FDA approves Kineret for the treatment of NOMID

Sobi (STO: SOBI) today announced that the US Food and Drug Administration (FDA) has approved Kineret® (anakinra) for the treatment of children and adults with neonatal-onset multisystem inflammatory disease (NOMID). Kineret® is the first and only FDA-approved therapy for NOMID, the most severe form of cryopyrin associated periodic syndromes (CAPS).

This is the first approval allowing the use of Kineret in children. Kineret was approved for NOMID under an Orphan Drug designation. A priority review was granted by the FDA based on the product’s potential to provide a significant advance in therapy for the NOMID patient population where no adequate therapy exists. Sobi will provide a prefilled syringe with a graduated label to allow flexible dosing in children. Kineret has been approved for the reduction of signs and symptoms of Rheumatoid Arthritis (RA) in adults since 2001.

CAPS is, in its most severe form of NOMID, a life-long and severely debilitating disease. The disease is associated with an overproduction of an immune system protein known as interleukin-1 (IL-1). Untreated patients develop progressive hearing and vision loss, variable degrees of cognitive impairment and joint contractures. Treatment of NOMID patients for 5 years with Kineret demonstrated that, in addition to controlling the daily symptoms of the disease such as fever, rash, headache and joint pain, important central nervous system (CNS) functions such as hearing and vision remain stable and do not progress on treatment. “In order to prevent organ damage that results from untreated disease, we have learned that early diagnosis and the initiation of IL-1 blocking therapy are critical,” said Dr. Raphaela Goldbach-Mansky, the principal investigator of the NOMID study that was conducted at the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS), part of the National Institutes of Health (NIH) in Bethesda, MD, USA.

Dr. Geoffrey McDonough, CEO of Sobi, said “The FDA approval of Kineret for NOMID is a significant result from a long-term collaboration with the NIH and patient societies, and marks an important milestone in our effort to bring therapeutics options to patients with rare inflammatory diseases. We look forward to making Kineret more widely available to patients with NOMID in the United States this year.”

About CAPS and NOMID

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 NOMID, also called chronic infantile neurologic cutaneous and arthritis syndrome (CINCA) in Europe, it is associated with increased mortality and fever, rash, chronic aseptic meningitis, sensorineural hearing loss, craniofacial abnormalities, and bone lesions. When of intermediate severity, 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 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 the treatment of children and adults with NOMID, and the reduction in signs and symptoms and slowing the progression of structural damage in moderately to severely active rheumatoid arthritis (RA), in patients 18 years of age or older who have failed one or more disease modifying antirheumatic drugs (DMARDs). 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 in both adults and children. For more information on Kineret see the Prescribing Information. (www.kineretrx.com)

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About Swedish Orphan Biovitrum (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 hemophilia and neonatology. We also market more than 40 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 2011, Sobi had total revenues of SEK 1.9 billion (€ 214 M) and about 500 employees. The share (STO: SOBI) is listed on NASDAQ OMX Stockholm. More information is available at www.sobi.com.