

PRESS RELEASE

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European Commission grants SOBI003 orphan designation for the treatment of MPS IIIA

[Swedish Orphan Biovitrum AB \(publ\)](#) (Sobi™) (STO: SOBI) has been granted orphan designation by the European Commission (EC) for the company's development product candidate SOBI003, a chemically modified human recombinant sulfamidase for the treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome). SOBI003 will be included in the EU Community Register of Orphan Medicinal Products.

MPS IIIA or Sanfilippo A syndrome is an inherited systemic lysosomal storage disease with a significant central nervous system component. Onset is in early childhood and the disease is characterised by severe and progressive development delay including behavioural problems, motor retardation and eventually dementia. Very few patients survive into their twenties. There is presently no treatment for MPS IIIA.

"MPS IIIA is a severe and debilitating disease with devastating consequences for patients. We hope that SOBI003 will provide these patients with a treatment where there is none today," said Stephen James, Head of Research and Translational Sciences at Sobi.

Sobi is in the late stages of preclinical development of SOBI003. Preclinical studies to date with repeated systemic infusions have demonstrated efficacy in reducing substrate levels in the brain and signs of disease modifying effects. Sobi is preparing for clinical studies, which it plans to start in 2018. Clinical studies will focus on exploring the safety and efficacy of SOBI003 in MPS IIIA patients.

About orphan designation

To be granted orphan designation in the EU, the new investigational product must be for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition that affects no more than five in 10,000 people in the EU and for which no satisfactory treatments exist or, where they do exist, where the new medicine will be of significant benefit to those affected by that condition. It can also be granted if a new product is not expected to make a sufficient return to justify the investment.

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 heparan sulfate accumulation in lysosomes. MPS IIIA mainly affects the central nervous system where it causes severe progressive degeneration.¹

Approximately 1,000-2,000 persons are estimated to live with MPS IIIA in the EU and US. 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.

Swedish Orphan Biovitrum AB (publ)

Postal address SE-112 76 Stockholm, Sweden

Phone: +46 697 20 00 www.sobi.com

About SOBI003

SOBI003 is a chemically modified variant of a recombinant human sulfamidase product candidate 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, which may enable transport of SOBI003 over the blood brain barrier and facilitate uptake of SOBI003 in to the brain.

About Sobi™

Sobi™ is an international speciality healthcare company dedicated to rare diseases. Our mission is to develop and deliver innovative therapies and services to improve the lives of patients. The product portfolio is primary focused on Haemophilia, Inflammation and Genetic diseases. We also market a portfolio of speciality 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 2015, Sobi had total revenues of SEK 3.2 billion (USD 385 M) and approximately 700 employees. The share (STO:SOBI) is listed on NASDAQ OMX Stockholm. More information is available at www.sobi.com.

For more information please contact**Media relations**

Linda Holmström, Senior Communications Manager
+46 708 734 095

linda.holmstrom@sobi.com

Investor relations

Jörgen Winroth, Vice President, Head of Investor Relations
+1 347-224-0819, +1 212-579-0506, +46 8 697 2135

jorgen.winroth@sobi.com

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