Mallinckrodt Completes Rolling Submission of New Drug Application to the U.S. Food and Drug Administration (FDA) for Terlipressin for the Treatment of Patients with Hepatorenal Syndrome Type 1 (HRS-1)

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- Terlipressin would be the first FDA-approved treatment option in the United States for adult patients with HRS-1, a life-threatening condition, if approved -

STAINES-UPON-THAMES, United Kingdom, March 17, 2020 /PRNewswire/ -- Mallinckrodt plc (NYSE: MNK), a global biopharmaceutical company, today announced the completion of its rolling submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for terlipressin, an investigational agent being evaluated for the treatment of hepatorenal syndrome type 1 (HRS-1). The company previously announced the rolling submission, which it initiated in February.

HRS-1 is an acute and life-threatening syndrome involving acute kidney failure in people with cirrhosis.[1] The condition has a median survival time of less than two weeks and greater than 80 percent mortality within three months if left untreated.[2,3] At present, there are no approved drug therapies for HRS-1 in the U.S.,[4] and it is estimated to affect between 30,000 and 40,000 patients in the U.S. annually.[5,6]

"Completion of the NDA submission for terlipressin is another important step forward in our goal of bringing the first FDA-approved therapeutic option to patients in the U.S. with HRS-1 – a life-threatening, difficult-to-treat condition[2]," said **Steven Romano, M.D., Executive Vice President and Chief Scientific Officer at Mallinckrodt**. "We look forward to working with the FDA during the regulatory review process in the coming months, and are grateful to the patients, caregivers and investigators who participated in our clinical trial."

In 2005, terlipressin was granted Fast Track designation by the FDA, which provides for expedited review to facilitate development of drugs intended to treat serious or life-threatening conditions and fill an unmet medical need.[7] In 2016, Mallinckrodt and the FDA reached agreement on the Phase 3 CONFIRM trial protocol design and data analysis under the agency's special protocol assessment (SPA) process. A SPA is an advance agreement with the FDA for the acceptability of the clinical design, endpoints and statistical data analyses for a Phase 3 trial before the start of the trial. The submission is a Class 2 resubmission.

The terlipressin NDA is based, in part, on results from the Phase 3 CONFIRM trial, which was the largest-ever prospective study (n=300) conducted in patients with HRS-1, and the culmination of a sustained, 17-year effort to develop terlipressin for potential use in the U.S. and Canada. Initial results were presented at The Liver Meeting® 2019, the annual meeting of the American Association for the Study of Liver Diseases (AASLD).

About Terlipressin
Terlipressin is a potent vasopressin analogue selective for V1 receptors being investigated for the treatment of HRS-1 in the U.S. and Canada. It is an investigational product in these countries as the safety and efficacy have not been established with, nor has approval been granted by, regulatory authorities in either country. Terlipressin is approved for use outside the U.S. and Canada.

ABOUT MALLINCKRODT
Mallinckrodt is a global business consisting of multiple wholly owned subsidiaries that develop, manufacture, market and distribute specialty pharmaceutical products and therapies. The company's Specialty Brands reportable segment's areas of focus include autoimmune and rare diseases in specialty areas like neurology, rheumatology, nephrology, pulmonology and ophthalmology; immunotherapy and neonatal respiratory critical care therapies; analgesics and gastrointestinal products. Its Specialty Generics reportable segment includes specialty generic drugs and active pharmaceutical ingredients. To learn more about Mallinckrodt, visit www.mallinckrodt.com.

Mallinckrodt uses its website as a channel of distribution of important company information, such as press releases, investor presentations and other financial information. It also uses its website to expedite public access to time-critical information regarding the company in advance of or in lieu of distributing a press release or a filing with the U.S. Securities and Exchange Commission (SEC) disclosing the same information. Therefore, investors should look to the Investor Relations page of the website for important and time-critical information. Visitors to the website can also register to receive automatic e-mail and other notifications alerting them when new information is made available on the Investor Relations page of the website.

CAUTIONARY STATEMENTS RELATED TO FORWARD-LOOKING STATEMENTS
This release includes forward-looking statements with regard to terlipressin, including the anticipated regulatory review process and its potential impact on patients. The statements are based on assumptions about many important factors, including the following, which could cause actual results to differ materially from those in the forward-looking statements: satisfaction of regulatory and other requirements; actions of regulatory bodies and other governmental authorities; changes in laws and regulations; issues with product quality, manufacturing or supply, or patient safety issues; and other risks identified and described in more detail in the "Risk Factors" section of Mallinckrodt's most recent Annual Report on Form 10-K and other filings with the SEC, all of which are available on its website. The forward-looking statements made herein speak only as of the date hereof and Mallinckrodt does not assume any obligation to update or revise any forward-looking statement, whether as a result of new information, future events and developments or otherwise, except as required by law.

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