

## PRESS RELEASE

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### **Sobi: Kiobrina pivotal phase 3 study did not meet primary endpoint**

**Swedish Orphan Biovitrum AB (publ) (Sobi) today announced topline data from the company's pivotal phase 3 study of its enzyme therapy Kiobrina (rhBSSL – recombinant human Bile Salt Stimulated Lipase). The primary endpoint of the study – growth velocity measured after four weeks of treatment with rhBSSL – was not met. No statistically significant improvement in growth velocity was demonstrated in preterm infants treated with rhBSSL compared to placebo.**

Topline results from the multi-centre phase 3 European trial of rhBSSL, called the LAIF study (Lipase Added to Infant Feeding), showed that in 410 infants born before 32 weeks of gestational age, the mean growth velocity did not differ when treated with rhBSSL (16.8 g/kg/day) compared to placebo (16.6 g/kg/day). The estimated difference in growth velocity was 0.21 g/kg/day (95% CI (-0.40; 0.83),  $p=0.49$ ). The growth velocity was comparable between groups throughout the treatment period, and the median enteral feeding volume was 2.7 mL/kg/day lower in the rhBSSL group. Ninety-eight per cent of infants completed the four week treatment period.

“The LAIF study was well designed and conducted, and will make a significant contribution to neonatology in the area of growth and development. Although the results are not what we expected, particularly regarding the growth of the placebo group, LAIF demonstrates that a large scale randomised clinical study can be conducted in this important field,” said Geoffrey McDonough, President and CEO at Sobi. “We will assess the remaining dataset from LAIF, including the fatty acids profiles and the pending neurocognitive assessments. Today the topline results do not support initiation of the US trial.”

The study also included several secondary endpoints. No significant differences between the rhBSSL and placebo groups were observed in the initial analysis of head circumference (3.92 cm compared to 3.88 cm; 95% CI (-0.15; 0.24),  $p=0.66$ ) and readiness for hospital discharge (45.1 compared to 45.2 days; 95% CI (-3.2; 2.9)  $p=0.93$ ).

A higher incidence of adverse events was observed during the four weeks treatment period in the rhBSSL treated infants, with signs of gastrointestinal intolerance and potential cases of necrotising enterocolitis (NEC). Further investigation is required to better understand these findings.

“The overall incidence of adverse events reported in the treatment group of the study for these very vulnerable infants appears consistent with what is seen in the day-to-day clinical practice in a neonatal intensive care unit,” said Charlotte Casper, International Coordinating Investigator and Professor of

Neonatology, Toulouse, France. “However the imbalance between the groups is not fully understood at this point and will need further assessment.”

Although the overall result was negative, infants small for gestational age (SGA) (n=62), demonstrated higher growth velocity compared to placebo (17.1 g/kg/day vs. 15.1 g/kg/day, with an estimated difference of 1.95; 95% CI (0.38; 3.52)). SGA babies have a birthweight below the tenth percentile for gestational age, and were a pre-specified subgroup. Further analysis is required to determine the relevance of this finding.

“This pivotal phase 3 trial of rhBSSL was conducted in more than 50 centres across ten countries in Europe, and included more than 400 preterm infants with balanced baseline demography between groups,” said Birgitte Volck, MD, PhD, Senior Vice President and Chief Medical Officer. “The study was designed to evaluate the efficacy, safety and tolerability of rhBSSL and was powered to allow for the detection of even small differences in the primary endpoint, growth velocity, measured after four weeks of treatment. Of the secondary endpoints we have analysed only part of the data so far. In addition, an assessment of neurodevelopment at 12 months for the full cohort of infants remains to be completed.”

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#### **About Sobi**

Sobi is an international specialty 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 primarily focused on Inflammation and Genetic diseases, with three late stage biological development projects within Haemophilia and Neonatology. We also market a portfolio of specialty and rare disease products for partner companies. Sobi is a pioneer in biotechnology with world-class capabilities in protein biochemistry and biologics manufacturing. In 2013, Sobi had total revenues of SEK 2.2 billion (€253 M) and about 550 employees. The share (STO: SOBI) is listed on NASDAQ OMX Stockholm. More information is available at [www.sobi.com](http://www.sobi.com).

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