

## PRESS RELEASE

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### FDA grants SOBI003 Orphan Drug Designation for the treatment of MPS IIIA

[Swedish Orphan Biovitrum AB \(publ\)](#) (Sobi™) has been granted Orphan Drug Designation (ODD) by the U.S. Food and Drug Administration (FDA) for the company's development candidate SOBI003, a chemically modified human recombinant sulfamidase for the treatment of mucopolysaccharidosis type IIIA (MPS IIIA), also known as Sanfilippo syndrome type A, a rare metabolic disorder.

"We are very pleased with the orphan drug designation from the FDA for SOBI003. MPS IIIA is a severe and debilitating disease, and this development program is an important part of our mission of being pioneers in rare diseases. We are currently in the late pre-clinical phase and expect to initiate the first clinical trial with SOBI003 in 2018," said Milan Zdravkovic, Chief Medical Officer and Head of Research and Development at Sobi.

Onset of Mucopolysaccharidosis type IIIA is in early childhood, and the disease is characterised by severe and progressive developmental delay, motor retardation and eventually dementia. One in 100,000 children are born with the inherited condition and very few patients survive into adulthood. There is presently no treatment for MPS IIIA. SOBI003 was granted orphan designation by the European Commission for MPS IIIA in October 2016.

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#### About Orphan Drug Designation

The Orphan Drug Designation programme provides orphan status to drugs and biologics which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the U.S., or that affect more than 200,000 persons but are not expected to recover the costs of developing and marketing a treatment drug. The granting of an orphan designation request does not alter the standard regulatory requirements and process for obtaining marketing approval. Orphan designation qualifies the sponsor of the drug for various development incentives of the Orphan Drug Act, such as a 50 per cent tax credit on the cost of clinical trials undertaken in the US and a seven-year period of marketing exclusivity following market approval.<sup>i</sup>

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

MPS IIIA or Sanfilippo A syndrome is a progressive, life-threatening and rare inherited metabolic disorder affecting children already 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.<sup>ii</sup>

Approximately 1,000-2,000 persons are estimated to live with MPS IIIA in the E.U. and U.S. The disease is usually identified at around four years of age and the life-span of an affected child does not usually extend beyond the third decade of life. There is no treatment for MPS IIIA to date.

#### **About SOBI003**

SOBI003 is a chemically modified variant of a recombinant human sulfamidase product candidate, using Sobi's proprietary glycan modification technology, 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.

#### **About Sobi™**

Sobi is an international specialty healthcare company dedicated to rare diseases. Sobi's mission is to develop and deliver innovative therapies and services to improve the lives of patients. The product portfolio is primarily focused on Haemophilia, Inflammation and Genetic diseases. Sobi also markets a portfolio of specialty and rare disease products across Europe, the Middle East, North Africa and Russia for partner companies. Sobi is a pioneer in biotechnology with world-class capabilities in protein biochemistry and biologics manufacturing. In 2016, Sobi had total revenues of SEK 5.2 billion (USD 608 M) and about 760 employees. The share (STO: SOBI) is listed on Nasdaq Stockholm. More information is available at [www.sobi.com](http://www.sobi.com).

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<sup>i</sup> FDA: Developing Products for Rare Diseases & Conditions.

<https://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/default.htm>

<sup>ii</sup> Valstar et al. Ann Neurol. 2010;68(6):876-87