PRESS RELEASE

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Sobi™ launches Ravicti® in Europe and advances the care for patients with Urea Cycle Disorders

Swedish Orphan Biovitrum AB (publ) (Sobi™) announces that the company has gained reimbursement for Ravicti®, a new therapy option for the treatment of patients with Urea Cycle Disorders (UCDs) in several EU member states and EEA-countries. Sweden, Denmark, Austria and Germany are the first countries to launch, followed by UK, Spain and the Netherlands.

“We are very pleased that Ravicti is gaining reimbursement in countries across Europe for patients with Urea Cycle Disorders”, said Norbert Oppitz, Head of Specialty Care at Sobi. “The reimbursement enables us to provide the medical community and patients with a sustainable access to the new drug.”

In Europe, Ravicti (glycerol phenylbutyrate) is indicated for chronic management of UCDs in adults and children from the age of two months, when the disease cannot be managed by dietary protein restriction and/or amino acid supplementation alone. UCDs are inborn errors of metabolism comprising a group of inherited deficiencies of one of the enzymes or transporters involved in the urea cycle, which converts ammonia to urea.

UCDs are very rare, serious and life-threatening disorders. Absence or severe dysfunction of the enzymes or transporters involved results in an accumulation of toxic levels of ammonia in the blood and brain of the patients. Elevated ammonia levels can cause coma and irreparable brain damage, potentially resulting in cognitive impairment, seizures, cerebral palsy, and even death if untreated.

For patients with UCD, early treatment and adequate ammonia control are critical for maintaining the intellectual function, preventing neurologic damage and reducing the frequency of hyperammonaemic crises.

“There is a significant medical need for a more effective treatment of UCDs”, said Armin Reininger, Head of Medical & Scientific Affairs at Sobi. “We believe that the introduction of Ravicti offers a new option to manage ammonia levels and will contribute to further advancing the care of patients with UCDs.”

About Urea Cycle Disorders

Urea cycle disorders are inborn errors of metabolism comprising a group of inherited deficiencies of one of the enzymes or transporters involved in the urea cycle, which converts ammonia to urea. They are very rare, serious and life-threatening disorders since absence or severe dysfunction of the enzymes or transporters results in the accumulation of toxic levels of ammonia in the blood and brain of affected patients. Elevated ammonia levels can cause coma related to hyperammonaemia and irreparable brain damage, potentially resulting in cognitive impairment, seizures, cerebral palsy, and even death if untreated. Although no formal estimates of the incidence of UCDs are available, an estimate of 1 in 35,000 births is generally accepted.

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About Ravicti®

Ravicti® (glycerol phenylbutyrate [GPB]) is a medicine indicated for use as adjunctive therapy for chronic management of adult and paediatric patients ≥2 months of age with urea cycle disorders (UCDs) (including deficiencies of carbamoyl phosphate synthetase I (CPS), ornithine carbamoyltransferase (OTC), argininosuccinate synthetase (ASS), argininosuccinate lyase (ASL), arginase I (ARG) and ornithine translocase deficiency hyperornithinaemia-hyperammonaemia homocitrullinuria syndrome (HHH)) 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, protein-free calorie supplements). Ravicti was granted a centralised marketing authorisation by the European Commission on 27 November 2015. Ravicti is approved for use in all 28 Member States of the EU and in three Member States of the European Economic Area (EEA). For full European prescribing information visit the EMA website.

About the Sobi™ and Horizon Pharma plc collaboration

On 7 December 2016, Sobi and Horizon Pharma plc signed a five-year distribution agreement for Ravicti® (glycerol phenylbutyrate) in European countries, including United Kingdom, Germany, France, Italy and Spain and for Ammonaps® (sodium phenylbutyrate) in the same European countries and certain Middle Eastern countries. Under the agreement, Sobi will have exclusive marketing, sales and distribution rights for the two medicines in the territory until 31 December 2021. Sobi and Horizon also have a distribution agreement for Ravicti in the Middle East that was signed in 2013.

About Sobi™

Sobi™ is an international speciality healthcare company dedicated to rare diseases. Our vision is to be recognised as a global leader in providing access to innovative treatments that make a significant difference for individuals with rare diseases.
The product portfolio is primarily focused on treatments in Haemophilia and Specialty Care. Partnering in the development and commercialisation of products in specialty care is a key element of our strategy. Sobi has pioneered in biotechnology with world-class capabilities in protein biochemistry and biologics manufacturing. In 2017, Sobi had total revenues of SEK 6.5 billion and approximately 850 employees. The share (STO:SOBI) is listed on Nasdaq Stockholm. More information is available at www.sobi.com.

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