PRESS RELEASE

Stockholm, Sweden, 30 January 2019

Ravicti® now available for all ages in Europe following approval of the expanded paediatric indication

Swedish Orphan Biovitrum AB (publ) (Sobi™) announces that Ravicti®, a therapy option for the treatment of patients with urea cycle disorders (UCDs), has received approval from the European Commission on 18 December 2018 for the paediatric indication (birth to two months of age) following a positive CHMP opinion from the European Medicines Agency (EMA).

The previous indication for Ravicti (glycerol phenylbutyrate) was restricted to children aged more than two months.

"This new expanded indication allows all patients access to treatment, which is particularly significant as the disease often presents in newborns," says Norbert Oppitz, Head of Specialty Care at Sobi. "Following stabilisation with acute intravenous therapy, children can now be managed long-term on Ravicti in the early days of their lives."

The approval was based on a multicentre, open-label clinical study of the safety, efficacy and pharmacokinetics of glycerol phenylbutyrate in paediatric subjects under two years of age with UCDs, carried out by Horizon Pharma plc (Nasdaq: HZNP). The study, which included a cohort of 16 patients from birth to two months, demonstrated that glycerol phenylbutyrate is safe and effective in establishing and maintaining ammonia control in paediatric UCD subjects aged from birth to two years.

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

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.

About Ravicti®

Ravicti® (glycerol phenylbutyrate [GPB]) is a medicine indicated for use as adjunctive therapy for chronic management of patients 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/Immedica Group collaboration

On 7 December 2016, Sobi and Horizon Pharma plc entered into a distribution agreement for Ravicti® (glycerol phenylbutyrate) and for Ammonaps® (sodium phenylbutyrate) in certain European countries, and for Ammonaps also in certain Middle Eastern countries. Sobi and Horizon also had a distribution agreement for Ravicti in the Middle East that was signed in 2013, the rights of which were later incorporated into the 2016 distribution agreement. On 28 December 2018, Horizon sold the rights to Ravicti® and Ammonaps® (known as Buphenyl® in the United States) outside of North America and Japan to Medical Need Europe AB, part of the Immedica Group, based in Stockholm, Sweden.

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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