PRESS RELEASE

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First patient dosed in phase 1/2 study evaluating SOBI003 for treatment of mucopolysaccharidosis type IIIA (MPS IIIA)

Swedish Orphan Biovitrum AB (publ) (Sobi™) announces that the first patient has been dosed in the phase 1/2 study SOBI003-001. The study is an open-label, non-controlled, multiple-dose study with the objective of assessing the safety, tolerability and efficacy of SOBI003 in nine children aged 1-6 years for the treatment of mucopolysaccharidosis type IIIA (MPS IIIA), also known as Sanfilippo A syndrome.

"Since there is currently no treatment available for MPS IIIA, the initiation of this study is an important first step towards finding a potential treatment for this debilitating disease," says Milan Zdravkovic, Chief Medical Officer and Head of Research & Development at Sobi.

"I am very pleased that we were able to enrol the first patient in this important study. We look forward to learning more about how SOBI003 may potentially be able to help patients in the future as we enrol more patients into the study," says Dr. Paul Harmatz, UCSF Benioff Children's Hospital in Oakland California.

The product candidate SOBI003 has been developed in-house by Sobi and is a chemically modified variant of recombinant human sulfamidase, using Sobi's proprietary glycan modification technology Modifa™. SOBI003 has been granted orphan designation by the European Commission and by the US Food and Drug Administration (FDA) for MPS IIIA. The FDA granted Fast Track status in early 2018.

About mucopolysaccharidosis type IIIA (MPS IIIA) (Sanfilippo A syndrome)

MPS IIIA or Sanfilippo A syndrome is a progressive, life-threatening and rare inherited metabolic disorder affecting children from a young age. MPS IIIA belongs to a group of diseases called lysosomal storage disorders (LSDs). In MPS IIIA, the body is unable to break down long chains of sugar molecules called heparan sulfate, resulting in the accumulation of heparan sulfate in lysosomes. MPS IIIA mainly affects the central nervous system where it causes severe progressive degeneration.[i]

Up to about 2,000 people are estimated to live with MPS IIIA in the EU and US. The disease is usually identified at three to four years of age and the life-span of an affected child does not usually extend past the end of the second or beginning of the third decade. There is no treatment for MPS IIIA to date.

About SOBI003

The product candidate SOBI003 is a chemically modified variant of recombinant human sulfamidase, using Sobi's proprietary glycan modification technology Modifa™, intended as an enzyme replacement therapy to reduce heparan sulfate storage materials in affected cells. SOBI003 is taken up by cells and transported into the lysosomal compartment where heparan sulfate is degraded. The modification of the molecule results in an extended half-life.

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SOBI003 was granted orphan designation by the European Commission for MPS IIIA in October 2016 and by the US Food and Drug Administration (FDA) in June 2017. In January 2018, the FDA accepted the investigational new drug application (IND) and granted Fast Track status for SOBI003.

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 transform lives 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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[i] Valstar et al. Ann Neurol. 2010;68(6):876-87