

## PRESS RELEASE

Stockholm, 23 September, 2013



### **Sobi receives positive CHMP opinion for Kineret® for treatment of rare disease CAPS**

Swedish Orphan Biovitrum AB (publ) (Sobi) announced today that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion for the use of Kineret for the treatment of patients with Cryopyrin-Associated Periodic Syndromes (CAPS).

The opinion is based on a positive benefit risk assessment of the results from a long-term treatment study in children and adults with NOMID/CINCA, the most severe form of CAPS.

“We look forward to final decision from the European Commission in the coming months”, said Birgitte Volck, Senior Vice President and Chief Medical Officer for Sobi. “CAPS is a group of rare inherited inflammatory disorders with symptoms involving fever, rash, joint and muscle pain, fatigue and eye manifestations which in the more severe cases are potentially life threatening.”

“If approved, Kineret will increase treatment flexibility and opportunities to tailor therapy to patients’ individual needs, including initiation of early treatment”, said PD Dr med Jasmin Kümmerle-Deschner at the Universitätsklinikum Tübingen in Germany.

In December 2012, Kineret became the first and only FDA-approved therapy for NOMID/CINCA. Sobi will provide Kineret for home treatment in a prefilled syringe with a graduated label to allow flexible dosing in children.

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### About CAPS

Cryopyrin associated periodic syndromes (CAPS) are a group of rare inherited autoinflammatory diseases caused by autosomal dominant mutations in a gene called NLRP3. CAPS is characterized by uncontrolled overproduction of IL-1beta. IL-1 induces a number of inflammatory responses such as fever, pain sensitization, bone and cartilage destruction and acute plasma protein responses. In the most severe form of CAPS, called Neonatal-Onset Multisystem Inflammatory Disease (NOMID) or Chronic Infantile Neurologic Cutaneous and Arthritis syndrome (CINCA), it is associated with increased mortality and fever, rash, chronic aseptic meningitis, sensorineural hearing loss, craniofacial abnormalities, and bone lesions. When of intermediate severity, called Muckle-Wells Syndrome (MWS), the disease is typically associated with episodic, intense and enduring flares and morbidity, including progressive hearing loss and kidney failure secondary to amyloidosis (a condition where amyloid proteins are deposited in organs and/or tissues). The mildest form, called Familial Cold Autoinflammatory Syndrome (FCAS), presents with cold-induced episodes of fever, rash and malaise. The incidence of CAPS is estimated to be 1:1,000,000 worldwide.

### About Kineret (anakinra)

Kineret is a recombinant protein drug approved for, and the reduction in signs and symptoms and slowing the progression of structural damage in moderately to severely active rheumatoid arthritis (RA) in combination with methotrexate, in patients 18 years of age or older who have had an inadequate response to methotrexate alone. Kineret blocks the biological activity of IL-1 by binding to the interleukin-1 type 1 receptor, expressed in a wide variety of tissues and organs. IL-1 is a key mediator of inflammation and driver of autoinflammatory diseases. For more information on Kineret see the Prescribing Information. ([www.kineretrx.com](http://www.kineretrx.com))

In early 2013, Sobi announced that the US Food and Drug Administration (FDA) had approved Kineret for the treatment of children and adults with neonatal-onset multisystem inflammatory disease (NOMID), the most severe form of CAPS. Kineret thereby became the first and only FDA-approved therapy for NOMID (CAPS).

### 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 2012, Sobi had total revenues of SEK 1.9 billion (€ 215 M) and about 500 employees. The share (STO: SOBI) is listed on NASDAQ OMX Stockholm. More information is available at [www.sobi.com](http://www.sobi.com).

### For more information – not for publication

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