Mallinckrodt Announces U.S. Food and Drug Administration (FDA) Advisory Committee Voted to Recommend Terlipressin for Approval to Treat Patients with Hepatorenal Syndrome Type 1 (HRS-1)

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

- Prescription Drug User Fee Act (PDUFA) target action date is September 12 -

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DUBLIN, July 15, 2020 /PRNewswire/ -- Mallinckrodt plc (NYSE: MNK), a global biopharmaceutical company, today announced that the Cardiovascular and Renal Drugs Advisory Committee of the U.S. Food and Drug Administration (FDA) voted to recommend approval for its investigational agent terlipressin to treat adults with hepatorenal syndrome type 1 (HRS-1) (8 yes; 7 no). HRS-1 is an acute and life-threatening syndrome involving acute kidney failure in people with cirrhosis.[1] Terlipressin is an investigational agent being evaluated for the treatment of HRS-1 in the U.S., and its safety and effectiveness have not yet been established by the FDA.

"Mallinckrodt is pleased with the advisory committee's positive vote in favor of approval for terlipressin, supporting the potential clinical value terlipressin can bring to patients with HRS-1 in need of an approved therapy in the U.S.," said **Steven Romano, M.D., Executive Vice President and Chief Scientific Officer at Mallinckrodt**. "We acknowledge the clinical challenges associated with treating this complex disease in such a critically ill patient population. We are committed to working closely with the FDA as it continues to review our application."

HRS-1 requires a diagnosis of exclusion, and is often a challenge to diagnose in a timely manner.[2] If left untreated, HRS-1 has a median survival time of approximately two weeks and greater than 80 percent mortality within three months.[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 Americans annually.[5,6]

"Terlipressin plus albumin is the recommended standard-of-care therapy for HRS-1 in many other countries, where terlipressin is approved," said **Francois Durand, M.D., on behalf of the International Club of Ascites**. "HRS-1 is one of the most severe complications of end stage liver disease and the favorable vote from the advisory committee is encouraging to U.S. patients in need of treatment."

While recommendations of the advisory committee are not binding, the FDA will consider the advice provided by the committee as part of the New Drug Application (NDA) review. The FDA assigned terlipressin a Prescription Drug User Fee Act (PDUFA) target date of September 12, 2020. The company announced the FDA accepted for review its NDA for terlipressin in April 2020, which was based, in part, on results from the Phase 3 CONFIRM trial. The CONFIRM trial was the largest-ever prospective study (n=300) conducted to assess the safety and efficacy of terlipressin in patients with HRS-1, 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.

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CAUTIONARY STATEMENTS RELATED TO FORWARD-LOOKING STATEMENTS

This release includes forward-looking statements with regard to terlipressin, including the regulatory review process and related timing, as well as 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.

CONTACT

For Media Inquiries
Caren Begun
Green Room Communications
201-396-8551
caren@greenroompr.com

Investor Relations
Daniel J. Speciale, CPA

Vice President, Finance and Investor Relations Officer
314-654-3638
daniel.speciale@mnk.com

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