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Medicure Announces Pivotal Phase 3 Trial IND Filing with FDA for Treatment of Seizures Associated with Pyridox(am)ine 5'-phosphate oxidase (PNPO) Deficiency

Winnipeg, Canada, January 7, 2021 – Medicure Inc. ("**Medicure**" or the "**Company**") (TSXV:MPH, OTC:MCUJF), a cardiovascular pharmaceutical company, today announced that through its Barbados subsidiary, Medicure International Inc., it intends to file an Investigational New Drug ("IND") application with the U.S. Food and Drug Administration ("FDA") pertaining to its legacy product Pyridoxal 5'-phosphate ("P5P", also referred to as "MC-1") for the treatment of seizures associated with pyridox(am)ine 5'-phosphate oxidase ("PNPO") deficiency. The majority of patients with PNPO deficiency have mutations in the PNPO gene, which is required for the production of normal levels of P5P. In connection with the IND, the Company will proceed with a Phase 3 clinical trial to treat PNPO deficient patients with a daily dose of MC-1.

The FDA and the European Medicines Agency ("EMA") have both granted a Rare Pediatric Disease Designation to MC-1 for the treatment of seizures associated with PNPO deficiency. Additionally, the FDA has granted Orphan Drug Status and a Rare Pediatric Disease Designation to MC-1 for the treatment of PNPO deficiency.

Under the Creating Hope Act passed into federal law in 2012, the FDA grants a Rare Pediatric Disease Designation for serious and life-threatening diseases that primarily affect children ages 18 years or younger and fewer than 200,000 people in the United States. If a new drug application ("NDA") for MC-1 for patients with PNPO deficiency is approved, the Company may be eligible to receive a priority review voucher ("PRV") from the FDA, which can be redeemed to obtain priority review for any subsequent marketing application.

"MC-1 has the potential to become the first FDA-approved therapy for patients with PNPO deficiency. Receiving Orphan Drug Status and a Rare Pediatric Disease Designation from the FDA is a significant milestone for this program and underscores the critical value of our work," said Dr. Albert D. Friesen, CEO of Medicure and Chair of its Board of Directors. "With this designation, we will work diligently towards FDA approval and the issuance of a PRV in order to expedite realization of the value of MC-1. We are grateful to the FDA and Congress for having enacted this law which helps Medicure and all companies develop innovative drugs for rare pediatric conditions."

About Medicure Inc.

Medicure is a pharmaceutical company focused on the development and commercialization of therapies for the U.S. cardiovascular market. The present focus of the Company is the marketing and distribution of AGGRASTAT® (tirofiban hydrochloride) injection and ZYPITAMAG® (pitavastatin) tablets in the United States, where they are sold through the Company's U.S. subsidiary, Medicure Pharma Inc. Medicure also operates Marley Drug, Inc. ("Marley"), a pharmacy located in North Carolina that offers an Extended Supply mail order drug program serving all 50 states, Washington D.C. and Puerto Rico. Marley is committed to improving the health status of their patients and the communities they serve while reducing overall health care

costs for employers and other health care consumers. For more information visit www.marleydrug.com. To learn more about The Extended Supply Generic Drug Program call 800.286.6781 or email marleydrug@bellsouth.net. For more information on Medicare please visit www.medicure.com. For additional information about AGGRASTAT®, refer to the full [Prescribing Information](#). For additional information about ZYPITAMAG®, refer to the full [Prescribing Information](#).

To be added to Medicare's e-mail list, please visit:
<http://medicare.mediaroom.com/alerts>

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