known cybersecurity threats, including as a result of any prior cybersecurity incidents, that have materially affected us, including our operations, business strategy, results of operations, or financial condition. We face risks from cybersecurity threats that, if realized, are reasonably likely to materially affect us, including our operations, business strategy, results of operations, or financial condition. See “Risk Factors – *We depend on our information technology systems, and any failure of these systems could harm our business. Any real or perceived security breach, loss of data, and other disruptions or incidents could compromise the privacy, security, integrity or confidentiality of sensitive information related to our business or prevent us from accessing critical information and expose us to liability and reputational harm, which could adversely affect our business, results of operations and financial condition.”*

Third Party Risk Management

We leverage multiple third parties to support our business operations. Throughout the lifecycle of the vendor relationship, security is integrated as part of each process. We maintain a vendor inventory, categorized based in risk tiers, and perform due diligence of the vendor’s security practices commensurate with the risk tier. Vendor contracts and security practices are monitored to ensure an accurate assessment of risk is maintained.

Third Party Support of the Cyber Risk Management Program

We employ third parties to support our cyber risk management program in the following ways:

- Use of internal and external auditors to maintain compliance with regulatory requirements.

- Use of cybersecurity consultants and managed security services providers to supplement the security program practices and evaluate the program's effectiveness, specifically:
    - Governance, risk, and compliance services.
    - Penetration testing and vulnerability management.
    - Continuous security event monitoring.
    - Data loss prevention.

Incident Management

We make continuous efforts to maintain the ability to respond quickly and effectively to security incidents to minimize their impact. We implement an incident response policy, incident response plan, and deploy comprehensive continuous security monitoring solutions to monitor events occurring across its assets. Additionally, a material risk determination model enables our cyber incident response team and senior leadership to determine materiality.

Item 2. Properties.

We do not own any real property. Our corporate headquarters, located in Plymouth Meeting, Pennsylvania, has a footprint of approximately 35,781 square feet of space pursuant to leases that expire in 2024. We believe that our facilities are suitable to meet our current needs.

Item 3. Legal Proceedings.

From time to time, we may be subject to legal proceedings and claims in the ordinary course of business. The results of any current or future litigation cannot be predicted with certainty, and regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

ANDA Litigation

On September 27, 2023, we and our licensor, Bioprojet, received notice from Lupin Limited ("Lupin") pursuant to 21 U.S.C. § 355(j) *et seq.* and 21 C.F.R. § 314.95 *et seq.* (the "Lupin Notice Letter") that Lupin has submitted ANDA No. 218846 (the "Lupin ANDA") to the FDA and is seeking regulatory approval to market a generic version of WAKIX® before the expiration of U.S. Patent Nos. 8,486,947 ("'947 patent") and 8,207,197 ("'197 patent"). On September 27, 2023, we and Bioprojet received notice from Novugen Pharma Sdn. Bhd. ("Novugen") pursuant to 21 U.S.C. § 355(j) *et seq.* and 21 C.F.R. § 314.95 *et seq.* (the "Novugen Notice Letter") that Novugen has submitted ANDA No. 218834 (the "Novugen ANDA") to the FDA and is seeking regulatory approval to market a generic version of WAKIX® before the expiration of the '947 patent and '197 patent. The '947 patent and '197 patent are listed with respect to WAKIX® in the FDA's Orange Book and will expire in September 2029 and March 2030, respectively. The Lupin Notice Letter and the Novugen Notice Letter assert that their generic product will not infringe the '947 patent and '197 patent and/or that the '947 patent and '197 patent are invalid or unenforceable. On November 9, 2023, we, Bioprojet and Bioprojet's wholly owned subsidiary, Bioprojet Pharma SAS ("Bioprojet Pharma"), filed a complaint for patent infringement of the '947 patent and '197 patent against Lupin, Novugen and certain of their affiliates and agents in the United States District Court for the District of Delaware in response to their filing of their respective ANDAs with the FDA.

On October 12, 2023, we and Bioprojet received notice from Novitium Pharma LLC ("Novitium"), pursuant to 21 U.S.C. § 355(j) *et seq.* and 21 C.F.R. § 314.95 *et seq.* (the "Novitium Notice Letter"), that Novitium has submitted ANDA No. 218495 (the "Novitium ANDA") to the FDA and is seeking regulatory approval to market a generic version of WAKIX® before the expiration of U.S. Patent No. 8,354,430 (the "'430 patent"), which is also listed with respect to WAKIX® in the FDA's Orange Book and will expire in February 2026, '947 patent, and '197 patent. On October 12, 2023, we and Bioprojet received notice from Zenara Pharma Pvt. Ltd. ("Zenara"), pursuant to 21 U.S.C. § 355(j) *et seq.* and 21 C.F.R. § 314.95 *et seq.* (the "Zenara Notice Letter"), that Zenara has submitted ANDA No. 218796 (the "Zenara ANDA") to the FDA and is seeking regulatory approval to market a generic version of WAKIX® before the expiration of the '430 patent, '947 patent and

’197 patent. On October 14, 2023, we and Bioprojet received notice from AET Pharma US, Inc. (“AET”), pursuant to 21 U.S.C. § 355(j) *et seq.* and 21 C.F.R. § 314.95 *et seq.* (the “AET Notice Letter”), that AET has submitted ANDA No. 218892 (the “AET ANDA”) to the FDA and is seeking regulatory approval to market a generic version of WAKIX® before the expiration of the ’947 patent and ’197 patent. On October 16, 2023, we and Bioprojet received notice from Annora Pharma Private Limited (“Annora”), pursuant to 21 U.S.C. § 355(j) *et seq.* and 21 C.F.R. § 314.95 *et seq.* (the “Annora Notice Letter”), that Annora has submitted ANDA No. 218832 (the “Annora ANDA”) to the FDA and is seeking regulatory approval to market a generic version of WAKIX® before the expiration of the ’430 patent, the ’947 patent and ’197 patent. AET’s Notice Letter asserts that AET’s generic product will not infringe the ’947 patent and ‘197 patent and/or that ’947 patent and ‘197 patent are invalid or unenforceable. The Annora Notice Letter asserts that its generic product will not infringe the ‘430 patent, ’947 patent and ‘197 patent and/or that the ‘430 patent, ’947 patent and ‘197 patent are invalid or unenforceable. The Novitium Notice Letter asserts that its generic product will not infringe the ‘430 patent, ’947 patent and ‘197 patent and/or that the ‘430 patent, ’947 patent and ‘197 patent are invalid or unenforceable. The Zenara Notice Letter asserts that its generic product will not infringe the ‘430 patent, ’947 patent and ‘197 patent and/or that the ‘430 patent, ’947 patent and ‘197 patent are invalid or unenforceable. On November 21, 2023, we, Bioprojet and Bioprojet Pharma filed a complaint for patent infringement of the ’947 patent and ‘197 patent against AET, Annora, Novitium and Zenara and certain of their affiliates and agents and for patent infringement of the ’430 patent against Annora, Novitium and Zenara and certain of their affiliates and agents in the United States District Court for the District of Delaware in response to their filing of their respective ANDAs with the FDA.

On or around October 13, 2023, MSN Pharmaceuticals Inc. (“MSN Pharma”) sent correspondence to us and Bioprojet stating that that MSN Pharma has submitted ANDA No. 218873 (the “MSN ANDA”) to the FDA and is seeking regulatory approval to market a generic version of WAKIX®. On December 8, 2023, MSN Laboratories Private Limited (“MSN”) filed a declaratory judgment action in the United States District Court for the Eastern District of Virginia against Bioprojet claiming that the ‘430 patent, ’947 patent and ‘197 patent will not be infringed by MSN’s generic version of WAKIX® and that the ‘947 patent is invalid. On December 11, 2023, we, Bioprojet and Bioprojet Pharma filed a complaint in the United States District Court for the District of Delaware for patent infringement of the ‘430 patent, ’947 patent and ‘197 patent against MSN and MSN Pharma. On January 12, 2024, the declaratory judgment action was transferred from the United States District Court for the Eastern District of Virginia to the United States District Court for the District of Delaware.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.

Market Information

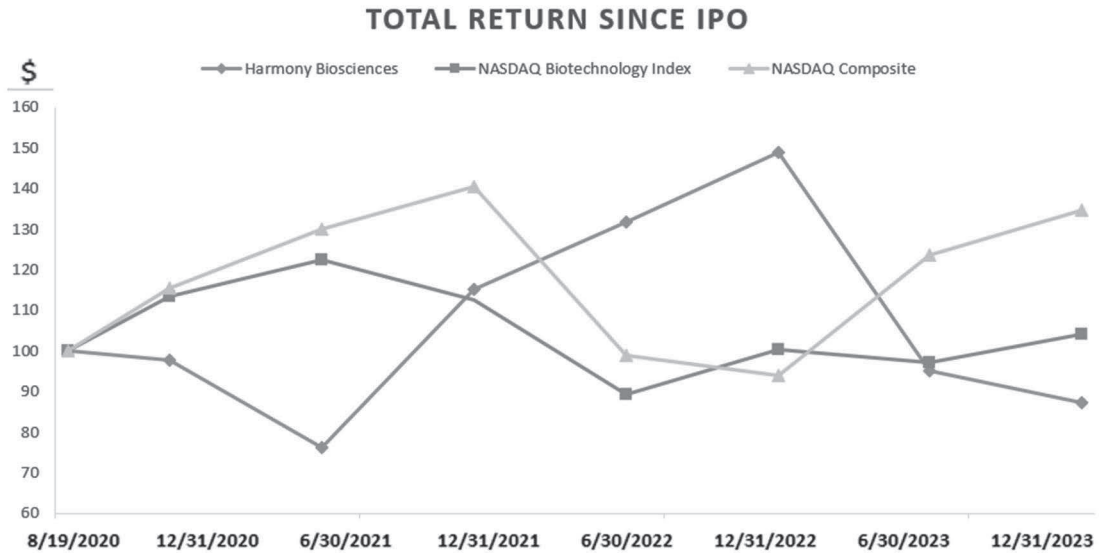
Our common stock is listed and traded on the Nasdaq Global Market under the symbol "HRMY."

Holders

As of February 16, 2024, we had 35 holders of record of our common stock. Because many of our shares of common stock are held by brokers and other institutions on behalf of shareholders, we are unable to estimate the total number of beneficial owners of our common stock represented by these holders.

Performance Graph

The following graph shows a comparison of the total cumulative shareholder returns of an investment of $100 in cash from August 19, 2020, the day we began trading, through December 31, 2023, in (i) our common stock, (ii) the Nasdaq Biotechnology Index, and (iii) the Nasdaq Composite Index. The shareholder returns in the graph below are based on historical data and are not indicative of, nor intended to forecast, future performance.



Purchases of Equity Securities

The following table summarizes information about our purchases of shares of our common stock for each of the months during the fourth quarter ended December 31, 2023.

Period	Total Number of Shares of Common Stock Purchased	Weighted Average Price Paid per Share of Common Stock (1)	Total Number of Shares Shares of Common Stock Purchases as Part of Publicly Announced Program (2)	Maximum Approximate Dollar Value of Shares of Common Stock that May Yet to be Purchased under the Program (in thousands) (3)
October 1, 2023, through October 31, 2023	—	$ —	—	$ 200,000
November 1, 2023, through November 31, 2023	1,436,666	$ 26.57	1,436,666	$ 161,821
December 1, 2023, through December 31, 2023	377,987	$ 31.27	377,987	$ 150,000
Total	1,814,653	$ 27.55	1,814,653	$ 150,000

(1) The weighted average price paid per share of common stock excludes commissions and transaction fees.

(2) On October 27, 2023, the Company's Board of Directors approved a program providing for the repurchase of shares of common stock in an aggregate amount of up to $200.0 million, excluding commissions and transaction fees. The repurchase program may be suspended, terminated or modified at any time for any reason. Such repurchases may be pursuant to Rule 10b-18 or Rule 10b5-1 agreements as determined by our management and in accordance with the requirements of the SEC. All repurchased shares of common stock shown in the table above were retired.

(3) The dollar amount shown (in thousands), represents, as of the end of each period, the approximate dollar value of shares of common stock that may yet be purchased under our publicly announced share repurchase program, exclusive of any commissions and transaction fees. The share repurchase program does not obligate us to repurchase any minimum dollar amount or number of shares of common stock.

Dividend Policy

We currently intend to retain all available funds and any future earnings to fund the development and growth of our business and to repay our debt obligations, therefore we do not anticipate declaring or paying any cash dividends on our common stock in the foreseeable future. Any future determination as to the declaration and payment of dividends, if any, will be at the discretion of our board of directors, subject to compliance with contractual restrictions and covenants in the agreements governing our current and future indebtedness. Any such determination will also depend upon our business prospects, results of operations, financial condition, cash requirements and availability, industry trends and other factors that our board of directors may deem relevant.

Initial Public Offering

In August 2020, we completed the IPO of our common stock, in which we issued and sold 6,151,162 shares, including 802,325 shares pursuant to the underwriters' over-allotment option at a price of $24.00 per share for an aggregate price of approximately $147.6 million. The offer and sale of the shares in our IPO was registered under the Securities Act pursuant to a registration statement on Form S-1 (File No. 333-240122), which was declared effective by the SEC on August 18, 2020 (the "Registration Statement"). We raised approximately $135.4 million, after deducting underwriting discounts and commissions and offering expenses of approximately $12.2 million. None of these expenses consisted of direct or indirect payments made by us to (i) our directors, officers or their associates, (ii) persons owning 10% or more of our common stock or (iii) to our affiliates.

As of December 31, 2023, we have applied all of the proceeds from our IPO.

Recent Sales of Unregistered Securities

Unless otherwise stated, the sales of the above securities were deemed to be exempt from registration under the Securities Act in reliance upon Section 4(a)(2) of the Securities Act (or Regulation D or Regulation S promulgated thereunder), or Rule 701 promulgated under Section 3(b) of the Securities Act as transactions by an issuer not involving any public offering or pursuant to benefit plans and contracts relating to compensation as provided under Rule 701. The recipients of the securities in each of these transactions represented their intentions to acquire the securities for investment only and not with a view to or for sale in connection with any distribution thereof, and appropriate legends were placed upon the stock certificates issued in these transactions.

Item 6. [Reserved].

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and related notes included elsewhere in this Annual Report on Form 10-K. This discussion contains forward-looking statements based upon current plans, expectations and beliefs involving risks and uncertainties. Our actual results may differ materially from those anticipated in these forward-looking statements as a result of various factors, including those set forth under "Risk Factors" and in other parts of this Annual Report on Form 10-K. A discussion of the year ended December 31, 2022, compared to the year ended December 31, 2021, has been reported previously under "Management's Discussion and Analysis of Financial Condition and Results of Operations" in our Annual Report on Form 10-K for the year ended December 31, 2022, filed with the SEC on February 21, 2023.

Company Overview

We are a commercial-stage, pharmaceutical company focused on developing and commercializing innovative therapies for patients living with rare neurological diseases as well as patients living with other neurological diseases who have unmet medical needs. Our product, WAKIX (pitolisant), is a first-in-class molecule with a novel mechanism of action ("MOA") specifically designed to increase histamine signaling in the brain by binding to H_3 receptors. In August 2019, WAKIX was approved by the U.S. Food and Drug Administration (the "FDA") for the treatment of excessive daytime sleepiness ("EDS") in adult patients with narcolepsy, and its U.S. commercial launch was initiated in November 2019. In October 2020, WAKIX was approved by the FDA for the treatment of cataplexy in adult patients with narcolepsy. WAKIX is the first-and-only approved product for patients with narcolepsy that is not scheduled as a controlled substance by the U.S. Drug Enforcement Administration (the "DEA").

We believe that pitolisant's ability to regulate histamine gives it the potential to provide therapeutic benefit in other rare neurological diseases that are mediated through H_3 receptors and histamine signaling. We are taking a mechanism-based approach to managing the life cycle of pitolisant and identified idiopathic hypersomnia ("IH"), another central disorder of hypersomnolence like narcolepsy, as our next potential new indication for WAKIX, which received orphan drug designation by the FDA in September 2023 and Fast Track Designation in November 2023. In April 2022, we initiated a Phase 3 registrational trial, the INTUNE Study, to evaluate the efficacy and safety of pitolisant in adult patients with IH. We completed enrollment in the INTUNE study in May 2023 and we announced topline data in October 2023. While the primary endpoint did not meet statistical significance, we believe the totality of the data showed favorable numerical trends for pitolisant in the treatment of adult patients with IH and we have scheduled a meeting with the FDA for March 2024 to discuss the path forward for IH. We anticipate meeting with the FDA towards the end of the first quarter of 2024. We are focusing our development efforts on other rare neurological disorders in which EDS is a prominent symptom, including Prader-Willi Syndrome ("PWS") and myotonic dystrophy, otherwise known as dystrophia myotonica ("DM"). Based on the positive signals from the data from our Phase 2 proof-of-concept signal detection clinical trial to evaluate pitolisant for the treatment of EDS and other key symptoms in patients with PWS, an end-of-phase 2 meeting with the FDA was held in June 2023. We believe we aligned with the FDA on the proposed Phase 3 registration study design to support further investigation of pitolisant as a potential treatment to address the unmet medical need for children, adolescents and adults with PWS experiencing EDS, for which there is currently no approved treatment. In October 2023, we received FDA alignment regarding the protocol for the Phase 3 TEMPO study in patients with PWS, which we believe will satisfy the requirements for both the registrational trial and one of the two requirements for pediatric exclusivity for pitolisant. In February 2024, the FDA granted Orphan Drug designation to pitolisant for the treatment of PWS. We expect to initiate the Phase 3 study in the first quarter of 2024. In

June 2021, we initiated a Phase 2 proof-of-concept signal detection clinical trial to evaluate pitolisant for the treatment of EDS, fatigue and cognitive dysfunction in adult patients with DM1 and announced topline results from this trial in the fourth quarter of 2023, in which clinically meaningful improvements were demonstrated in EDS and fatigue. The safety profile was consistent with the established safety profile of pitolisant.

Our partner, Bioprojet completed a Phase 3 trial in pediatric patients with narcolepsy and submitted the trial data to the European Medicines Agency (the "EMA") seeking approval for a pediatric narcolepsy indication. On January 26, 2023, Bioprojet received a positive opinion from the EMA's Committee for Medicinal Products for Human Use ("CHMP") and in March 2023, the EMA granted approval for the marketing authorization of WAKIX for the treatment of narcolepsy in children 6 and older. Based on the data from the positive Phase 3 trial conducted by Bioprojet, we submitted an sNDA for pediatric narcolepsy in December 2023. On February 21, 2024, we announced that the FDA has granted priority review of our pediatric narcolepsy sNDA and has set a Prescription Drug User Fee Act, or target action date, of June 21, 2024. We remain committed to obtaining pediatric exclusivity for WAKIX.

We also seek to expand our pipeline through the acquisition of additional assets that focus on addressing the unmet needs of patients living with rare neurological diseases as well as patients living with other neurological diseases who have unmet medical needs. We are targeting assets that will allow us to further leverage the expertise and infrastructure that we have successfully built at Harmony so we can optimize the benefit of internal synergies. Consistent with this objective, on July 31, 2022, we entered into a License and Commercialization Agreement (the "2022 LCA") with Bioprojet whereby we obtained exclusive rights to manufacture, use and commercialize one or more new products based on pitolisant in the United States and Latin America, with the potential to add additional indications and formulations upon the agreement of both parties. We have made progress in the development of two new formulations of pitolisant, Next Gen 1 ("NG1") and Next Gen 2 ("NG2"). Both formulations entered clinical studies in the fourth quarter of 2023 and we anticipate data in the first half of 2024.

In addition, on October 10, 2023, we completed a tender offer to acquire all of the outstanding shares of common stock of Zynerba Pharmaceuticals, Inc. (together with its subsidiary, Zynerba Pharmaceutical Pty, Ltd., "Zynerba") for $60.0 million, exclusive of transaction related costs of approximately $2.6 million. Zynerba is a clinical-stage pharmaceutical company focused on innovative pharmaceutically produced transdermal cannabidiol therapies for orphan neuropsychiatric disorders, including Fragile X syndrome ("FXS"). The phase 3 RECONNECT registration study in FXS is ongoing.

On August 4, 2021, we acquired HBS-102, a Melanin-concentrating hormone receptor 1 (MCHR1) antagonist previously developed as CSTI-100/ALB-127258(a)/ALB-127258 (the "Compound"), along with intellectual property and other assets related to the development, manufacture, and commercialization of the Compound from ConSynance Therapeutics, Inc. In connection with the acquisition, we made an upfront payment of $3.5 million and will be required to make certain payments upon the achievement of certain development milestones, regulatory milestones, and sales milestones and pay ongoing royalties upon commercialization. We acquired full development and commercialization rights for HBS-102 globally, but we have provided an indication-limited grant-back license to ConSynance for the development and commercialization of the Compound in Greater China. We are conducting a preclinical proof-of-concept study to assess the effect of HBS-102 on hyperphagia, weight gain and other metabolic parameters in a mouse model of PWS and are also conducting a thirteen-week toxicology study. We anticipate data from both studies in the first half of 2024.

Pitolisant was developed by Bioprojet and approved by the EMA in 2016 for the treatment of narcolepsy in adult patients with or without cataplexy and in 2021 for the treatment of EDS in adult patients with obstructive sleep apnea. We acquired an exclusive license to develop, manufacture and commercialize pitolisant in the United States pursuant to our license agreement with Bioprojet (as amended, the "2017 LCA") in July 2017. See "Part I—Item 1. Business.—Strategic Agreement—License and Commercialization Agreement with Bioprojet" for further information regarding the 2017 LCA. Pitolisant was granted Orphan Drug Designation for the treatment of narcolepsy by the FDA in 2010. It received Breakthrough Therapy designation for the treatment of cataplexy in patients with narcolepsy and Fast Track status for the treatment of EDS and cataplexy in patients with narcolepsy in April 2018.

Our operations are conducted by our wholly owned subsidiaries, Harmony Biosciences, LLC, which was formed in May 2017, and Zynerba.

Commercial Performance Metrics

As of December 31, 2023, we continued to see growth in the number of unique healthcare professional ("HCP") prescribers of WAKIX since it became available in November 2019. The average number of patients on WAKIX at the end of 2023 was approximately 6,150. Additionally, as of December 31, 2023, we have secured formulary access for more than 80% of all insured lives (Commercial, Medicare and Medicaid) in the United States. Within these covered lives, we have observed favorable access to WAKIX subsequent to the expanded approval of WAKIX for the treatment of cataplexy in adult patients with narcolepsy in October 2020.

Financial Operations Overview

Net Product Revenue

Net product revenue includes gross product shipments less provisions for sales discounts and allowances, which includes trade allowances, rebates to government and commercial entities, and other discounts. Although we expect net sales to increase over time, provisions for sales discounts and allowances may fluctuate based on the mix of sales to different customer segments and/or changes in our estimates. For further discussion of the components of Revenue, see "—Critical Accounting Policies and Significant Judgments and Estimates."

Cost of Product Sales

Cost of product sales includes manufacturing and distribution costs, the cost of API, FDA program fees, royalties due to third parties on net product sales, freight, shipping, handling, storage costs and salaries of employees involved with oversight of production. We expect the cost of product sales to increase as we continue to ramp up production in order to meet future demand for WAKIX and diversify our supply chain for WAKIX.

The shelf life of WAKIX is three years from date of manufacture, with the earliest expiration of current inventory expected to be May 2025. We regularly review our inventory levels and expect write-offs from time to time. We will continue to assess inventory levels in future periods as demand for WAKIX and the rate of inventory turnover evolves. We currently have adequate supply of WAKIX to cover demand into the second quarter of 2025, with additional API on-hand inventory to support at least 36 months beyond this time frame.

Research and Development Expenses

Research and development expenses primarily include development programs for potential new indications for pitolisant in patients with IH, PWS and DM. We also incur research and development expenses related to our team of Medical Science Liaisons ("MSLs") who interact with key opinion leaders, with a focus on the science, the role of histamine in sleep-wake state stability and the novel mechanism of action of pitolisant. In addition, our MSLs support our market access team with the presentation of clinical data to payors upon request and our clinical development team to identify potential clinical trial sites. Research and development costs are expensed as incurred. We have significantly increased our research and development efforts as we advance our clinical programs in IH, PWS and DM and assess other product candidates to expand our pipeline. Research and development expenses also include:

- employee-related expenses, such as salaries, share-based compensation, benefits and travel expenses for our research and development personnel;
- direct third-party costs such as expenses incurred under agreements with CROs, and contract manufacturing organizations ("CMOs");
- manufacturing costs in connection with producing materials for use in conducting clinical trials;
- costs related to packaging and labeling of clinical supplies;
- other third-party expenses (e.g., consultants, advisors) directly attributable to the development of our product candidates; and

- amortization expense for assets used in research and development activities.

We do not track research and development expenses on an indication-by-indication basis. A significant portion of our research and development costs are external costs, such as fees paid to CROs and CMOs, central laboratories, contractors, and consultants in connection with our clinical development programs. Internal expenses primarily relate to personnel who are deployed across multiple programs.

Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials, milestone payments, and the cost of submitting an NDA to the FDA (and/or other regulatory authorities). We expect our research and development expenses to be significant over the next several years as we advance our current clinical development programs and prepare to seek regulatory approval for additional indications for pitolisant, advance HBS-102 from preclinical studies into the clinic, and identify potential new product candidates to develop toward new indications.

At this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the development of any additional indications for pitolisant or other product candidates that we move forward for regulatory approval. There are numerous risks and uncertainties associated with developing product candidates, including uncertainty related to:

- the duration, costs and timing of clinical trials of our current development programs and any further clinical trials related to new product candidates;
- the sufficiency of our financial and other resources to complete the necessary preclinical studies and clinical trials;
- the impact of the COVID-19 pandemic, including any future resurgence or new variants, on the ability to initiate new clinical trials and/or maintain the continuity of ongoing clinical trials, including our ability to access sleep labs in order to conduct objective sleep testing, that could be impacted by future shelter-in-place orders and needs of the health care system to focus on managing patients affected by COVID-19;
- receiving Bioprojet's consent to pursue additional indications for pitolisant;
- the acceptance of INDs for our planned clinical trials or future clinical trials;
- the successful and timely enrollment and completion of clinical trials;
- the successful completion of preclinical studies and clinical trials;
- successful data from our clinical programs that support an acceptable risk-benefit profile of our product candidates in the intended populations;
- the receipt and maintenance of regulatory and marketing approvals from applicable regulatory authorities;
- establishing agreements with third-party manufacturers for clinical supply for our clinical trials and commercial manufacturing, if our product candidate is approved;
- the entry into collaborations to further the development of our product candidates;
- obtaining and maintaining patent and trade secret protection or regulatory exclusivity for our product candidates; and
- successfully launching our product candidates and achieving commercial sales, if and when approved.

A change in the outcome of any of these variables with respect to the development of any of our programs or any product candidate we develop would significantly change the costs, timing and viability associated with the development and/or regulatory approval of such programs or product candidates.

Sales and Marketing Expenses

Our sales and marketing expenses primarily relate to the market development and commercialization activities of WAKIX for the treatment of EDS and cataplexy in adult patients with narcolepsy. Market development and commercial activities account for a significant portion of our operating expenses and are expensed as incurred. We expect our sales and marketing expenses to increase in the near- and mid-term to support WAKIX's indications for the treatment of EDS or cataplexy in adult patients with narcolepsy and to expand our portfolio with the anticipated growth from potential additional indications.

Sales and marketing expenses include:

- employee-related expenses, such as salaries, share-based compensation, benefits and travel expenses for our sales, marketing and market access personnel;
- healthcare professional-related expenses, including marketing programs, healthcare professional promotional medical education, disease education, conference exhibits and market research;
- patient-related expenses, including patient awareness and education programs, disease awareness education, patient reimbursement programs, patient support services and market research;
- market access expenses, including payor education, specialty pharmacy programs and services to support the continued commercialization of WAKIX; and
- secondary data purchases (i.e., patient claims and prescription data), data warehouse development and data management.

In addition, sales and marketing expenses include external costs such as website development, media placement fees, agency fees for patient, medical education and promotional expenses, market research, analysis of secondary data, conference fees, and consulting fees.

General and Administrative Expenses

General and administrative expenses consist primarily of employee-related expenses, such as salaries, share-based compensation, benefits and travel expenses for our personnel in executive, legal, finance and accounting, human resources, investor relations, and other administrative departments. General and administrative expenses also consist of office leases, and professional fees, including legal, tax and accounting and consulting fees.

We anticipate that our general and administrative expenses will increase in the future to support our continued commercialization efforts, ongoing and future potential research and development activities, and increased costs of operating as a public company. These increases will likely be driven by costs associated with the hiring of additional personnel and fees paid to outside consultants, lawyers and accountants, among other expenses. Additionally, we anticipate increased costs associated with being a public company, including expenses related to services associated with maintaining compliance with the requirements of Nasdaq and the SEC, insurance and investor relations costs. If any of our current or future indication expansion programs or new product candidates obtain U.S. regulatory approval, we expect that we would incur significantly increased expenses associated with building a sales and marketing team.

Paragon Agreement

We are party to a right-of-use agreement with Paragon Biosciences, LLC ("Paragon") whereby we have access to and the right to use certain office space leased by Paragon in Chicago, Illinois. For the year ended December 31, 2023, we paid fees of $0.3 million pursuant to this agreement. Paragon is related party as they share common ownership with the Company and the Chairman of the Company's board of directors was the President and owner of the entity.

Loss on Debt Extinguishment

Loss on debt extinguishment consists primarily of costs of extinguishment of debt during the applicable period related to the prepayment of our credit agreements.

Interest Expense

Interest expense consists primarily of interest expense on debt facilities, amortization of debt issuance costs and amortization of premiums on our debt securities.

Interest Income

Interest income consists primarily of cash interest earned on our cash and investment balances and accretion of the discount on our investments in debt securities.

Results of Operations

The following table sets forth selected items in our consolidated statements of operations for the periods presented:

	Year Ended December 31,	
	2023	2022
	(In thousands)	
Net product revenue	$ 582,022	$ 437,855
Cost of product sales	121,236	83,481
Gross profit	460,786	354,374
Operating expenses:		
Research and development	76,063	70,886
Sales and marketing	97,404	79,285
General and administrative	95,289	84,017
Total operating expenses	268,756	234,188
Operating income	192,030	120,186
Loss on debt extinguishment	(9,766)	—
Other (expense) income, net	159	169
Interest expense	(23,757)	(18,795)
Interest income	14,730	3,126
Net income before provision for income taxes	173,396	104,686
Income tax (expense) benefit	(44,543)	76,782
Net income	$ 128,853	$ 181,468

Net Product Revenue

Net product revenue increased by $144.2 million, or 32.9%, for the year ended December 31, 2023, compared to the same period in 2022. The increase was primarily due to a 33.5% increase in units shipped, and the net impact of a 7.0% price increase which occurred in January 2023.

Cost of Product Sales

Cost of product sales increased by $37.8 million, or 45.2%, for the year ended December 31, 2023, compared to the same period in 2022. Cost of product sales as a percentage of net product revenue was 20.8% for the year ended December 31, 2023, compared to 19.1% for the year ended December 31, 2022. The increase in cost of product sales was due to higher royalties as a result of higher sales of WAKIX in the current year. Higher sales of WAKIX in the year ended

December 31, 2023, resulted in us entering a higher royalty tier earlier in 2023 compared to 2022, which also caused royalties to be higher in the current year.

Research and Development Expenses

Research and development expenses increased by $5.2 million, or 7.3%, for the year ended December 31, 2023 compared to the same period in 2022. The increase was primarily driven by a $22.9 million increase in clinical development work associated with PWS, ZYN002, IH, NG1/NG2 and DM1, a $4.0 million increase in personnel costs, a $1.3 million increase in stock compensation associated with new awards, a $3.8 million severance charge, and a $2.3 million IPR&D charge, both related to the acquisition of Zynerba, and a $0.8 million IPR&D charge related to preclinical milestones achieved for HBS-102, partially offset by a $30 million licensing fee incurred upon entering the 2022 LCA with Bioprojet during the year ended December 31, 2022.

Sales and Marketing Expenses

Sales and marketing expenses increased by $18.1 million, or 22.9%, for the year ended December 31, 2023 compared to the same period in 2022. The increase was primarily due to a $7.6 million increase in personnel costs, a $6.5 million increase in patient engagement and marketing activities, a $2.7 million increase in travel, and a $1.2 million increase in stock compensation associated with new awards. The increase in patient engagement and marketing activities for both comparable periods was driven by our continued growth of WAKIX and the increase in personnel costs for both comparable periods was related increased headcount and increased sales force incentives.

General and Administrative Expenses

General and administrative expenses increased by $11.3 million, or 13.4%, for the year ended December 31, 2023 compared to the same period in 2022. The increase was primarily due to a $4.1 million severance charge associated with the acquisition of Zynerba, a $2.9 million increase in personnel costs, a $1.9 million increase in legal and professional fees due to patent lawsuits, a $1.7 million increase to stock compensation associated with new awards and a $0.9 million increase in intangible asset amortization as a result of the $40.0 million milestone payment in March 2022 upon attaining $500.0 million in life-to-date aggregate net sales of WAKIX in the United States.

Loss on Debt Extinguishment

Loss on debt extinguishment was $9.8 million for the year ended December 31, 2023. There was no comparable amount in the prior year periods.

Interest Expense

Interest expense increased by $5.0 million, or 26.4%, for the year ended December 31, 2023 compared to the same period in 2022. The increase was primarily due to higher interest rates in 2023 compared to 2022 and $2.0 million in unamortized fees and ticking fees pertaining to the DDTL (defined below).

Interest Income

Interest income increased by $11.6 million, or 371.2%, for the year ended December 31, 2023 compared to the same period in 2022. The increase was primarily due to a $9.2 million increase in cash interest generated from our investments and cash equivalents and a $2.4 million increase in accretion of discount on our investments.

Income Taxes

Income tax expense was $44.5 million, representing a 25.7% effective tax rate, for the year ended December 31, 2023 compared to an income tax benefit of $76.8 million for the year ended December 31, 2022. The income tax benefit related to the year ended December 31, 2022 was due to the release of the valuation allowance on our deferred tax assets, partially offset by current period provision for state and federal income taxes. The effective tax rate of 25.7% for the year ended December 31, 2023 included 21.0% for the provision of federal income taxes and 5.7% for the provision of state income taxes, partially offset by a 2.1% benefit from research and development credits.

Liquidity, Sources of Funding and Capital Resources

Overview

As of December 31, 2023, we had cash, cash equivalents, and investments of $425.6 million and accumulated deficit of $143.3 million. As of December 31, 2023, we had outstanding debt of $196.3 million.

The consolidated financial statements have been prepared as though we will continue as a going concern, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business.

We believe that our existing cash, cash equivalents and investments on hand as of December 31, 2023, will enable us to meet our operational liquidity needs for the next 12 months. In addition, we plan to maintain disciplined capital allocation to maximize shareholder value, which includes investing in the expansion of our product portfolio and opportunistically executing on our share repurchase program to return value to shareholders. We have based our liquidity and cash flow projections on assumptions that may prove to be incorrect, and we could use our capital resources sooner than we expect.

Term Loan A Credit Agreement

On July 26, 2023, we entered into a Credit Agreement (the "TLA Credit Agreement") with JPMorgan Chase Bank, N.A., as "Administrative Agent", and certain lenders. The TLA Credit Agreement provides for a five-year senior secured term loan (the "TLA Term Loan") in an aggregate principal amount of $185.0 million.

On September 21, 2023, we entered into the First Incremental Amendment (the "First Incremental Amendment") with the Administrative Agent and Bank of America, N.A., as incremental lender. The First Incremental Amendment provides for an incremental senior secured term loan (the "Incremental Term Loan") in an aggregate principal amount of $15.0 million. The First Incremental Amendment amends the TLA Credit Agreement and provides that the Incremental Term Loan will have identical terms as the TLA Term Loan.

The repayment schedule for both the TLA Term Loan and the Incremental Term Loan (together, the "Term Loans") consists of $3.8 million quarterly principal payments, which commence on December 31, 2023, increasing to $5.0 million quarterly principal payments beginning on December 31, 2025, with a $115.0 million payment due on the maturity date of July 26, 2028. The Term Loans bear interest at a per annum rate equal to, at our option, (i) a base rate plus a specified margin ranging from 2.50% to 3.00%, based on our senior secured net leverage ratio (as defined in the TLA Credit Agreement) or (ii) Term SOFR plus a credit spread adjustment of 0.10% plus a specified margin ranging from 3.50% to 4.00%, based on our senior secured net leverage ratio.

The TLA Credit Agreement contains customary affirmative and negative covenants, financial covenants, representations and warranties, events of default and other provisions. We were in compliance with all covenants as of December 31, 2023.

Blackstone Credit Agreement

In August 2021, we entered into the Blackstone Credit Agreement that provided for (i) a senior secured term loan facility in an aggregate original principal amount of $200.0 million (the "Initial Term Loan") and (ii) a senior secured delayed draw term loan facility in an aggregate principal amount up to $100.0 million (the "DDTL" and, together with the Initial Term Loans, the "Loans"). The DDTL was initially available to draw down through August 9, 2022. In August 2022, we entered into an agreement to extend the expiration date of the DDTL to August 9, 2023, for which we will pay a ticking fee at a rate of 1% per annum on the undrawn portion of the DDTL, which commenced on August 10, 2022. We used substantially all of the proceeds from the Blackstone Credit Agreement, and the related sale of our common stock, to repay the balance of the OrbiMed Credit Agreement.

In connection with the TLA Credit Agreement, we fully extinguished the Blackstone Credit Agreement, which required a payoff amount of $207.3 million consisting of principal repayment, interest, exit fees, a ticking fee and a prepayment premium. We recognized a loss on extinguishment of debt of $9.8 million relating to the Blackstone Credit Agreement for the year ended December 31, 2023.We have no further obligations under the Blackstone Credit Agreement.

Share Repurchases

On August 1, 2023, our Board of Directors approved a program providing for the repurchase of shares of common stock in an aggregate amount of up to $125.0 million, excluding commissions and transaction fees. The repurchase program may be suspended, terminated or modified at any time for any reason. During the year ended December 31, 2023, we repurchased and retired 1,439,792 shares of common stock at an aggregate cost of approximately $50.0 million, excluding commissions and transaction fees.

On October 27, 2023, our Board of Directors terminated the August 2023 Repurchase Program and any remaining amount authorized for the repurchase of shares. Simultaneously, our Board of Directors approved a share repurchase program (the "October 2023 Repurchase Program") providing for the repurchase of shares of common stock in an aggregate amount of up to $200.0 million, excluding commissions and transaction fees. The October 2023 Repurchase Program may be suspended, terminated, or modified at any time for any reason. During the year ended December 31, 2023, the Company repurchased and retired 1,814,653 shares of common stock at an aggregate cost of $50.0 million under the October 2023 Repurchase Program, excluding commissions and transaction fees. As of December 31, 2023, the remaining amount of common stock authorized for repurchase was $150.0 million.

Acquisitions

On October 10, 2023, we completed a tender offer (the "Tender Offer") to acquire all of the outstanding shares of common stock of Zynerba Pharmaceuticals, Inc. ("Zynerba Common Stock").

Under the terms of the Tender Offer, we paid (i) $1.1059 per share of Zynerba Common Stock (the "Common Cash Amount"), plus (ii) one contingent value right (each, a "CVR") per share of Zynerba Common Stock (the "Common CVR Amount"), which represents the right to receive up to approximately $2.5444 per share of Zynerba Common Stock, subject to the achievement of certain clinical, regulatory and sales-based milestones. Both the Common Cash Amount and Common CVR Amount are to be paid in cash, subject to any applicable withholding of taxes and without interest. The aggregate consideration to acquire the Zynerba Common Stock upon completion of the Tender Offer was $60 million, excluding transaction related fees, which we paid using cash on hand.

Asset Purchase Agreement

In August 2021, we entered into the APA to acquire HBS-102, a potential first-in-class molecule with a novel mechanism of action. Under the terms of the agreement, the Company acquired full development and commercialization rights globally, with the exception of Greater China, for $3.5 million, which was recorded in research and development expense in the Company's statement of operations and comprehensive income (loss) for the year ended December 31, 2021. Additionally, there are payments due upon the achievement of certain milestones including $1.0 million for preclinical milestones, $19.0 million for development milestones, $44.0 million for regulatory milestones and $110.0 million for sales milestones.

License Agreement

In July 2022, we entered into the 2022 LCA with Bioprojet whereby we obtained exclusive rights to manufacture, use and commercialize one or more new products based on pitolisant in the United States and Latin America, with the potential to add additional indications and formulations upon the agreement of both parties. We paid an initial, non-refundable $30.0 million licensing fee in October 2022, and additional payments of up to $155.0 million are potentially due under the 2022 LCA upon the achievement of certain future development and sales-based milestones. In addition, certain payments will become due upon the achievement of development milestones for new indications and formulations as agreed upon by both parties. The 2022 LCA also includes a fixed trademark royalty and a tiered royalty payable on net sales of any new products commercialized, which will be payable to Bioprojet on a quarterly basis. The $30.0 million licensing fee was recorded in research and development expenses within the consolidated statement of operations and comprehensive income (loss) for the year ended December 31, 2022.

Recent Milestone Payments

In March 2023, we achieved a preclinical milestone, which triggered a $0.8 million payment under the provisions of the APA, which was paid in April 2023.

In March 2022, we made a final $40.0 million milestone payment to Bioprojet upon WAKIX attaining $500.0 million in life-to-date aggregate net sales in the United States.

Cash Flows

The following table sets forth a summary of our cash flows for the years ended December 31, 2023, and 2022:

	Year Ended December 31,	
	2023	2022
Selected cash flow data	(In thousands)	
Cash provided by (used in):		
Operating activities	$ 219,387	$ 144,466
Investing activities	(46,439)	(141,832)
Financing activities	(105,552)	6,841

Operating Activities

Net cash provided by operating activities for the year ended December 31, 2023, consisted of our net income of $128.9 million adjusted for non-cash items of $24.4 million related to intangible amortization and depreciation and $31.2 million related to stock-based compensation expense. Net working capital excluding cash decreased by $35.0 million.

Net cash provided by operating activities for the year ended December 31, 2022, consisted of our net income of $181.5 million adjusted for non-cash items of $23.4 million related to intangible amortization and depreciation and $26.9 million related to stock-based compensation expense. Net working capital excluding cash decreased by $4.1 million.

Investing Activities

Net cash used in investing activities for the year ended December 31, 2023, was $46.4 million, which was primarily attributable to $127.5 million in purchases of debt securities, $37.0 million related to the acquisition of Zynerba, net of cash received, and $0.3 million in purchases of property and equipment, partially offset by $118.3 million in proceeds from sales and maturities of investments.

Net cash used in investing activities for the year ended December 31, 2022, was $141.8 million, which was primarily attributable to $110.7 million in purchases of debt securities and a final $40.0 million initial payment associated with the 2017 LCA, partially offset by $9.1 million in proceeds from sales and maturities of investments.

Financing Activities

Net cash used in financing activities for the year ended December 31, 2023, was $105.6 million, which primarily consisted of $202.3 million in payments of principal and exit fees associated with the extinguishment of the Blackstone Credit Agreement, $100 million in share repurchases, $1.0 million in principal payments associated with the Blackstone Credit Agreement, and $3.8 million principal payments related to the TLA Credit Agreement, partially offset by $197.0 million in proceeds associated with the TLA Credit Agreement, net of issuance costs, and $5.1 million in proceeds from the exercise of employee stock options.

Net cash provided by financing activities for the year ended December 31, 2022 was $6.8 million, which primarily consisted of $8.8 million in proceeds from the exercise of employee stock options and stock issuances offset by $2.0 million in principal payments associated with the Blackstone Credit Agreement.

Critical Accounting Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States of America, or GAAP. The preparation of these financial statements requires us to make estimates, judgments and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities as of the dates of the balance sheets and the reported amounts of expenses during the reporting periods. In accordance with GAAP, we evaluate our estimates and judgments on an ongoing basis.

Significant estimates include assumptions used in the determination of the amount of revenue recognized on sales of WAKIX, costs incurred under services type agreements related to the performance of research and development activities, and the measurement of compensation expense pursuant to stock-based awards. We base our estimates on contractual terms, historical experience and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

We define our critical accounting policies as those under GAAP that require us to make subjective estimates and judgments about matters that are uncertain and are likely to have a material impact on our financial condition and results of operations, as well as the specific manner in which we apply those principles. While our accounting policies are more fully described in Note 3 to our consolidated financial statements included herein under "Part II—Item 8. Financial Statements and Supplementary Data", we believe the following are the critical accounting policies used in the preparation of our consolidated financial statements that require significant estimates and judgments.

Revenue Recognition

We account for contracts with our customers in accordance with ASC 606, Revenue from Contracts with Customers (ASC 606), or ASC 606. Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of ASC 606, the entity performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. We only apply the five-step model to contracts when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services it transfers to the customer. The amount of variable consideration which is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized under contracts will not occur in a future period. Our analyses contemplate the application of the constraint in accordance with ASC 606. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from our estimates, we will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.

We have determined that the delivery of our product to our customer constitutes a single performance obligation as there are no other promises to deliver goods or services in contracts with our customers. Shipping and handling activities are considered to be fulfilment activities and are not considered to be a separate performance obligation. The payment terms with our customers do not exceed one year and therefore, no amount of consideration has been allocated as a financing component. Taxes collected related to product sales are remitted to governmental authorities and are excluded from revenue.

Product Sales, Net

We recognize revenue on sales of WAKIX when the customer obtains control of the product, which occurs at a point in time, typically upon delivery. Product revenue is recorded at the product's list price, net of reserves for variable consideration that is offered within contracts between us and our customers, payors, and other indirect customers relating to the sale of WAKIX. Components of variable consideration include government (as detailed below) and commercial rebates, commercial co-payment assistance and distribution service fees. These deductions are based on the amounts earned, or to be claimed on the related sales, and are classified as a current liability or reduction of receivables in our consolidated balance sheet.

Government Contracts

We have entered into contracts (i) to participate in the Medicaid Drug Rebate Program and the Medicare Part D program, and (ii) to sell to the U.S. Department of Veterans Affairs, 340b entities and other government agencies, or government payors, so that WAKIX will be eligible for purchase by, in partial or full reimbursement from, such government payors. We record reserves for rebates due pursuant to these contracts as a reduction of revenue in the same period in which the revenue is recognized. The liability for government rebates is included in accrued expenses in our consolidated balance sheet.

We estimate rebates due pursuant to government contracts based upon our historical payment trends, information obtained from third parties estimating current payment trends, the government-mandated discounts applicable to government-funded programs, as well as information obtained from our customers. The liability for these government rebates consists of estimates of claims for WAKIX dispensed in the current period, plus an estimate for product which has shipped and has been recognized as revenue but remains in the distribution channel at the end of a reporting period.

Research and Development Expenses

We base our expenses for research and development services rendered on estimates of the timing of services received and the total cost of those services pursuant to quotes, contracts and communicating with our vendors. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payments. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. In accruing service fees, we estimate the time period over which services will be performed and the total costs of the services to be performed. If the actual timing of the performance of services, or the total cost of those services is materially different from our estimates, we adjust the accrual or amount of prepaid or accrued expenses accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in us reporting amounts that are too high or too low in any particular period. To date, we have not made any material adjustments to our prior estimates of research and development expenses.

Stock-Based Compensation

The Company recognizes stock-based compensation expense in operating results using a fair value measurement method, in accordance with FASB ASC 718, Compensation-Stock Compensation. ASC 718 requires all stock-based payments to employees to be recognized in operating results as compensation expense based on fair value over the requisite service period of the awards. The vesting periods have a time-based provision consisting of one to five years and expire no more than 10 years after the date of grant. Upon a change of control, certain unvested awards will immediately vest. The Company determines the fair value of stock-based awards using the Black-Scholes option-pricing model, which uses both historical and current market data to estimate fair value. The method incorporates various assumptions, such as the risk-free interest rate, expected volatility, expected dividend yield, and expected life of the options.

Income Taxes

Income taxes are accounted for under the asset and liability method. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carryforwards. Deferred tax assets and liabilities are measured using enacted rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment date.

Deferred tax assets may be reduced by a valuation allowance if, based on all available evidence, it is more likely than not that some portion or all of the deferred income tax assets will not be realized. Management judgment is required in determining the period in which a reversal of a valuation allowance should occur. We are required to consider all available evidence, both positive and negative, such as historical levels of income and future forecasts of taxable income among other items, in determining whether a full or partial release of its valuation allowance is required. Our accounting for deferred tax consequences represents the best estimate of those future events. We present deferred income taxes on the consolidated balance sheet on a jurisdictional basis as either a net noncurrent asset or liability.

We record liabilities for uncertain tax positions based on a two-step approach. The first step is recognition, where we evaluate whether an individual tax position has a likelihood of greater than 50% of being sustained upon examination based solely on the technical merits of the position, including resolution of any related appeals or litigation processes. For tax positions that are currently estimated to have less than a 50% likelihood of being sustained, no tax benefit is recorded. For tax positions that have met the recognition threshold in the first step, we perform the second step of the approach of measuring the benefit (expense) to be recorded. The actual benefits (expense) ultimately realized may differ from our estimates. In future periods, changes in facts, circumstances, and new information may require us to change the recognition and measurement estimates with regard to individual tax positions. Changes in recognition and measurement estimates are recorded in the statement of income and balance sheet in the period in which such changes occur. As of December 31, 2023, we did not have any liabilities for unrecognized tax positions. As it relates to any interest and penalties associated with any uncertain tax positions, our position is to include those interest and penalties as a component of income tax expense.

Business Combinations

We account for business combinations and asset acquisitions in accordance with FASB ASC 805 Business Combinations. The Zynerba Acquisition was accounted for as an asset acquisition under ASC Topic 805, Business Combinations, because substantially all of the fair value of the gross assets acquired was concentrated in a single identifiable IPR&D asset, ZYN002, Zynerba's lead asset. ZYN002 is the first and only pharmaceutically manufactured, synthetic cannabidiol, a non-euphoric cannabidiol, formulated as a patent-protected permeation-enhanced gel for transdermal delivery through the skin and into the circulatory system and is currently in Phase III clinical trial for the potential treatment of Fragile X Syndrome. The Company recognized the acquired assets and assumed liabilities based on the consideration paid, including transaction costs, on a relative fair value basis, and after first allocating the preliminary excess of the fair value of net assets acquired over the purchase price consideration to certain qualifying assets, principally, the IPR&D asset. In accordance with the accounting for asset acquisitions, an entity that acquires IPR&D assets in an asset acquisition follows the guidance in ASC Topic 730 Research and Development, which requires that both tangible and intangible identifiable research and development assets with no alternative future use be allocated a portion of the consideration transferred and recorded as research and development expense at the acquisition date.

Recent Accounting Pronouncements

See Note 3 to our consolidated financial statements included herein under "Part II—Item 8. Financial Statements and Supplementary Data." for more information.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

Interest Rate Fluctuation Risk

We are exposed to market risk related to changes in interest rates. We invest a portion of our cash in investment-grade, interest-bearing securities. The primary objectives of our investment activities are to preserve principal, maintain liquidity and maximize total return. In order to achieve these objectives, we invest in money market funds, U.S. government and agency securities, corporate bonds and commercial paper in accordance with our investment policy. Our investment policy defines allowable investments and establishes guidelines relating to credit quality, diversification, and maturities of our investments to preserve principal and maintain liquidity. All investment securities have a credit rating of at least A-2/P-2/F2 from at least two National Recognized Statistical Rating Organizations. We do not have any direct investments in asset-backed securities, collateralized debt or loan obligations, or structured investment vehicles. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. Based on our $358.5 million of investments in money market funds, U.S. treasury notes, corporate bonds and municipal obligations as of December 31, 2023, an immediate 10% change in market interest rates would not have a material impact on the fair market value of our investment portfolio or on our financial position or results of operations.

As of December 31, 2023, we had $196.3 million in borrowings outstanding. The Term Loans bear interest at a per annum rate equal to, at our option, (i) a base rate plus a specified margin ranging from 2.50% to 3.00%, based on our senior secured net leverage ratio (as defined in the TLA Credit Agreement) or (ii) Term SOFR plus a credit spread adjustment of 0.10% plus a specified margin ranging from 3.50% to 4.00%, based on our senior secured net leverage ratio. Based on the

$196.3 million of principal outstanding as of December 31, 2023, an immediate 10% change in the SOFR would not have a material impact on our debt-related obligations, financial position or results of operations.

Foreign Currency Fluctuation Risk

We are not currently exposed to significant market risk related to changes in foreign currency exchange rates; however, we have contracted with and may continue to contract with foreign vendors that are located in Europe and Australia. Our operations may be subject to fluctuations in foreign currency exchange rates in the future.

Inflation Fluctuation Risk

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation had a material effect on our business, financial condition or results of operations for the years ended December 31, 2023, and 2022.

Item 8. Financial Statements and Supplementary Data.

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the shareholders and the Board of Directors of Harmony Biosciences Holdings, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Harmony Biosciences Holdings, Inc. and subsidiaries (the "Company") as of December 31, 2023 and 2022, the related consolidated statements of operations and comprehensive income (loss), stockholders' equity (deficit), and cash flows for each of the three years in the period ended December 31, 2023, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2023 and 2022, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2023, in conformity with accounting principles generally accepted in the United States of America.

We have also audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2023, based on criteria established in Internal Control — Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission and our report dated February 22, 2024, expressed an unqualified opinion on the Company's internal control over financial reporting.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matters

The critical audit matters communicated below are matters arising from the current-period audit of the financial statements that were communicated or required to be communicated to the audit committee and that (1) relate to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of critical audit matters does not alter in any way our opinion on the financial statements, taken as a whole, and we are not, by communicating the critical audit matters below, providing separate opinions on the critical audit matters or on the accounts or disclosures to which they relate.

Rebate Accrual for Medicaid – Refer to "Note 3 – Product Sales, Net" to the financial statements

Critical Audit Matter Description

As more fully disclosed in Note 3 to the financial statements, the Company recognizes revenue on sales of WAKIX when the customer obtains control of the product, which occurs at a point in time, typically upon delivery. Product revenue is recorded at the product's wholesale acquisition costs, net of applicable reserves for variable consideration that are offered within contracts between the Company and its customers, payors, and other indirect customers relating to the sale of WAKIX. Components of variable consideration include government and commercial contracts, product returns, commercial co-payment assistance program transactions, and distribution service fees.

The rebate provision and related liability related to the Medicaid Drug Rebate Program (the "Medicaid rebate accrual") involves the use of significant assumptions and judgments in the Company's calculation. These significant assumptions and judgments include consideration of legal interpretations of applicable laws and regulations, historical claims experience, payer channel mix, current contract prices, unbilled claims, claims submission time lags, and inventory levels in the distribution channel.

Given the complexity involved in determining the significant assumptions used in calculating the Medicaid rebate accrual, auditing these estimates involved especially subjective judgment.

How the Critical Audit Matter Was Addressed in the Audit

Our audit procedures related to the Medicaid rebate accrual included the following, among others:

- We evaluated the appropriateness and consistency of the Company's methods and assumptions used to calculate the Medicaid rebate accrual.
- We tested the effectiveness of internal controls over the review of the Company's estimation model, including underlying assumptions and key inputs into the Company's process to calculate the Medicaid rebate accrual.
- We tested the mathematical accuracy of the Medicaid rebate accrual.
- We tested significant assumptions and key inputs used to calculate the Medicaid rebate accrual.
- We evaluated the Company's ability to estimate the Medicaid rebate accrual accurately by comparing actual amounts incurred for the Medicaid rebate accrual to historical estimates.
- We tested the overall reasonableness of the Medicaid rebate accrual recorded at period end by developing an expectation for comparison to actual recorded balances.

Accounting Treatment of the Asset Acquisition – Refer to "Note 4 – Acquisition" to the financial statements

Critical Audit Matter Description

As described further in Note 4 to the financial statements, during the year ended December 31, 2023, the Company completed an asset acquisition for which substantially all of the fair value of the gross assets acquired was concentrated in a single identifiable in-process research and development ("IPR&D") asset, ZYN002. The transaction was accounted for as an asset acquisition, and as such, the total consideration was allocated to the acquired asset based upon its relative fair value. The Company utilized a third-party valuation specialist to assist in the determination of the fair value of the asset acquired. The methods used to estimate the fair value involved significant assumptions. We identified the accounting for the asset acquisition versus a business combination and the valuation of the asset as a critical audit matter.

The principal considerations for our determination that the accounting for the transaction as an asset acquisition versus a business combination and valuation of the asset acquisition is a critical audit matter included (i) the significant judgment by the Company in the interpretation and application of the relevant accounting literature to conclude on asset acquisition; (ii) a high degree of auditor judgment, subjectivity, and effort in performing procedures and evaluating audit evidence related to the Company's fair value estimate of the asset acquired, and (iii) the audit effort involved the use of professionals with specialized skill and knowledge. Given the complexity involved in interpreting the guidance for the accounting treatment of the asset acquisition, auditing these estimates involved especially subjective judgment.

How the Critical Audit Matter Was Addressed in the Audit

Our audit procedures related to the audit considerations of the asset acquisition included the following, among others:

- We evaluated the underlying terms of the Agreement and Plan of Merger and the Company's accounting memoranda, including application of the relevant accounting guidance.

- With the assistance of internal subject matter experts, we evaluated the appropriateness and consistency of the Company's application of accounting literature related to the transaction.

- We tested the effectiveness of internal controls over the Company's asset acquisition evaluation, including those over the determination of the fair value of IPR&D.

- With the assistance of fair value specialists, we evaluated the appropriateness of the Company's methodology used to estimate the fair value of the IPR&D asset and the significant assumptions utilized.

/s/ Deloitte & Touche LLP

Philadelphia, PA
February 22, 2024

We have served as the Company's auditor since 2017.

HARMONY BIOSCIENCES HOLDINGS, INC. AND SUBSIDIARIES
CONSOLIDATED BALANCE SHEETS
(In thousands, except share and per share data)

	December 31, 2023	December 31, 2022
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 311,660	$ 243,784
Investments, short-term	41,800	79,331
Trade receivables, net	74,140	54,740
Inventory, net	5,363	4,297
Prepaid expenses	12,570	9,347
Other current assets	5,537	8,786
Total current assets	451,070	400,285
NONCURRENT ASSETS:		
Property and equipment, net	371	573
Restricted cash	270	750
Investments, long-term	72,169	22,568
Intangible assets, net	137,108	160,953
Deferred tax asset	144,162	85,943
Other noncurrent assets	6,298	2,798
Total noncurrent assets	360,378	273,585
TOTAL ASSETS	$ 811,448	$ 673,870
LIABILITIES AND STOCKHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Trade payables	$ 17,730	$ 3,786
Accrued compensation	23,747	11,532
Accrued expenses	99,494	59,942
Current portion of long-term debt	15,000	2,000
Other current liabilities	7,810	1,624
Total current liabilities	163,781	78,884
NONCURRENT LIABILITIES:		
Long-term debt, net	178,566	189,647
Other noncurrent liabilities	2,109	2,501
Total noncurrent liabilities	180,675	192,148
TOTAL LIABILITIES	344,456	271,032
COMMITMENTS AND CONTINGENCIES (Note 13)		
STOCKHOLDERS' EQUITY:		
Common stock—$0.00001 par value; 500,000,000 shares authorized at December 31, 2023 and December 31, 2022, respectively; 56,769,081 and 59,615,731 shares issued and outstanding at December 31, 2023 and December 31, 2022, respectively	1	1
Additional paid in capital	610,266	675,118
Accumulated other comprehensive (loss) income	2	(151)
Accumulated deficit	(143,277)	(272,130)
TOTAL STOCKHOLDERS' EQUITY	466,992	402,838
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	$ 811,448	$ 673,870

The accompanying notes are an integral part of the consolidated financial statements

HARMONY BIOSCIENCES HOLDINGS, INC. AND SUBSIDIARIES CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE INCOME (LOSS) (In thousands, except share and per share data)

	Year Ended December 31,		
	2023	2022	2021
Net product revenue	$ 582,022	$ 437,855	$ 305,440
Cost of product sold	121,236	83,481	55,518
Gross profit	460,786	354,374	249,922
Operating expenses:			
Research and development	76,063	70,886	30,367
Sales and marketing	97,404	79,285	68,118
General and administrative	95,289	84,017	63,909
Total operating expenses	268,756	234,188	162,394
Operating income	192,030	120,186	87,528
Loss on debt extinguishment	(9,766)	—	(26,146)
Other (expense) income, net	159	169	16
Interest expense	(23,757)	(18,795)	(24,194)
Interest income	14,730	3,126	224
Income before income taxes	173,396	104,686	37,428
Income tax (expense) benefit	(44,543)	76,782	(2,831)
Net income	$ 128,853	$ 181,468	$ 34,597
Unrealized income (loss) on investments	153	(151)	—
Comprehensive income	$ 129,006	$ 181,317	$ 34,597
EARNINGS PER SHARE:			
Basic	$ 2.17	$ 3.07	$ 0.60
Diluted	$ 2.13	$ 2.97	$ 0.58
Weighted average number of shares of common stock - basic	59,469,648	59,173,121	57,531,540
Weighted average number of shares of common stock - diluted	60,372,397	61,097,045	59,205,213

The accompanying notes are an integral part of the consolidated financial statements

HARMONY BIOSCIENCES HOLDINGS, INC. AND SUBSIDIARIES CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY (DEFICIT)
(In thousands, except share and per share data)

	Common Stock		Additional paid-in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total stockholders' equity (deficit)
	Shares (1)	Amount				
Balance as of December 31, 2020	56,890,569	1	585,374	—	(488,195)	97,180
Net income	—	—	—	—	34,597	34,597
Issuance of common stock	1,270,462	—	29,700	—	—	29,700
Exercise of options	664,738	—	9,371	—	—	9,371
Stock-based compensation	—	—	15,659	—	—	15,659
Balance as of December 31, 2021	58,825,769	$ 1	$ 640,104	$ —	$ (453,598)	$ 186,507
Net income	—	—	—	—	181,468	181,468
Unrealized loss on investments	—	—	—	(151)	—	(151)
Issuance of common stock	8,050	—	408	—	—	408
Exercise of options	781,912	—	8,433	—	—	8,433
Stock-based compensation	—	—	26,173	—	—	26,173
Balance as of December 31, 2022	59,615,731	$ 1	$ 675,118	$ (151)	$ (272,130)	$ 402,838
Net income	—	—	—	—	128,853	128,853
Unrealized gain on investments	—	—	—	153	—	153
Repurchase of common stock	(3,254,445)	—	(101,097)	—	—	(101,097)
Exercise of options and vesting of restricted stock units (2)	407,795	—	4,540	—	—	4,540
Stock-based compensation	—	—	31,705	—	—	31,705
Balance as of December 31, 2023	56,769,081	$ 1	$ 610,266	$ 2	$ (143,277)	$ 466,992

(1) Common stock of Harmony Biosciences Holdings, Inc.
(2) Inclusive of fees and 1% excise tax on shares repurchased.

The accompanying notes are an integral part of the consolidated financial statements

HARMONY BIOSCIENCES HOLDINGS, INC. AND SUBSIDIARIES
CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands, except share and per share data)

	Year Ended December 31,		
	2023	2022	2021
CASH FLOWS FROM OPERATING ACTIVITIES			
Net income	$ 128,853	$ 181,468	$ 34,597
Adjustments to reconcile net income to net cash used in operating activities:			
Depreciation	514	419	416
Intangible amortization	23,845	22,966	18,424
Acquired in-process research & development (IPR&D) expense	2,260	—	—
Stock-based and employee stock purchase compensation expense	31,705	26,173	15,659
Stock appreciation rights market adjustment	(499)	732	446
Debt issuance costs amortization	2,274	1,663	2,238
Deferred taxes	(13,419)	(85,943)	—
Amortization of premiums and accretion of discounts on Investment securities	(2,715)	(432)	—
Loss on debt extinguishment	9,766	—	26,146
Other non-cash expenses	1,768	1,526	—
Change in operating assets and liabilities:			
Trade receivables	(19,400)	(19,897)	(12,667)
Inventory	(1,066)	135	(609)
Prepaid expenses and other assets	74	(7,548)	(1,655)
Trade payables	8,949	2,785	(1,555)
Accrued expenses and other current liabilities	46,479	20,570	17,263
Other non-current liabilities	(1)	(151)	(146)
Net cash provided by operating activities	219,387	144,466	98,557
CASH FLOWS FROM INVESTING ACTIVITIES:			
Purchase of investment securities	(127,469)	(110,729)	—
Proceeds from maturities and sales of investment securities	118,309	9,069	—
Purchase of property and equipment	(312)	(172)	(298)
Acquisition of Zynerba Pharmaceuticals, Inc., net of cash acquired	(36,967)	—	—
Milestone payments	—	(40,000)	(100,000)
Net cash used in investing activities	(46,439)	(141,832)	(100,298)
CASH FLOWS FROM FINANCING ACTIVITIES:			
Proceeds from issuance of common stock	—	—	30,000
Common stock issuance costs	—	—	(300)
Proceeds from issuance of debt	200,000	—	200,000
Debt issuance costs	(2,997)	—	(9,152)
Extinguishment of debt	(196,500)	—	(200,000)
Extinguishment of debt exit fees	(5,846)	—	(22,000)
Principal repayment of long term debt	(4,750)	(2,000)	(500)
Repurchase of common stock	(100,000)	—	—
Payments of employee withholding taxes related to stock-based awards	(514)	—	—
Proceeds from exercised options	5,055	8,841	9,371
Net cash (used in) provided by financing activities	(105,552)	6,841	7,419
NET INCREASE IN CASH, CASH EQUIVALENTS, AND RESTRICTED CASH	67,396	9,475	5,678
CASH, CASH EQUIVALENTS, AND RESTRICTED CASH—Beginning of period	244,534	235,059	229,381
CASH, CASH EQUIVALENTS, AND RESTRICTED CASH—End of period	$ 311,930	$ 244,534	235,059
Supplemental Disclosure of Cash Flow Information:			
Cash paid during the year for interest	$ 20,051	$ 16,364	$ 19,830
Cash paid during the year for taxes	48,233	12,645	2,875

The accompanying notes are an integral part of the consolidated financial statements

1. ORGANIZATION AND DESCRIPTION OF BUSINESS

The Company

Harmony Biosciences Holdings, Inc., and its consolidated subsidiaries (the "Company") was founded in July 2017 as Harmony Biosciences II, LLC, a Delaware limited liability company. The Company converted to a Delaware corporation named Harmony Biosciences II, Inc. in September 2017 and, in February 2020, the Company changed its name to Harmony Biosciences Holdings, Inc. The Company's operations are conducted in its wholly owned subsidiary, Harmony Biosciences, LLC ("Harmony"), which was formed in May 2017. The Company is a commercial-stage pharmaceutical company focused on developing and commercializing innovative therapies for patients living with rare neurological disorders as well as patients living with other neurological diseases who have unmet medical needs. The Company is headquartered in Plymouth Meeting, Pennsylvania.

On October 10, 2023, the Company completed a tender offer to acquire all of the outstanding shares of common stock of Zynerba Pharmaceuticals, Inc. (together with its subsidiary, Zynerba Pharmaceutical Pty, Ltd., "Zynerba"). Zynerba is a clinical-stage pharmaceutical company focused on innovative pharmaceutically produced transdermal cannabidiol therapies for orphan neuropsychiatric disorders, including Fragile X syndrome.

2. LIQUIDITY AND CAPITAL RESOURCES

The consolidated financial statements have been prepared as though the Company will continue as a going concern, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business. The Company had an accumulated deficit of $143,277 and $272,130, as of December 31, 2023, and 2022, respectively. As of December 31, 2023, the Company had cash, cash equivalents and investments of $425,629.

The Company believes that its existing cash, cash equivalents and investments on hand as of December 31, 2023, as well as additional cash generated from operating and financing activities will meet its operational liquidity needs and fund its planned investing activities for the next twelve months from the date of issuance of these consolidated financial statements.

3. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Basis of Presentation

The consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP") and include all adjustments necessary for the fair presentation of the Company's financial position for the periods presented. All intercompany accounts and transactions have been eliminated.

Significant Risks and Uncertainties

The Company's operations are subject to a number of factors that can affect its operating results and financial condition. Such factors include, but are not limited to, the results of clinical testing and trial activities of the Company's product candidates; the Company's ability to obtain regulatory approval to market its products; competition from products manufactured and sold or being developed by other companies; the price of, and demand for, the Company's products, if approved; the Company's ability to negotiate favorable licensing or other manufacturing and marketing agreements for its product candidates; and the Company's ability to raise capital.

The Company currently has one commercially approved product, WAKIX, and there can be no assurance that the Company's research and development efforts will result in successfully commercialized products in addition to WAKIX. Developing and commercializing a product requires significant time and capital and is subject to regulatory review and approval as well as competition from other biotechnology and pharmaceutical companies. The Company operates in an environment of rapid change and is dependent upon the continued services of its employees and consultants and obtaining and protecting intellectual property.

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts and disclosures in the consolidated financial statements, including the notes thereto, and elsewhere in this report. Actual results may differ significantly from estimates, which include rebates due pursuant to commercial and government contracts, accrued research and development expenses, stock-based compensation expense and income taxes.

Operating Segments

The Company holds all its tangible assets, conducts its operations, and generates its revenue in the U.S. Operating segments which are defined as components of an entity for which separate financial information is available and that is regularly reviewed by the Chief Operating Decision Makers in deciding how to allocate resources to an individual segment and in assessing performance. The Company has determined it operates in a single operating segment and has one reportable segment.

Fair Value of Financial Instruments

The Company's consolidated financial statements include cash, cash equivalents, accounts payable, and accrued liabilities, all of which are short term in nature and, accordingly, approximate fair value. Additionally, prior to the IPO, the Company's consolidated financial statements included a warrant liability that was carried at fair value and was re-measured at each balance sheet date until it would be exercised or expired. In connection with the IPO, the Warrants were re-evaluated under the Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") 480, *Distinguishing Liabilities from Equity,* and reclassified to equity. See Note 16 for a further discussion of the warrants.

It is the Company's policy to measure non-financial assets and liabilities at fair value on a nonrecurring basis. These non-financial assets and liabilities are not measured at fair value on an ongoing basis but are subject to fair value adjustments in certain circumstances (such as evidence of impairment), which, if material, are disclosed in the accompanying footnotes.

The Company measures certain assets and liabilities at fair value based on the fair value hierarchy that prioritizes inputs to valuation techniques used to measure fair value into three levels based on the source of inputs as follows:

Level 1—Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities.

Level 2—Valuations based on observable inputs and quoted prices in active markets for similar assets and liabilities.

Level 3—Valuations based on unobservable inputs and models that are supported by little or no market activity.

Money market funds are classified as Level 1 fair value instruments. Investments in available-for-sale debt securities are classified as Level 2 and carried at fair value, which we estimate utilizing a third-party pricing service. The pricing service utilizes industry standard valuation models whereby all significant inputs, including benchmark yields, reported trades, broker/dealer quotes, issuer spreads, bids, offers, or other market-related data, are observable. We validate valuations obtained from third-party services by obtaining market values from other pricing sources. The Company did not classify any assets or liabilities as Level 3 as of December 31, 2023, or December 31, 2022.

Cash, Cash Equivalents and Restricted Cash

Cash and cash equivalents and restricted cash consist of cash and, if applicable, highly liquid investments with an original maturity of three months or less when purchased, including investments in money market funds and debt securities

that approximate fair value. The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the balance sheet and in the statements of cash flows.

	As of	
	December 31, 2023	December 31, 2022
Cash and cash equivalents	$ 311,660	$ 243,784
Restricted cash	270	750
Total cash, cash equivalents, and restricted cash shown in the statements of cash flows	$ 311,930	$ 244,534

Restricted cash includes amounts required to be held as a security deposit in the form of letters of credit for the Company's credit card program and the fleet program.

Investments

The Company's investments consist of debt securities that are classified as available-for-sale. Short-term and long-term investments are carried at fair value and unrealized gains and losses are recorded as a component of accumulated comprehensive income in stockholders' equity. The amortization of premiums and accretion of discounts adjust the carrying value of investments and are recorded in interest expense, net, on the unaudited condensed consolidated statements of operations and comprehensive income. Interest income and realized gains and losses, if any, are also recorded in interest expense, net, on the unaudited condensed consolidated statement of operations and comprehensive income. Realized gains and losses that result from the sale of investments are determined on a specific identification basis.

At each reporting period, the Company reviews any unrealized losses position to determine if the decline in the fair value of the underlying investments is a result of credit losses or other factors. If the assessment indicates that a credit loss exists, any impairment is recognized as an allowance for credit losses in our consolidated statement of operations.

Concentrations of Risk

Substantially all of the Company's cash and money market funds are held in five financial institutions. Due to their size, the Company believes these financial institutions represent minimal credit risk. Deposits may exceed the amount of insurance provided on such deposits by the Federal Deposit Insurance Corporation for U.S. institutions. The Company has not experienced any losses on its deposits of cash and cash equivalents. The Company believes that it is not exposed to significant credit risk due to the financial position of the depository institutions in which those deposits are held.

The Company is subject to credit risk from its trade receivables related to its product sales. The Company extends credit to specialty pharmaceutical distribution companies within the United States. Customer creditworthiness is monitored and collateral is not required. Historically, the Company has not experienced credit losses on its accounts receivable. The Company monitors its exposure within accounts receivable and would record a reserve against uncollectible accounts receivable if necessary. As of December 31, 2023, three customers accounted for 100% of gross accounts receivable, Accredo Health Group, Inc. ("Accredo"), which accounted for 39% of gross accounts receivable; Caremark LLC ("CVS Caremark"), which accounted for 32% of gross accounts receivable; and PANTHERX Specialty Pharmacy LLC ("Pantherx), which accounted for 29% of gross accounts receivable. As of December 31, 2022, three customers accounted for 100% of gross accounts receivable; CVS Caremark, which accounted for 41% of gross accounts receivable, Accredo, which accounted for 35% of gross accounts receivable and Pantherx, which accounted for 24% of gross accounts receivable.

For the year ended December 31, 2023, three customers accounted for 100% of gross product revenue; CVS Caremark accounted for 37% of gross product revenue; Accredo accounted for 32% of gross product revenue; and Pantherx accounted for 31% of gross product revenue. For the year ended December 31, 2022, three customers accounted for 100% of gross product revenue; CVS Caremark accounted for 42% of gross product revenue; Pantherx accounted for 29% of gross product revenue; and Accredo accounted for 29% of gross product revenue. For the year ended December 31, 2021, three customers accounted for 100% of gross product revenue; CVS Caremark accounted for 36% of gross product revenue; Pantherx accounted for 35% of gross products revenue; and Accredo accounted for 29% of gross product revenue.

The Company depends on a single source supplier for each of its product and active pharmaceutical ingredient.

Share Repurchases

The Company accounts for share repurchases as constructive retirements, whereby it reduces common stock and additional paid-in capital by the amount of the original issuance, with any excess purchase price recorded as a reduction to

retained earnings. Under this method, issued and outstanding shares of common stock are reduced by the amount of shares of common stock repurchased, and no treasury stock is recognized on the condensed consolidated financial statements.

Inventory

Inventory is valued at the lower of cost or net realizable value. Cost is determined using the specific identification method for all inventory. Our policy is to write down inventory that has become obsolete, that has a cost basis in excess of its expected net realizable value and/or that we have quantities in excess of expected future demand. The estimate of excess quantities is subjective and primarily dependent on our estimates of future demand. If our estimate of future demand changes, we consider the impact on the reserve for excess inventory and adjust the reserve as required. Inventory reserves are recorded as a component of cost of product sales in our consolidated statement of operations.

We may capitalize inventory costs associated with products prior to regulatory approval when future commercialization is probable. Otherwise, such costs are expensed as research and development. The determination to capitalize inventory costs is based on various factors, including status and expectations of the regulatory approval process, any known safety or efficacy concerns, potential labeling restrictions, and any other impediments to obtaining regulatory approval.

Property and Equipment

Property and equipment are stated at cost less accumulated depreciation. Depreciation is computed using the straight-line method over the estimated useful lives of the assets, generally between three and ten years. Leasehold improvements are depreciated using the straight-line method over the shorter of the estimated useful life of the asset or the term of the lease. The Company's leasehold improvements primarily relate to its corporate headquarters in Plymouth Meeting, PA, and are generally being depreciated through the end of the lease term in May 2024. When assets are retired or otherwise disposed of, the cost and accumulated depreciation are removed from the balance sheet and any resulting gain or loss is reflected in the consolidated statement of operations and comprehensive income (loss) in the period realized.

Intangible Assets

Intangible assets with finite useful lives consist primarily of purchased developed technology and are amortized on a straight-line basis over their estimated useful lives. The estimated useful lives associated with finite-lived intangible assets are consistent with the estimated lives of the associated products and may be modified when circumstances warrant. Such assets are reviewed for impairment when events or circumstances indicate that the carrying value of an asset may not be recoverable. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of an asset and its eventual disposition are less than its carrying amount. The amount of any impairment is measured as the difference between the carrying amount and the fair value of the impaired asset.

Revenue Recognition

We account for contracts with our customers in accordance with ASC 606, Revenue from Contracts with Customers (ASC 606), or ASC 606. Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of ASC 606, the entity performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. We only apply the five-step model to contracts when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services it transfers to the customer. The amount of variable consideration which is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized under contracts will not occur in a future period. Our analyses contemplate the application of the constraint in accordance with ASC 606. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from our estimates, we will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.

We have determined that the delivery of our product to our customer constitutes a single performance obligation as there are no other promises to deliver goods or services in contracts with our customers. Shipping and handling activities are considered to be fulfilment activities and are not considered to be a separate performance obligation. The payment terms with our customers do not exceed one year and therefore, no amount of consideration has been allocated as a

financing component. Taxes collected related to product sales are remitted to governmental authorities and are excluded from revenue.

Net Product Revenue

We recognize revenue from sales of WAKIX when the customer obtains control of the product, which occurs at a point in time, typically upon delivery. Product revenue is recorded at the product's list price, net of applicable reserves for variable consideration that are offered within contracts between us and our customers, payors, and other indirect customers relating to the sale of WAKIX. Components of variable consideration include government (as detailed below) and commercial contracts, commercial co-payment assistance program, and distribution service fees. These deductions are based on the amounts earned, or to be claimed on the related sales, and are classified as a current liability or reduction of receivables in our consolidated balance sheet.

Government Contracts

We have entered into contracts (i) to participate in the Medicaid Drug Rebate Program and the Medicare Part D program, and (ii) to sell to the U.S. Department of Veterans Affairs, 340b entities and other government agencies, or government payors, so that WAKIX will be eligible for purchase by, in partial or full reimbursement from, such government payors. We record reserves for rebates due pursuant to these contracts as a reduction of revenue in the same period in which the revenue is recognized. The liability for government rebates is included in accrued expenses in our consolidated balance sheet.

We estimate rebates due pursuant to government contracts based upon our historical payment trends, information obtained from third parties estimating current payment trends, the government-mandated discounts applicable to government-funded programs, as well as information obtained from our customers. The liability for these government rebates consists of estimates of claims for WAKIX dispensed in the current period, plus an estimate for product which has shipped and has been recognized as revenue but remains in the distribution channel at the end of a reporting period.

Cost of Product Sold

Cost of product sold includes manufacturing and distribution costs, the cost of drug substance, FDA program fees, royalties due to third parties on net product sales, freight, shipping, handling, storage costs, and salaries of employees involved with production.

Research and Development Expenses

Research and development costs are expensed as incurred. Liabilities due to third parties in connection with research and development collaborations prior to regulatory approval are expensed as incurred.

Upfront payments and pre-FDA approval milestone payments made for licensing of technology are expensed as research and development in the period in which they are incurred. Advance payments for goods and services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the related goods are delivered or the services are performed.

Advertising Expenses

We expense the costs of advertising, including promotional expenses, as incurred. Advertising expenses were $21,544, $22,428 and $19,558 for the years ended December 31, 2023, 2022 and 2021, respectively.

Stock-Based Compensation

The Company recognizes stock-based compensation expense in operating results using a fair value measurement method, in accordance with FASB ASC 718, Compensation-Stock Compensation. ASC 718 requires all stock-based payments to employees to be recognized in operating results as compensation expense based on fair value over the requisite service period of the awards. The vesting periods have a time-based provision consisting of one to five years and expire no more than 10 years after the date of grant. Upon a change of control, certain unvested awards will immediately vest. The Company determines the fair value of stock-based awards on their grant date using the Black-Scholes option-pricing model, which uses both historical and current market data to estimate fair value. The method incorporates various assumptions, such as the risk-free interest rate, expected volatility, expected dividend yield, and expected life of the options.

The Company also had non-employee stock awards subject to a performance condition that are recognized based on probable outcome of achieving future events.

Basic and Diluted Net Income per Share

Basic net income per share is determined using the weighted average number of shares of common stock outstanding during each period. Diluted net income per share includes the effect, if any, from the potential exercise or conversion of securities, such as stock options, and warrants which would result in the issuance of incremental shares of common stock.

Income Taxes

Income taxes are accounted for under the asset and liability method. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carryforwards. Deferred tax assets and liabilities are measured using enacted rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment date.

Deferred tax assets may be reduced by a valuation allowance if, based on all available evidence, it is more likely than not that some portion or all of the deferred income tax assets will not be realized. Management judgment is required in determining the period in which a reversal of a valuation allowance should occur. The Company is required to consider all available evidence, both positive and negative, such as historical levels of income and future forecasts of taxable income among other items, in determining whether a full or partial release of its valuation allowance is required. Our accounting for deferred tax consequences represents the best estimate of those future events. The Company presents deferred income taxes on the Consolidated Balance Sheet on a jurisdictional basis as either a net noncurrent asset or liability.

The Company recognizes the effect of income tax positions only if those positions are more likely than not sustainable, based solely on its technical merits and consideration of the relevant taxing authority's widely understood administrative practices and precedents. Recognized income tax positions are measured at the largest amount that is greater than 50% likely of being realized. Changes in recognition or measurement are reflected in the period in which a change in judgment occurs. At December 31, 2023 and 2022, the Company did not have any unrecognized uncertain tax positions. The Company's policy is to include any interest and penalties as a component of income tax expense.

Business Combinations

Business combinations and asset acquisitions are accounted for in accordance with FASB ASC 805 Business Combinations. Refer to Note 4, *Acquisition*, for a more detailed discussion of the Zynerba Acquisition.

Recently Issued Accounting Pronouncements

In November 2023, the FASB issued Accounting Standards Update ("ASU") No. 2023-07, *Improvements to Reportable Segment Disclosures* ("ASU 2023-07"). ASU 2023-07 is intended to improve reportable segment disclosures primarily through enhanced disclosure of reportable segment expenses and requires that a public entity that has a single reportable segment provide all the disclosures required by ASU 2023-07 and all existing segment disclosures in Topic 280. This ASU is effective for annual reporting periods beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024. Early adoption is permitted. ASU 2023-07 is required to be applied retrospectively to all prior periods presented in the financial statements. The Company has one reportable segment and is currently evaluating the impact that ASU 2023-07 will have on its consolidated financial statements.

In December 2023, the FASB issued Accounting Standards Update ("ASU") No 2023-09, *"Income Taxes (Topic 740): Improvements to Income Tax Disclosures"* ("ASU 2023-09"). ASU 2023-09 expands disclosures in the rate reconciliation and requires disclosure of income taxes paid by jurisdiction. ASU 2023-09 is effective for fiscal years beginning after December 15, 2024. Early adoption is permitted. The Company is currently evaluating the impact that ASU 2023-09 will have on its consolidated financial statements.

4. ACQUISITION

On October 10, 2023, the Company completed a tender offer to purchase the outstanding common stock of Zynerba ("Zynerba Common Stock") for (i) $1.1059 per share of Zynerba Common Stock (the "Common Cash Amount"), plus (ii) one

contingent value right (each, a “CVR”) per share of Zynerba Common Stock (the “Common CVR Amount”), which represents the right to receive up to approximately $2.5444 per share of Zynerba Common Stock, subject to the achievement of certain clinical, regulatory and sales-based milestones. Both the Common Cash Amount and Common CVR Amount are to be paid in cash, subject to any applicable withholding of taxes and without interest. The aggregate amount of consideration to acquire Zynerba Common Stock was $60,000, excluding transaction related fees of $2,645 and was paid by the Company using cash on hand.

The total purchase consideration for Zynerba was as follows:

Cash consideration paid to selling shareholders (i)	$	55,960
Cash consideration paid to settle Zynerba restricted stock awards ("RSAs") as stock options (ii)		4,040
Transaction costs		2,645
Total purchase consideration	$	62,645

(i) The cash consideration paid to selling shareholders was determined based on the total number of Zynerba shares tendered at closing of 50,602,656 at a per share price of $1.1059.

(ii) The cash consideration paid to settle Zynerba restricted stock awards (“RSAs”) and stock options under Zynerba equity incentive plans was determined based on the total number of underlying shares of 4,000,169 at a per share price of $1.1059, less exercise price for the stock options.

All consideration was paid during the year ended December 31, 2023.

The Zynerba Acquisition was accounted for as an asset acquisition under ASC Topic 805, Business Combinations, because substantially all of the fair value of the gross assets acquired was concentrated in a single identifiable IPR&D asset, ZYN002, Zynerba’s lead asset. ZYN002 is the first and only pharmaceutically manufactured, synthetic cannabidiol, a non-euphoric cannabidiol, formulated as a patent-protected permeation-enhanced gel for transdermal delivery through the skin and into the circulatory system and is currently in Phase III clinical trial for the potential treatment of Fragile X Syndrome. The Company recognized the acquired assets and assumed liabilities based on the consideration paid, including transaction costs, on a relative fair value basis, and after first allocating the preliminary excess of the fair value of net assets acquired over the purchase price consideration to certain qualifying assets, principally, the IPR&D asset. In accordance with the accounting for asset acquisitions, an entity that acquires IPR&D assets in an asset acquisition follows the guidance in ASC Topic 730 Research and Development, which requires that both tangible and intangible identifiable research and development assets with no alternative future use be allocated a portion of the consideration transferred and recorded as research and development expense at the acquisition date. As a result, the Company recorded a charge of $2,260 related to acquired in-process research and development expense related to the ZYN002 IPR&D asset during the year ended December 31, 2023.

The following table shows the allocation of the purchase consideration based on the relative fair value of assets acquired and liabilities assumed by the company, after reducing the excess fair value of the IPR&D asset as described above:

Assets acquired		
Cash and cash equivalents	$	25,658
Prepaid expenses and other current assets		3,540
Deferred tax asset		44,800
Restricted cash		20
Acquired in-process research and development		2,260
Total assets acquired	$	76,278
Liabilities assumed		
Accounts payable		4,995
Accrued expenses and accrued compensation		8,479
Other current liabilities		159
Total liabilities assumed	$	13,633
Net assets acquired	$	62,645

Subsequent to the acquisition, The Company incurred $7,544 in severance charges related to the acquisition, of which $3,858 was included in general administrative expenses and $3,686 was included in research and development expenses, within the consolidated statements of operations for the year ended December 31, 2023. As of December 31, 2023, the Company had accrued $7,544 related to these severance charges, which is included in accrued compensation in the consolidated balance sheet.

The amount of Zynerba's net loss included in the consolidated statements of operations and comprehensive income for the year ended December 31, 2023, was $14,451. There was no revenue from Zynerba included in the consolidated statements of operations and comprehensive income for the year ended December 31, 2023, as Zynerba has no historical sales.

Unaudited Pro Forma Financial Information

Unaudited pro forma net income was $108,350 and $135,567 for the year ended December 31, 2023, and 2022, respectively and there was no adjustment to unaudited pro forma net product revenue for the year ended December 31, 2023, and 2022, as Zynerba has no historical sales through December 31, 2023.

The unaudited pro forma combined financial information presented above has been prepared from historical financial statements that have been adjusted to give effect to the acquisition of Zynerba as though it had occurred on January 1, 2022. They include adjustments for severance expense and acquired in-process research and development expense. The unaudited pro forma financial information is not intended to reflect the actual results of operations that would have occurred if the acquisition had occurred on January 1, 2022, nor is it indicative of future operating results.

5. INVESTMENTS

The carrying value and amortized cost of the Company's available-for-sale debt securities, summarized by type of security, consisted of the following:

	December 31, 2023			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Short-term:				
Commercial paper	$ 23,832	36	(3)	$ 23,865
Corporate debt securities	15,968	28	—	15,996
U.S. government securities	1,940	—	(1)	1,939
Total short-term investments	$ 41,740	64	(4)	$ 41,800
Long-term:				
Commercial paper	$ 744	—	—	$ 744
Corporate debt securities	42,688	81	(28)	42,741
U.S. government securities	28,795	7	(118)	28,684
Total long-term investments	$ 72,227	88	(146)	$ 72,169

	December 31, 2022			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Short-term:				
Commercial paper	$ 26,553	15	(34)	$ 26,534
Corporate debt securities	49,213	9	(73)	49,149
U.S. government securities	3,658	—	(10)	3,648
Total short-term investments	$ 79,424	24	(117)	$ 79,331
Long-term:				
Commercial paper	$ 853	1	—	$ 854
Corporate debt securities	21,516	11	(68)	21,459
U.S. government securities	257	—	(2)	255
Total long-term investments	$ 22,626	12	(70)	$ 22,568

The Company classifies investments with an original maturity of less than one year as current and investments with an original maturity date of greater than one year as noncurrent on its consolidated balance sheet. The investments classified as noncurrent have original maturity dates ranging from 1-2 years. The Company did not have any available-for-sale debt security investments in a continuous unrealized loss position of greater than 12 months as of December 31, 2023, and December 31, 2022, respectively.

6. FAIR VALUE MEASUREMENTS

Money market funds are classified as Level 1 fair value instruments. Investments in available-for-sale debt securities are classified as Level 2 and carried at fair value, which we estimate utilizing a third-party pricing service. The pricing service utilizes industry standard valuation models whereby all significant inputs, including benchmark yields, reported trades, broker/dealer quotes, issuer spreads, bids, offers, or other market-related data, are observable. We validate valuations obtained from third-party services by obtaining market values from other pricing sources. The Company did not classify any assets or liabilities as Level 3 as of December 31, 2023, or December 31, 2022.

The Company's assets measured at fair value consisted of the following:

	December 31, 2023			December 31, 2022		
	Total	Level 1	Level 2	Total	Level 1	Level 2
Assets						
Cash equivalents	$ 244,569	243,685	884	$ 184,977	184,977	—
Commercial paper	24,609	—	24,609	27,388	—	27,388
Corporate debt securities	58,737	—	58,737	70,608	—	70,608
U.S. government securities	30,623	—	30,623	3,903	—	3,903
Total	$ 358,538	243,685	114,853	$ 286,876	184,977	101,899

7. INVENTORY

Inventory, net consisted of the following:

	As of	
	December 31, 2023	December 31, 2022
Raw materials	$ 1,060	$ 838
Work in process	2,020	1,513
Finished goods	2,283	2,565
Inventory, gross	5,363	4,916
Reserve for excess inventory	—	(619)
Total inventory, net	$ 5,363	$ 4,297

8. INTANGIBLE ASSETS

In August 2019, the Company received FDA approval of WAKIX® (pitolisant) for the treatment of excessive daytime sleepiness ("EDS") in adult patients with narcolepsy. This event triggered a milestone payment of $75,000 under the provisions of the 2017 LCA (defined below) which the Company capitalized as an intangible asset. The Company determined a useful life of 10 years for such intangible asset, and, as of December 31, 2023, the remaining useful life was 5.8 years.

In October 2020, the Company received FDA approval for the New Drug Application ("NDA") for WAKIX® for the treatment of cataplexy in adult patients with narcolepsy. This event triggered a milestone payment of $100,000 under the provisions of the 2017 LCA which the Company capitalized as an intangible asset and paid in January of 2021. The Company determined a useful life of 9 years for such intangible asset, and, as of December 31, 2023, the remaining useful life was 5.8 years.

In February 2022, the Company attained $500,000 in life-to-date aggregate net sales of WAKIX in the United States. This event triggered a final $40,000 payment under the provisions of the 2017 LCA which the Company capitalized as an intangible asset and paid in March of 2022. The Company determined a useful life of 7.6 years for such intangible asset, and, as of December 31, 2023, the remaining useful life was 5.8 years.

Amortization expense was $23,845, $22,966 and $18,424 for the years ended December 31, 2023, 2022 and 2021, respectively, and is recorded in general and administrative expenses in the consolidated statements of operations and comprehensive income (loss).

Future annual amortization expense for the unamortized intangible assets is as follows:

Years ending December 31,	
2024	$ 23,845
2025	23,845
2026	23,845
2027	23,845
2028	23,845
Thereafter	17,883
Total	$ 137,108

The gross carrying amount and net book value of the intangible asset is as follows:

	As of	
	December 31, 2023	December 31, 2022
Gross Carrying Amount	$ 215,000	$ 215,000
Accumulated Amortization	(77,892)	(54,047)
Net Book Value	$ 137,108	$ 160,953

9. LICENSE AGREEMENTS AND ASSET PURCHASE AGREEMENTS

In July 2017, Harmony entered into a License Agreement (the "2017 LCA") with Bioprojet Société Civile de Recherche ("Bioprojet") whereby Harmony acquired the exclusive right to commercialize the pharmaceutical compound pitolisant for the treatment, and/or prevention, of narcolepsy, obstructive sleep apnea, idiopathic hypersomnia, and Parkinson's disease as well as any other indications unanimously agreed by the parties in the United States and its territories. A milestone payment of $50,000 was due upon acceptance by the FDA of pitolisant's NDA, which was achieved in February 2019 and was expensed within research and development for the year ended December 31, 2019. A milestone payment of $77,000, which included a $2,000 fee that is described below, was due upon FDA approval of WAKIX (pitolisant) for treatment of EDS in adult patients with narcolepsy, which was achieved in August 2019. The $2,000 payment and $75,000 milestone payment were paid in August and November 2019, respectively. In addition, a milestone payment of $102,000, which included a $2,000 fee was due upon the FDA approval of the NDA for WAKIX for the treatment of cataplexy in adult patients with narcolepsy. The $2,000 payment was paid in October 2020 and a $100,000 milestone payment was paid in January 2021. A final $40,000 milestone payment was paid to Bioprojet in March 2022 upon WAKIX attaining $500,000 in aggregate net sales in the United States. The 2017 LCA also requires a fixed trademark royalty and a tiered royalty based on net sales, which is payable to Bioprojet on a quarterly basis. The Company incurred $111,685, $77,107 and $50,957 for the years ended December 31, 2023, 2022 and 2021, respectively, for sales-based, trademark and tiered royalties recognized as cost of product sold. As of December 31, 2023, and 2022, the Company had accrued $40,419 and $25,367, respectively, for sales-based, trademark and tiered royalties.

In July 2022, Harmony entered into a License and Commercialization Agreement (the "2022 LCA") with Bioprojet whereby Harmony obtained exclusive rights to manufacture, use and commercialize one or more new products based on pitolisant in the United States and Latin America, with the potential to add additional indications and formulations upon agreement of both parties. Harmony paid an initial, non-refundable $30,000 licensing fee in October 2022 and additional payments of up to $155,000 are potentially due under the 2022 LCA upon the achievement of certain future development and sales-based milestones. In addition, there are other payments due upon achievement of development milestones for new indications and formulations as agreed upon by both parties. The 2022 LCA also requires a fixed trademark royalty and a tiered royalty based on net sales upon commercialization, which will be payable to Bioprojet on a quarterly basis. The $30,000 licensing fee was recorded in research and development within the consolidated statement of operations and comprehensive income (loss) for the year ended December 31, 2022.

Agreement Related to Intellectual Property

In August 2021, the Company entered into an asset purchase agreement with ConSynance Therapeutics, Inc. (the "APA") to acquire HBS-102 (formerly referred to as "CSTI-100"), a potential first-in-class molecule with a novel mechanism

of action. Under the terms of the APA, the Company acquired full development and commercialization rights globally, with the exception of Greater China, for $3,500. The Company accounted for the transaction as an asset acquisition as substantially all of the fair value of the assets acquired was concentrated in a single identified asset. Additionally, there are payments due under the APA upon the achievement of certain milestones including $1,750 for preclinical milestones, $19,000 for development milestones, $44,000 for regulatory milestones and $110,000 for sales milestones.

10. ACCRUED EXPENSES

Accrued expenses consist of the following:

	As of	
	December 31, 2023	December 31, 2022
Royalties due to Bioprojet	$ 40,419	$ 25,367
Rebates and other sales deductions	38,842	27,860
Interest	3,354	3,286
Selling and marketing	2,354	1,135
Research and development	9,835	358
Professional fees, consulting, and other services	2,195	1,163
Other expenses	2,495	773
	$ 99,494	$ 59,942

11. DEBT

Credit Agreements

Term Loan A Credit Agreement

On July 26, 2023, the Company entered into a Credit Agreement (the "TLA Credit Agreement") with JPMorgan Chase Bank, N.A., as "Administrative Agent", and certain lenders. The TLA Credit Agreement provides for a five-year senior secured term loan (the "TLA Term Loan") in an aggregate principal amount of $185,000.

On September 21, 2023, the Company entered into the First Incremental Amendment (the "First Incremental Amendment") with the Administrative Agent and Bank of America, N.A., as incremental lender. The First Incremental Amendment provides for an incremental senior secured term loan (the "Incremental Term Loan") in an aggregate principal amount of $15,000. The First Incremental Amendment amends the TLA Credit Agreement and provides that the Incremental Term Loan will have identical terms as the TLA Term Loan.

The repayment schedule for both the TLA Term Loan and the Incremental Term Loan (together, the "Term Loans") consists of quarterly $3,750 principal payments, which commence on December 31, 2023, increasing to quarterly $5,000 principal payments beginning on December 31, 2025, with a $115,000 payment due on the maturity date of July 26, 2028. The Term Loans bear interest at a per annum rate equal to, at the Company's option, (i) a base rate plus a specified margin ranging from 2.50% to 3.00%, based on the Company's senior secured net leverage ratio (as defined in the TLA Credit Agreement) or (ii) Term SOFR plus a credit spread adjustment of 0.10% plus a specified margin ranging from 3.50% to 4.00%, based on the Company's senior secured net leverage ratio.

The net cash received related to the Term Loans as a result of the transactions, less debt issuance costs of $2,997, was $197,003. The debt issuance costs related to the Term Loans will be amortized as additional interest expense over the loan term of the TLA Credit Agreement. The fair value of the Term Loans as of December 31, 2023, was $199,859.

Blackstone Credit Agreement

In August 2021, the Company entered into the Blackstone Credit Agreement that provides for (i) a senior secured term loan facility in an aggregate original principal amount of $200,000 (the "Initial Term Loan") and (ii) a senior secured delayed draw term loan facility in an aggregate principal amount up to $100,000 (the "DDTL" and, together with the Initial Term Loan, the "Loans"). The DDTL was initially available to draw down through August 9, 2022. In August 2022, the Company entered into an agreement to extend the expiration date of the DDTL to August 9, 2023, for which the Company will pay a ticking fee at a rate of 1% per annum on the undrawn portion of the DDTL, which commenced on August 10, 2022.

Net cash received from the Initial Term Loan was $191,849, net of debt issuance costs of $8,151. In addition, the Company paid $1,000 in debt issuance costs relating to the DDTL, which was initially recorded in other current assets within the unaudited condensed consolidated balance sheet.

In connection with the TLA Credit Agreement, the Company extinguished the Blackstone Credit Agreement, which required a payoff amount of $207,308 consisting of principal repayment, interest, exit fees, Ticking Fee and a prepayment premium. The Company recognized a loss on extinguishment of debt of $9,766 relating to the Blackstone Credit Agreement within the Company's consolidated statements of operation and comprehensive income for the year ended December 31, 2023. In addition, the Company recognized $1,972 relating to unamortized debt issuance costs relating to the DDTL and the Ticking Fee, which was recorded in interest expense, net within the consolidated statement of operations and comprehensive income for the year ended December 31, 2023.

OrbiMed Credit Agreement

In January 2020, the Company entered into a credit agreement with OrbiMed for an aggregate amount of $200,000 (the "OrbiMed Loan"), with a maturity date of January 2026. Borrowings under the OrbiMed Loan were collateralized by all of the Company's assets, excluding the intellectual property licensed through the License Agreement. The OrbiMed Loan had an interest rate equal to the sum of (i) the greater of (a) 1-month LIBOR or (b) 2.00% per annum, plus (ii) 11.00% per annum, paid in cash monthly in arrears on the last day of each month starting in January 2020. At the time of prepayment or repayment of all or any portion of the principal of the OrbiMed Loan, the Company was required to pay an exit fee of 7.0% of the principal amount of the OrbiMed Loan prepaid, repaid, or required to be prepaid or repaid. The Company recorded the exit fee as a liability and debt discount at the origination of the term loan.

In connection with the OrbiMed Loan, the Company extinguished its $200,000 multi-draw loan agreement with CRG Servicing LLC (the "CRG Loan"), which required a payoff amount of $120,893 consisting of principal repayment, interest, and exit fees. In connection with extinguishment of the CRG Loan, the Company recognized a loss on extinguishment of $22,639 within the Company's consolidated statement of operations for the year ended December 31, 2020.

In connection with the Blackstone Credit Agreement, the Company extinguished the OrbiMed Loan, which required a payoff amount of $222,666 consisting of principal repayment, interest, exit fees and a prepayment premium. The Company recognized a loss on extinguishment of $26,146 relating to the OrbiMed Loan within the Company's consolidated statement of operation for the year ended December 31, 2021.

Long-term debt, net consists of the following:

	December 31, 2023	December 31, 2022
Liability component - principal	$ 196,250	$ 197,500
Unamortized debt discount associated with debt financing costs	(2,684)	(5,853)
Liability component - net carrying value	193,566	191,647
Less current portion	(15,000)	(2,000)
Long-term debt, net	$ 178,566	$ 189,647

Future minimum payments relating to long term debt, net as of December 31, 2023, for the periods indicated below consists of the following:

Years ending December 31,	
2024	$ 15,000
2025	16,250
2026	20,000
2027	20,000
2028	125,000
Thereafter	—
Total	$ 196,250

Interest expense related to the Company's long term debt, net, is included in interest expense, net in the consolidated statements of operations and comprehensive income (loss) and consists of the following:

	Year Ended December 31,		
	2023	2022	2021
Interest on principal balance	$ 20,511	$ 17,132	$ 21,955
Amortization of deferred financing costs	3,246	1,663	2,238
Total term loan interest expense	$ 23,757	$ 18,795	$ 24,193

12. LEASES

In June 2018, the Company entered into an operating lease for approximately fifteen thousand square feet of office space in Plymouth Meeting, PA, which expires in May 2024. The Company subsequently entered into two separate operating leases for additional office space in Plymouth Meeting, PA, which include approximately thirteen thousand square feet and seven thousand square feet of additional office space, respectively, and expire in May 2024. The terms of the lease payments provide for rental payments on a monthly basis and on a graduated scale. The Company also leases a fleet of automobiles that are used by its sales representatives and are classified as operating leases.

Operating lease right-of-use assets and operating lease liabilities are recognized based on the present value of the future lease payments using our incremental borrowing rate. Leases with an initial term of 12 months or less are not recorded on the balance sheet. Our leases have remaining lease terms of less than 1 year to 3 years, some of which may include the option to extend or terminate the leases.

The company recorded operating lease costs of $1,811 and $1,526 for the years ended December 31, 2023, and 2022, respectively.

As of December 31, 2023, the weighted-average remaining lease term for operating leases was 2.0 years and the weighted-average discount rate for operating leases was 6.73%.

Supplemental balance sheet information related to operating leases was as follows:

Leases	Classification	December 31, 2023	December 31, 2022
Assets			
Operating lease right-of-use assets	Other noncurrent assets	$ 2,344	$ 2,312
Liabilities			
Operating lease liability, current portion	Other current liabilities	$ 1,437	$ 1,614
Operating lease liability, long-term	Other long-term liabilities	1,082	975
Total operating lease liabilities		$ 2,519	$ 2,589

Supplemental cash flow information related to operating leases was as follows:

	December 31, 2023	December 31, 2022
Operating cash flows from operating leases	$ 2,016	$ 1,716
Right of use assets obtained in exchange for operating lease obligations	$ 2,163	$ 485

Future payments under noncancelable operating leases with initial terms of one year or more as of December 31, 2023, consisted of the following:

Years ending December 31,	
2024	$ 1,550
2025	732
2026	425
2027	-
2028	-
Thereafter	-
Total lease payments	2,707
Less: imputed interest	(188)
Total lease liabilities	$ 2,519

13. COMMITMENTS AND CONTINGENCIES

Litigation

From time to time, the Company is subject to claims and suits arising in the ordinary course of business. The Company accrues such liabilities when they are known, if they are deemed probable and can be reasonably estimated. As of December 31, 2023, there were no material claims or suits outstanding.

14. STOCKHOLDERS' EQUITY

Common Stock

The holders of common stock are entitled to one vote for each share held on all matters submitted to a vote of the Company's stockholders. The holders of common stock do not have any cumulative voting rights. Holders of common stock are entitled to receive ratably any dividends declared by the Company's board of directors out of funds legally available for that purpose, subject to any preferential dividend rights of any outstanding preferred stock. The Company's common stock has no preemptive rights, conversion rights or other subscription rights or redemption or sinking fund provisions.

In connection with the Blackstone Credit Agreement, in August 2021, the Company sold an aggregate of 1,048,951 shares of our common stock, par value $0.00001 per share, for an aggregate cash consideration of $30,000, or $28.60 per share. This sale did not involve a public offering and was therefore exempt from registration under Section 4(a)(2) of the Securities Act of 1933, as amended, and Regulation D thereunder as a transaction not involving any public offering.

In connection with the OrbiMed Loan, the Company issued warrants to OrbiMed Royalty & Credit Opportunities, LP on January 9, 2020. Pursuant to the warrants, OrbiMed Royalty & Credit Opportunities, LP had the option to purchase up to 410,239 shares of the Company's common stock for an initial exercise price of $16.10. In September 2021, OrbiMed exercised its option to purchase shares of the Company's common stock which resulted in the net settlement of 221,511 shares of common stock being issued to OrbiMed.

Share Repurchase Program

On August 1, 2023, the Company's Board of Directors approved a program providing for the repurchase of shares of common stock in an aggregate amount of up to $125,000, excluding commissions and transaction fees (the "August 2023 Repurchase Program"). The August 2023 Repurchase Program may be suspended, terminated or modified at any time for any reason. During the year ended December 31, 2023, the Company repurchased and retired 1,439,792 shares of common stock at an aggregate cost of $50,000 under the August 2023 Repurchase Program, excluding commissions and transaction fees.

On October 27, 2023, the Company's Board of Directors terminated the August 2023 Repurchase Program and any remaining amount authorized for the repurchase of shares. Simultaneously, the Company's Board of Directors approved a share repurchase program (the "October 2023 Repurchase Program") providing for the repurchase of shares of common stock in an aggregate amount of up to $200,000, excluding commissions and transaction fees. The October 2023 Repurchase Program may be suspended, terminated, or modified at any time for any reason. During the year ended December 31, 2023, the Company repurchases and retired 1,814,653 shares of common stock at an aggregate cost of $50,000 under the October 2023 Repurchase Program, excluding commissions and transaction fees.

15. STOCK INCENTIVE PLAN AND STOCK-BASED COMPENSATION

2020 Stock Incentive Plan

In August 2020, the Company adopted, and its stockholders approved, the 2020 Incentive Award Plan (the "2020 Plan"), in order to facilitate the grant of cash and equity incentives to directors, employees (including the Company's named executive officers) and consultants of the Company and its subsidiaries. The 2020 Plan provides for the grant of stock

options, including incentive stock options ("ISOs") and non-qualified stock options ("NSOs"), SARs, restricted stock, dividend equivalents, restricted stock units ("RSUs") and other stock or cash-based awards.

Stock options and stock appreciation rights under the 2020 Plan have a 10-year contractual term and vest over the vesting period specified in the applicable award agreement, at achievement of a performance requirement, or upon change of control (as defined in the applicable plan). RSUs vest over the vesting period specified in the applicable award agreement, at achievement of a performance requirement, or upon change of control (as defined in the applicable plan). As of December 31, 2023, there were 6,691,219 shares of common stock available for issuance under the 2020 Plan. The number of shares that may be issued under the 2020 Plan will automatically increase on January 1 of each year in an amount equal to the lesser of (i) 4.0% of the shares of the Company's common stock outstanding on December 31 of the preceding year or (ii) an amount determined by the Company's board of directors.

2017 Stock Incentive Plan

In August 2017, the Company adopted an equity incentive plan (the "2017 Plan"). Under the 2017 Plan, directors, officers, employees, consultants, and advisors of the Company can be paid incentive compensation measured by the value of the Company's common shares through grants of stock options, stock appreciation rights ("SARs"), or restricted stock. Following the adoption of the 2020 Plan, no further grants have been, or will be, made under the 2017 Plan. However, the 2017 Plan will continue to govern the terms and conditions of outstanding awards granted under it.

Stock Options

The following table summarizes stock option activity for the year ended December 31, 2023:

	Number of Awards	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term
Awards outstanding—December 31, 2022	6,460,947	$ 30.90	7.86
Awards issued	440,294	$ 34.80	
Awards exercised	(371,896)	$ 12.00	
Awards forfeited	(212,923)	$ 25.25	
Awards outstanding—December 31, 2023	6,316,422	$ 32.47	7.17

Stock Appreciation Rights

The following table summarizes SARs activity for the year ended December 31, 2023:

	Number of Awards	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term
Awards outstanding—December 31, 2022	43,208	$ 9.38	6.32
Awards issued	—	$ —	
Awards exercised	—	$ —	
Awards forfeited	—	$ —	
Awards outstanding—December 31, 2023	43,208	$ 9.38	5.32

Restricted Stock Units

The following table summarizes RSU activity for the year ended December 31, 2023:

	Number of Awards	Weighted-Average Grant Date Fair Value
Awards outstanding—December 31, 2022	60,000	$ 29.03
Awards issued	300,000	$ 31.78
Awards vested	(30,000)	$ 29.03
Awards forfeited	—	$ —
Awards outstanding—December 31, 2023	330,000	$ 31.53

As of December 31, 2023, and 2022, stock awards issued under the 2017 and 2020 Plans of 3,298,284 and 1,818,045 common shares, respectively, were vested.

Value of Stock Options and SARs

The Company values options and SARs using the Black-Scholes option-pricing model. The Company lacks sufficient historical company-specific volatility information. Therefore, the Company estimates expected stock volatility based on historical volatility of peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. For options with service-based vesting conditions, the expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. For SARs, the expected term is based upon the weighting of certain future events. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for the time periods approximately equal to the expected term of the award. An expected dividend yield of 0% is based on the fact that the Company has never paid cash dividends and does not expect to do so in the foreseeable future.

The assumptions used to value the awards are summarized in the following table.

	As of	
	December 31, 2023	December 31, 2022
Dividend yield	0.00 %	0.00 %
Expected volatility	74.87 - 80.78 %	72.57 - 77.08 %
Risk-free interest rate	3.42 - 4.62 %	1.99 - 4.05 %
Lack of marketability discount	0.00 %	0.00 %
Expected term (years)	2.26 - 10.77	3.1 - 6.3

Value of RSUs

The fair value of RSUs is equal to the value of the Company's common stock on the grant date.

The weighted average per share fair value of awards issued under the 2017 Plan and the 2020 Plan was $20.64, $18.88 and $12.82 in 2023, 2022 and 2021, respectively.

Stock-Based Compensation Expense

Stock-based compensation expense was $31,206, $26,905 and $16,105 for the years ended December 31, 2023, 2022 and 2021, respectively, and is recorded in the consolidated statements of operations and comprehensive income (loss) in the following line items:

	Year Ended December 31,		
	2023	2022	2021
Research and development expense	$ 3,962	$ 2,614	$ 2,121
Sales and marketing expense	5,148	3,886	3,103
General and administrative expense	22,096	20,405	10,881
	$ 31,206	$ 26,905	$ 16,105

Stock-based compensation expense, net related to options and RSUs issued under the 2017 Plan and 2020 Plan is included in stockholder's equity, and a liability for SARs is included in other non-current liabilities, in the Company's consolidated balance sheet. As of December 31, 2023, the total unrecognized stock-based compensation expense related to options and RSUs was $69,594 which will be recognized in the Company's consolidated statement of operations over a weighted average period of 2.3 years.

Employee Stock Purchase Plan

The 2021 Employee Stock Purchase Plan ("ESPP") was adopted by the Company's Board of Directors in April 2021. The ESPP permits eligible employees to purchase shares of the Company's common stock at a 15% discount from the lesser of the fair market value per share of the Company's common stock on the first day of the offering period or the fair market value of the Company's common stock on the purchase date. Funds are collected from employees through after-tax payroll deductions. The total number of shares reserved for issuance under the ESPP was initially 629,805, which will automatically increase on January 1 of each year in an amount equal to the lesser of (i) 1.0% of the shares of the Company's common stock outstanding on December 31 of the preceding year or (ii) an amount determined by the Company's board of directors. It is intended that the ESPP meet the requirements for an "employee stock purchase plan" under Section 423 of the Internal Revenue Code. For the years ended December 31, 2023, 2022 and 2021, there were 21,313, 21,943 and 11,010 shares issued under the ESPP, respectively. The discount on the ESPP was $393, $363 and $173 for the years ended December 31, 2023, 2022 and 2021, respectively, and is recorded within stock-based compensation expense.

16. EARNINGS PER SHARE

Basic earnings per share is calculated by diving net income by the weighted average number of shares of common stock outstanding. For the years ended December 31, 2023, 2022 and 2021, respectively, the Company calculated Diluted net income per common share is computed under the treasury stock method by using the weighted average number of shares of common stock outstanding, plus, for periods with net income attributable to common stockholders, the potential dilutive effects of stock options, stock appreciation rights and restricted stock units.

The following table sets forth the computation of basic and diluted net income (loss) per share:

	Year Ended December 31,		
	2023	2022	2021
Numerator			
Net income	$ 128,853	$ 181,468	$ 34,597
Net income available to common shareholders	$ 128,853	$ 181,468	$ 34,597
Denominator			
Net income per share of common stock - basic	$ 2.17	$ 3.07	$ 0.60
Net income per share of common stock- diluted	$ 2.13	$ 2.97	$ 0.58
Weighted average number of shares of common stock - basic	59,469,648	59,173,121	57,531,540
Weighted average number of shares of common stock - diluted	60,372,397	61,097,045	59,205,213

Securities outstanding that were included in the computation above, utilizing the treasury stock method are as follows:

	Year Ended December 31,		
	2023	2022	2021
Stock options, SARs, and RSUs to purchase common stock	902,749	1,923,924	1,536,825
Warrants	—	—	136,848
Total	902,749	1,923,924	1,673,673

Potential common shares issuable upon conversion of preferred stock and exercise of stock options that were excluded from the computation of diluted weighted-average shares outstanding excluded from the numerator are as follows:

	Year Ended December 31,		
	2023	2022	2021
Stock options, SARs, and RSUs to purchase common stock	5,786,881	4,640,231	4,289,066

17. INCOME TAXES

Details of the provision for income taxes consist of the following:

	Year Ended December 31,		
	2023	2022	2021
Federal	$ 31,084	$ 10,011	$ 6,487
State	11,884	9,573	2,852
Valuation allowance	1,575	(96,366)	(6,508)
	$ 44,543	$ (76,782)	$ 2,831
Current	$ 57,962	$ 9,161	$ 2,831
Deferred	(14,994)	10,423	6,508
Valuation allowance	1,575	(96,366)	(6,508)
Total	$ 44,543	$ (76,782)	$ 2,831

The reasons for the difference between the statutory federal income tax rate and the Company's effective income tax rate as of December 31, 2023, 2022 and 2021 are as follows:

	Year Ended December 31,		
	2023	2022	2021
Federal income tax rate	21.0 %	21.0 %	21.0 %
Stock-based compensation	(0.4)	(4.9)	(2.3)
State taxes	5.7	(14.1)	6.4
Credits	(2.1)	(4.8)	—
Other	0.9	(0.3)	(0.2)
Valuation allowance	0.6	(70.2)	(17.3)
Total	25.7 %	(73.3)%	7.6 %

Significant components of the Company's deferred tax assets and liabilities as of December 31, 2023, and 2022, are as follows:

	As of December 31,			
	2023		2022	
	Assets	Liabilities	Assets	Liabilities
Acquired in-process research and development	$ 35,337	$ —	$ 45,938	$ —
Net operating loss carryforward	45,917	—	6,370	—
Accrued compensation	19,915	—	11,551	—
Lease obligations, net	-	6	71	—
Fixed assets	173	—	115	—
Inventory	5,344	—	6,781	—
Accrued rebates	3,791	—	7,118	—
Research and development	38,799	—	8,802	—
Other	1,132	790	15	818
Total	$ 150,408	796	$ 86,761	818
Net deferred tax asset	$ 149,612	$ —	$ 85,943	$ —
Valuation allowance	$ (5,450)	$ —	$ —	$ —
Total	$ 144,162	$ —	$ 85,943	$ —

As of December 31, 2023, and December 31, 2022, our deferred tax assets were primarily the result of acquired in-process research and development costs, operating loss carryforwards, capitalized research and development costs, inventory, and accrued rebates. As of December 31, 2023, the Company recorded a valuation allowance on federal net operating losses of Zynerba's subsidiary, Zynerba Pharmaceuticals Pty Ltd., and the state net operating losses of Zynerba. The Company previously recorded a valuation allowance against Harmony's historical deferred tax assets. The Company recorded $96,336 as a benefit to the income tax provision upon release of this valuation allowance during year ended December 31, 2022, in part because in the year ended December 31, 2022, we achieved three years of cumulative pretax income, which was a positive indication of the Company's ability to generate sufficient future taxable income, the Company determined that there was sufficient positive evidence to conclude that it was more likely than not that additional deferred taxes are realizable and, therefore, released the valuation allowance accordingly. Changes in the valuation allowance for deferred tax assets during the years ended December 31, 2023, 2022 and 2021 were as follows:

	Year Ended December 31,		
	2023	2022	2021
Valuation allowance at the beginning of the year	$ —	$ (96,366)	$ (102,874)
Decreases recorded as a benefit to income tax provision	—	96,366	6,508
Increases due to Zynerba Acquisition	(3,875)	—	—
Increases recorded to income tax provision	(1,575)	—	—
Valuation allowance at the end of the year	$ (5,450)	$ —	$ (96,366)

As of December 31, 2023, and 2022, the Company has approximately $179,390 and $0, respectively, of federal net operating loss ("NOL") carryforward available to offset future federal taxable income. The Company also has approximately $137,331 and $95,230 of state NOL carryforwards as of December 31, 2023, and 2022, respectively, available to offset future state taxable income. All of the Company's tax years remain open to examination by federal and state taxing authorities. The Company's state NOLs begin to expire in 2037. Utilization of the net operating loss carryforwards may be subject to a substantial limitation due to state provisions. These changes may limit the amount of net operating loss and tax credit carryforwards that can be utilized annually to offset future taxable income and tax, respectively. In general, an "ownership change" as defined by Section 382 of the Code results from a transaction or series of transactions over a three-year period resulting in an ownership change of more than 50 percentage points of the outstanding stock of a company by certain stockholders or public groups.

As of December 31, 2023, and 2022, the Company has federal tax credits of $0 and $0, respectively.

18. RETIREMENT PLAN

The Company formed a 401(k) defined contribution savings plan (the "401(k) Plan") in January 2021. The 401(k) Plan is for the benefit of all qualifying employees and permits voluntary contributions by employees limited by the IRS-imposed maximum. Employer contributions were $2,128, $1,705, and $2,479 for the years ended December 31, 2023, 2022 and 2021, respectively.

19. RELATED-PARTY TRANSACTIONS

The Company was party to a management agreement for professional services provided by a related party, Paragon. The related party is an entity that shares common ownership with the Company. In addition, the Chairman of the Company's board of directors was the President and owner of the entity. The Company is party to a right of use agreement with the related party whereby it has access to and the right to use certain office space leased by the related party in Chicago, Illinois. For the years ended December 31, 2023, 2022 and 2021, the Company incurred $290, $284 and $284, respectively, in expenses pursuant to the right of use agreement with this related party, which are included in general and administrative expense in the consolidated statements of operations and comprehensive income (loss). In August 2021, the Company paid a $2,300 advisory fee to Paragon in connection with the Blackstone Credit Agreement. $2,000 of this payment was recorded in debt issuance costs as a component of long-term debt, net and $300 was recorded in common stock issuance costs as a component of additional paid-in capital, within the consolidated balance sheet as of December 31, 2022. As of December 31, 2023, and 2022, there were no amounts due to or due from related parties included within the consolidated balance sheet.

20. VALUATION AND QUALIFYING ACCOUNTS – ACCOUNTS RECEIVABLE, NET

Changes in the valuation allowance for accounts receivable, net during the years ended December 31, 2023, 2022, and 2021, were as follows:

	Year Ended December 31,		
	2023	2022	2021
Allowance for distribution fees, discounts and chargebacks at the beginning of the year	$ 1,830	$ 1,885	$ 806
Additions due to current period provision	21,821	14,806	12,174
Deductions due to payment of distribution fees, discount and chargebacks	(21,007)	(14,861)	(11,095)
Allowance for distribution fees, discounts and chargebacks at the beginning of the year	$ 2,644	$ 1,830	$ 1,885

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure.

None.

Item 9A. Controls and Procedures.

Limitations on Effectiveness of Controls and Procedures

Our management does not expect that our disclosure controls and procedures or our internal control over financial reporting will prevent all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, within our Company have been detected.

Evaluation of Disclosure Controls and Procedures

Our management, including our principal executive officer and our principal financial officer, has evaluated the effectiveness of our disclosure controls and procedures as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the "Exchange Act"), as of the end of the period covered by this Annual Report on Form 10-K. Based on that evaluation, our principal executive officer and principal financial officer have concluded that, as of the end of the period covered by this Annual Report on Form 10-K, our disclosure controls and procedures were effective to provide reasonable assurance that information we are required to disclose in reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in SEC rules and forms, and that such information is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

Management's Annual Report on Internal Control over Financial Reporting

Management of the Company is responsible for establishing and maintaining adequate internal control over the Company's financial reporting, as defined in Rule 13a-15(f) of the Exchange Act. Internal control over financial reporting is a process designed under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles in the United States of America. The Company acquired Zynerba on October 10, 2023. SEC guidance permits companies to exclude certain acquisitions from the assessment of internal control over financial reporting during the first year following acquisition. Accordingly, management has excluded Zynerba from its assessment of the effectiveness of the Company's internal control over financial reporting as of December 31, 2023. Zynerba represents approximately 8% and 0% of total assets and net product revenue, respectively, of the consolidated financial statement amounts as of and for the year ended December 31, 2023. Our independent registered public accounting firm, Deloitte & Touche LLP, has issued an attestation report on our internal control over financial reporting that is included in this Annual Report on Form 10-K.

As of December 31, 2023, our management assessed the effectiveness of our internal control over financial reporting using the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission in Internal Control-Integrated Framework (2013). Based on this assessment, management, under the supervision and with the participation of our principal executive officer and principal financial officer, concluded that, as of December 31, 2023, our internal control over financial reporting was effective. Our independent registered public accounting firm, Deloitte & Touche LLP, has audited the effectiveness of our internal control over financial reporting as of December 31, 2023, as stated in their report which is included in this Annual Report on Form 10-K.

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the shareholders and the Board of Directors of Harmony Biosciences Holdings, Inc.

Opinion on Internal Control over Financial Reporting

We have audited the internal control over financial reporting of Harmony Biosciences Holdings, Inc., and subsidiaries (the "Company") as of December 31, 2023, based on criteria established in Internal Control — Integrated Framework (2013)

issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). In our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2023, based on criteria established in Internal Control — Integrated Framework (2013) issued by COSO.

We have also audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the consolidated financial statements as of and for the year ended December 31, 2023, of the Company and our report dated February 22, 2024, expressed an unqualified opinion.

As described in Management's Annual Report on Internal Control over Financial Reporting, management excluded from its assessment the internal control over financial reporting at Zynerba Pharmaceuticals, Inc., which was acquired on October 10, 2023, and whose financial statements constitute 8% of total assets and 0% of net product revenue of the consolidated financial statement amounts as of and for the year ended December 31, 2023. Accordingly, our audit did not include the internal control over financial reporting at Zynerba Pharmaceuticals, Inc.

Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying Management's Annual Report on Internal Control over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

Definition and Limitations of Internal Control over Financial Reporting

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

/s/ Deloitte & Touche LLP

Philadelphia, PA
February 22, 2024

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting during the quarter ended December 31, 2023, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

(a) None.

(b) On November 2, 2023, Sandip Kapadia, Chief Financial Officer and Chief Administrative Officer of the Company, modified a Rule 10b5-1 trading arrangement, originally adopted on February 22, 2023, to change the amount of shares to be sold related to the exercise of options under the plan. The modified plan is intended to satisfy the affirmative defense of Rule 10b5-1(c) and provides for the sale of up to 30,000 shares of the Company's common stock until April 3, 2024. On November 2, 2023, Mr. Kapadia also adopted a Rule 10b5-1 trading arrangement that is intended to satisfy the affirmative defense of Rule 10b5-1(c) and provides for the sale of up to 55,000 shares of the Company's common stock related to the exercise of options beginning on April 4, 2024, and until December 30, 2024.

On December 7, 2023, Jeffrey Dierks, Chief Commercial Officer of the Company, adopted a Rule 10b5-1 trading arrangement that is intended to satisfy the affirmative defense of Rule 10b5-1(c) for the sale of up to 16,097 shares of the Company's common stock until June 30, 2024, related to the exercise of options.

During the quarter ended December 31, 2023, no director or officer of the Company terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement," as each term is defined in Item 408(a) of Regulation S-K.

Item 9C. Disclosures Regarding Foreign Jurisdictions that Prevent Inspections.

Not applicable.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

The information required by this item will be set forth in our Proxy Statement for the 2024 Annual Meeting of Stockholders and is incorporated by reference. The Proxy Statement will be filed with the SEC within 120 days of the fiscal year ended December 31, 2023.

Our board of directors has adopted a code of business conduct and ethics that applies to all of our employees, officers and directors, including our Chief Executive Officer, Chief Financial Officer and other executive and senior financial officers. The full text of our code of business conduct and ethics is posted on the investor relations page on our website which is located at https://ir.harmonybiosciences.com. We will post any amendments to our code of business conduct and ethics other than technical, administrative or other non-substantive amendments, or waivers of its requirements, on our website or in a Form 8-K filed with the SEC.

Item 11. Executive Compensation.

The information required by this item will be set forth in our Proxy Statement for the 2024 Annual Meeting of Stockholders and is incorporated herein by reference. The Proxy Statement will be filed with the SEC within 120 days of the fiscal year ended December 31, 2023.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

The information required by this item will be set forth in our Proxy Statement for the 2024 Annual Meeting of Stockholders and is incorporated herein by reference. The Proxy Statement will be filed with the SEC within 120 days of the fiscal year ended December 31, 2023.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

The information required by this item will be set forth in our Proxy Statement for the 2024 Annual Meeting of Stockholders and is incorporated herein by reference. The Proxy Statement will be filed with the SEC within 120 days of the fiscal year ended December 31, 2023.

Item 14. Principal Accountant Fees and Services.

The information required by this item will be set forth in our Proxy Statement for the 2024 Annual Meeting of Stockholders and is incorporated herein by reference. The Proxy Statement will be filed with the SEC within 120 days of the fiscal year ended December 31, 2023.

PART IV

Item 15. Exhibits, Financial Statement Schedules.

(a)(1) Financial Statements

See "Part II—Item 8. Financial Statements and Supplementary Data.—Index to Financial Statements."

(a)(2) Financial Statement Schedules

All financial statement schedules have been omitted as the information is not required under the related instructions or is not applicable or because the information required is already included in the financial statements or the notes those financial statements.

(a)(3) Exhibits.

The documents set forth below are filed herewith or incorporated herein by reference to the location indicated.

EXHIBIT INDEX

Exhibit No.	Exhibit Description	Incorporated by Reference			Filed Herewith
		Form	Date	Number	
2.1	Agreement and Plan of Merger, dated August 14, 2023, by and among Harmony Biosciences Holdings, Inc., Xylophone Acquisition Corp. and Zynerba Pharmaceuticals, Inc.	8-K/A	September 14, 2023	2.1	
3.1	Amended and Restated Certificate of Incorporation of Harmony Biosciences Holdings, Inc.	8-K	August 21, 2020	3.1	
3.2	Amended and Restated Bylaws.	8-K	August 21, 2020	3.2	
4.1	Form of Common Stock Certificate.	S-1/A	August 6, 2020	4.1	
4.2	Description of Registrant's Securities.	10-K	March 25, 2021	4.3	
10.1	Credit Agreement, dated as of August 9, 2021, among Harmony Biosciences, Inc., as Borrower, Harmony Biosciences, LLC, as Guarantor, the Guarantors from time to time party thereto, the Lenders from time to time party thereto, and Wilmington Trust, National Association, as Administrative Agent.	10-Q	August 10, 2021	10.2	
10.2	Pledge and Security Agreement, dated as of August 9, 2021, among Harmony Biosciences, Inc. and Harmony Biosciences, LLC, as Grantors, the Grantors from time to time party thereto, and Wilmington Trust, National Association, as Administrative Agent.	10-Q	August 10, 2021	10.3	
10.3*	Harmony Biosciences Holdings, Inc. 2020 Incentive Award Plan, as amended by the First Amendment to the Plan, dated March 24, 2022.	10-K	February 21, 2023	10.3	

Exhibit No.	Exhibit Description	Incorporated by Reference			Filed Herewith
		Form	Date	Number	
10.4*	Form of Option Agreement under Harmony Biosciences Holdings, Inc. 2020 Incentive Award Plan.	S-8	August 21, 2020	10.3	
10.5*	Form of Restricted Stock Unit Agreement under Harmony Biosciences Holdings, Inc. 2020 Incentive Award Plan.	S-1/A	August 11, 2020	10.6	
10.6*	Harmony Biosciences Holdings, Inc. 2020 Employee Stock Purchase Plan.	S-1/A	August 11, 2020	10.7	
10.7.1*	Amended and Restated Employment Agreement, dated August 11, 2020, by and between Harmony Biosciences, LLC and John C. Jacobs.	S-1/A	August 11, 2020	10.8	
10.7.2*	Acknowledgment and Release Agreement, dated January 20, 2023, by and between Harmony Biosciences Holdings, Inc. and John C. Jacobs.	10-K	February 21, 2023	10.7.2	
10.8	Form of Indemnification Agreement between Harmony Biosciences, LLC and each director and executive officer.	S-1/A	August 11, 2020	10.12	
10.9	Harmony Biosciences, LLC Separation Plan.	S-1/A	August 11, 2020	10.13	
10.10*	Harmony Biosciences Holdings, Inc. Amended and Restated 2017 Equity Incentive Plan.	S-1/A	August 11, 2020	10.3	
10.11*	Harmony Biosciences Holdings, Inc. Non-Employee Director Compensation Program.	10-Q	November 12, 2020	10.9	
10.12.1*	Offer Letter, dated October 10, 2017, by and between Harmony Biosciences, LLC and Jeffrey Dayno.	S-1/A	August 11, 2020	10.9	
10.12.2*	Promotion Letter Agreement, dated January 23, 2023, by and between Harmony Biosciences Holdings, Inc. and Jeffrey M. Dayno, M.D.	8-K/A	January 23, 2023	10.1	
10.12.3*	Executive Employment Agreement, dated April 24, 2023, between Harmony Biosciences, LLC and Jeffrey M. Dayno, M.D.	8-K	April 24, 2023	10.1	
10.13*	Offer Letter, dated September 8, 2017, by and between Harmony Biosciences, LLC and Andrew Serafin.	S-1/A	August 11, 2020	10.10	
10.14+	License and Commercialization Agreement, dated July 28, 2017, by and between Bioprojet Société Civile de Recherche and Harmony Biosciences, LLC.	S-1	July 27, 2020	10.10	
10.15	Amendment No. 1 to License and Commercialization Agreement, dated August 27, 2018, by and between Bioprojet Société Civile de Recherche and Harmony Biosciences, LLC.	S-1	July 27, 2020	10.11	

Exhibit No.	Exhibit Description	Incorporated by Reference			Filed Herewith
		Form	Date	Number	
10.16+	Trademark License Agreement, dated August 23, 2018, by and among Bioprojet Europe, Ltd., Bioprojet Société Civile de Recherche and Harmony Biosciences, LLC.	S-1	July 27, 2020	10.12	
10.17	Management Services Agreement, dated September 22, 2017, by and between Paragon Biosciences, LLC and Harmony Biosciences, LLC.	S-1	July 27, 2020	10.13	
10.18	Right of Use Agreement, dated November 1, 2019, by and between Paragon Biosciences, LLC and Harmony Biosciences, LLC.	S-1	July 27, 2020	10.14	
10.19	Second Amended and Restated Investors' Rights Agreement, dated August 9, 2019, by and among the Registrant and the other parties thereto.	S-1	July 27, 2020	10.15	
10.20*	Offer Letter, dated September 7, 2017, by and between Harmony Biosciences, LLC and Jeffrey Dierks.	10-K	March 25, 2021	10.20	
10.21*	Employment Agreement, dated March 4, 2021, between Harmony Biosciences, LLC and Sandip Kapadia.	8-K	March 15, 2021	10.1	
10.22*	Promotion Increase Award Letter to Jeffrey Dierks, dated June 9, 2021.	10-Q	August 10, 2021	10.1	
10.23*	Restricted Stock Unit Award Agreement dated October 4, 2023 with Sandip Kapadia	8-K	October 5, 2023	10.1	
10.24*	Restricted Stock Unit Award Agreement dated October 4, 2023 with Jeffrey Dierks	8-K	October 5, 2023	10.2	
10.25	Credit Agreement, dated as of July 26, 2023, by an among JPMorgan Chase Bank, N.A., as administrative agent, and the lenders from time to time party thereto.	8-K	July 27, 2023	10.1	
10.26	Guaranty, dated July 26, 2023, by and among each of the subsidiaries of Harmony Biosciences Holdings, Inc. and JPMorgan Chase Bank, N.A., as administrative agent.	8-K	July 27, 2023	10.2	
10.27	Pledge and Security Agreement, dated July 26, 2023, by and among Harmony Biosciences Holdings, Inc. and each of the subsidiaries of Harmony Biosciences Holdings, Inc., and JPMorgan Chase Bank, N.A., as administrative agent.	8-K	July 27, 2023	10.3	
10.28	First Incremental Amendment dated September 21, 2023 by and among Harmony Biosciences Holdings, Inc., JPMorgan Chase Bank, N.A., as administrative agent, and Bank of America, N.A., as incremental lender.	8-K	September 25, 2023	10.1	
21.1	List of Subsidiaries of Harmony Biosciences Holdings, Inc.				X

Exhibit No.	Exhibit Description	Incorporated by Reference			Filed Herewith
		Form	Date	Number	
23.1	Consent of Independent Registered Public Accounting Firm				X
31.1	Certification of Chief Executive Officer pursuant to Rules 13a-14(a) and 15d-14(a) of the Securities Exchange Act of 1934, as amended, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.				X
31.2	Certification of Chief Financial Officer pursuant to Rules 13a-14(a) and 15d-14(a) of the Securities Exchange Act of 1934, as amended, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.				X
32.1**	Certification of Chief Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.				X
32.2**	Certification of Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.				X
97.1	Policy Relating to Recovery of Erroneously Awarded Compensation				X
101	The following financial statements from the Company's Yearly Report on Form 10-K for the fiscal year ended December 31, 2023 formatted in Inline XBRL: (i) Balance Sheets, (ii) Statements of Operations, (iii) Statements of Stockholders' Equity (Deficit) and (vi) Notes to Financial Statements, tagged as blocks of text and including detailed tags.				X
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101)				X

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* Indicates management contract or compensatory plan or arrangement.

** This certification is deemed furnished, and not filed, with the SEC and is not to be incorporated by reference into any filing of Harmony Biosciences Holdings, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Annual Report on Form 10-K, irrespective of any general incorporation language contained in such filing.

\+ Certain portions of this document that the Company customarily and actually treats as private or confidential and is not material have been redacted in accordance with Regulation S-K, Item 601(b)(10)

Item 16. Form 10-K Summary.

None.

Signatures

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

HARMONY BIOSCIENCES HOLDINGS, INC.

By: /s/ Jeffrey M. Dayno
Name: Jeffrey M. Dayno
Title: President, Chief Executive Officer and Director (principal executive officer)

Date: February 22, 2024

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated:

Signature	Title	Date
/s/ Jeffrey M. Dayno Jeffrey M. Dayno	President, Chief Executive Officer and Director (Principal Executive Officer)	February 22, 2024
/s/ Sandip Kapadia Sandip Kapadia	Chief Financial Officer and Chief Administrative Officer (Principal Financial Officer and Principal Accounting Officer)	February 22, 2024
/s/ Jeffrey S. Aronin Jeffrey S. Aronin	Chairman of the Board	February 22, 2024
/s/ Peter Anastasiou Peter Anastasiou	Director	February 22, 2024
/s/ Antonio Gracias Antonio Gracias	Director	February 22, 2024
/s/ R. Mark Graf R. Mark Graf	Director	February 22, 2024
/s/ Jack Bech Nielsen Jack Bech Nielsen	Director	February 22, 2024
/s/ Juan A. Sabater Juan A. Sabater	Director	February 22, 2024
/s/ Gary Sender Gary Sender	Director	February 22, 2024
/s/ Linda Szyper Linda Szyper	Director	February 22, 2024
/s/ Andreas Wicki Andreas Wicki	Director	February 22, 2024

Executive Officers and Board of Directors

Executive Officers

Jeffrey M. Dayno, M.D.
President, Chief Executive Officer

Kumar Budur, M.D., M.S.
Chief Medical Officer

Jeffrey Dierks, MBA
Chief Commercial Officer

Sandip Kapadia, CPA, MBA
Chief Financial Officer and Chief Administrative Officer

Andrew Serafin, JD, MBA
Chief Strategy Officer

Board of Directors

Jeff Aronin
Harmony Founder and Chairman
Paragon Chairman and CEO

Peter Anastasiou
CEO, Capsida Biotherapeutics

Jeffrey M. Dayno, M.D.
Harmony President, Chief Executive Officer

Antonio Gracias
CEO and
Chief Investment
Officer Valor Equity

Mark Graf
Private Investor
Former Chief Financial Officer
Discover Financial Services

Jack Nielsen
Managing Partner, Vivo Capital LLC

Juan Sabater
Partner and Co-President
Valor Equity

Gary Sender
Former Chief Financial Officer
Nabriva plc

Linda Szyper
Former Chief Operating Officer
McCann Health

Dr. Andreas Wicki
CEO
HBM Healthcare Investments