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Horizon Pharma plc Receives European Commission Approval for RAVICTI(R) (glycerol phenylbutyrate) Oral Liquid for the Treatment of Urea Cycle Disorders in Patients Two Months of Age and Older

Approval Marks Horizon's First Medicine to Receive Centralized Marketing Authorization

DUBLIN, IRELAND -- (Marketwired) -- 11/30/15 -- Horizon Pharma plc (NASDAQ: HZNP), a biopharmaceutical company focused on improving patients' lives by identifying, developing, acquiring and commercializing differentiated and accessible medicines that address unmet medical needs, and its affiliate, Horizon Therapeutics Limited, today announced that the European Commission (EC) has adopted a binding decision to approve RAVICTI® (glycerol phenylbutyrate) Oral Liquid for use as an adjunctive therapy for chronic management of adult and pediatric patients two months of age and older with six subtypes of Urea Cycle Disorders (UCDs). This decision follows the Positive Opinion previously adopted on [September 24, 2015](#) by the Committee for Medicinal Products for Human Use of the European Medicines Agency. The approval authorizes Horizon to market RAVICTI in all 28 Member States of the European Union (EU), and the Centralized marketing authorization will form the basis for recognition by the Member States of the European Economic Area (EEA), namely Norway, Iceland and Liechtenstein, for the product to be placed on the market.

As RAVICTI is a designated orphan medicinal product containing a new active substance, it will benefit from a period of 10 years of regulatory data/market protection with a possibility of extension to 11 years and a period of 10 years of orphan market exclusivity concurrently applied to each of the approved six sub-types of the UCDs.

UCDs are metabolic diseases that affect a specific enzyme or transporter of the urea cycle, causing heightened levels of ammonia in the blood stream. Symptoms of the disorder can begin at any age, with more severe defects beginning early in life. UCD patients may experience episodes, called hyperammonemic crises, when ammonia levels in the blood become excessively high, which can result in irreversible brain damage, coma or death.

"The approval of RAVICTI in the European Union and the European Economic Area represents a significant milestone for Horizon Pharma as we expand our business globally," said Timothy P. Walbert, chairman, president and chief executive officer, Horizon Pharma plc. "With this approval, we will now focus on developing country-by-country plans to make RAVICTI available to people with urea cycle disorders throughout Europe."

About RAVICTI®

RAVICTI is now indicated for use in all 28 Member States of the EU and 3 Member States of the EEA as a nitrogen-binding agent for chronic management of adult and pediatric patients two months of age and older with UCDs who cannot be managed by dietary protein restriction and/or amino acid supplementation alone. RAVICTI must be used with dietary protein restriction and, in some cases, dietary supplements (e.g., essential amino acids, arginine, citrulline and protein-free calorie supplements). RAVICTI was approved by the U.S. Food and Drug Administration in February 2013 for chronic management of UCDs in adult and pediatric patients greater or equal to 2 years of age.

Important Safety Information

The most common side effects are abdominal pain, nausea, diarrhoea, and/or headache. The most frequently reported adverse reactions were diarrhoea, flatulence, and headache (8.8% each); decreased appetite (7.0%), vomiting (6.1%); and fatigue, nausea and, skin odour abnormal (5.3% each). These reactions usually disappear within a few days even if treatment is continued.

It is proposed that RAVICTI be prescribed by physicians experienced in the management of urea cycle disorders.

Detailed recommendations for the use of RAVICTI will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available by the European Medicines Agency on its website in all official European Union languages after the marketing authorization has been granted by the European Commission.

About Horizon Pharma plc

Horizon Pharma plc is a biopharmaceutical company focused on improving patients' lives by identifying, developing, acquiring

and commercializing differentiated and accessible medicines that address unmet medical needs. The Company markets seven medicines through its orphan, primary care and specialty business units. Horizon's global headquarters are in Dublin, Ireland. For more information, please visit www.horizonpharma.com. Follow [@HZNPplc](https://twitter.com/HZNPplc) on Twitter or view careers on our [LinkedIn](#) page.

Forward-Looking Statements

This press release contains forward-looking statements, including statements regarding the potential of RAVICTI to treat UCD patients, the periods of regulatory data/market protection and orphan market exclusivity attaching to the marketing authorization for RAVICTI in the EU/EEA and Horizon Pharma's strategy and plans to commercialize RAVICTI in Europe and continuing to expand its operations in the rest of the world. These forward-looking statements are based on management expectations and assumptions as of the date of this press release, and actual results may differ materially from those in these forward-looking statements as a result of various factors. These factors include risks regarding whether Horizon Pharma will be able to obtain adequate pricing and reimbursement and successfully commercialize RAVICTI in Europe or elsewhere outside of the United States, whether physicians outside of the United States will prescribe RAVICTI, the availability and acceptance of competing products, whether Horizon Pharma is able to maintain regulatory data and market protections and orphan exclusivity for RAVICTI in Europe, whether Horizon Pharma will be able to successfully execute its growth strategy outside of the United States, and other factors described in Horizon's filings with the United States Securities and Exchange Commission, including those factors discussed under the caption "Risk Factors" in those filings. Forward-looking statements speak only as of the date of this press release and Horizon does not undertake any obligation to update or revise these statements, except as may be required by law.

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