PRESS RELEASE

Stockholm, Sweden, 18 September 2020

Orfadin® (nitisinone) receives positive opinion from CHMP for treatment of AKU

Swedish Orphan Biovitrum AB (publ) (Sobi™) today announces that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion for Orfadin® (nitisinone) for the treatment of adult patients with alkaptonuria (AKU), the first described human genetic disease. The opinion is now referred to the European Commission for a decision.

AKU is a serious, multifaceted, debilitating and slowly progressive disease affecting approximately 1 in every 250 000 to 1 million people. Also known as Black Bone Disease or Black Urine Disease due to the disease characteristics, it is an extremely rare genetic condition, which can cause significant damage to the bones, cartilage and tissue which eventually leads to joint disease. The medical need is high as there is currently no pharmacological treatment available. Current therapy primarily consists of palliative analgesia and arthroplasty.

“Today’s announcement is the result of a ground-breaking collaboration between a pharmaceutical company, academia and a patient organisation. AKU is an iconic disease which has had no licensed treatment for over a century, so today represents a pivotal milestone for the international patient community,” says Dr Nicolas Sireau, CEO and Chairman of the AKU Society and father of two sons with AKU.

The opinion is based on the scientific results of the DevelopAKUre clinical development programme, a research consortium initiated by the AKU Society and clinical experts. DevelopAKUre is an award-winning example of patient-centric collaboration on a European scale in order to treat an ultra-rare disease.

“We are very proud of the collaboration between Sobi and the DevelopAKUre partners, especially the AKU Society, which has resulted in today’s announcement. This recommendation, if approved by the European Commission, would help address a medical need that has been unmet for people with AKU since the discovery of the condition more than 120 years ago,” says Ravi Rao, Chief Medical Officer and Head of Research and Development at Sobi.

The DevelopAKUre programme included a long-term, international phase 3 clinical efficacy study in 138 patients demonstrating that nitisinone 10 mg per day was well tolerated and shown to be effective in reducing urinary excretion of the causal agent of alkaptonuria, homogentisic acid (HGA), resulting in a reduction of the disease process (ochronosis) and clinical signs, indicating a slower disease progression in adult patients with AKU. The study was recently published in the Lancet Diabetes and Endocrinology.[1]

About Orfadin®

Orfadin®(nitisinone) is currently approved for treatment of hereditary tyrosinemia type 1 (HT-1). Orfadin is a proprietary product and is developed by and made available globally by Sobi. It was originally developed as a treatment by Swedish scientists and approved by the FDA in 2002 and the EMA in 2005.

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[1] Ranganath et al. *Lancet Diabetes Endocrinol* 2020; 8: 762–72

Before Orfadin became available, the survival rate in HT-1 was 29 per cent after two years for children who developed symptoms before two months of age[2] After the introduction of Orfadin, the survival rate is 93 per cent after two years in patients with treatment initiation before two months of age.[3]

For full European prescribing information, please visit the EMA website.

About the DevelopAKUre

DevelopAKUre was a clinical development programme designed to assess whether nitisinone is an effective treatment for AKU. It was led by a consortium of 13 partners across Europe, including the coordinator and main sponsors (Royal Liverpool University Hospital / University of Liverpool), Sobi and the AKU Society. The programme was supported by European Commission Seventh Framework Programme funding granted in 2012 (project number: 304985).

About Sobi™

Sobi is a specialised international biopharmaceutical company transforming the lives of people with rare diseases. Sobi is providing sustainable access to innovative therapies in the areas of haematology, immunology and specialty indications. Today, Sobi employs approximately 1,400 people across Europe, North America, the Middle East, Russia and North Africa. In 2019, Sobi's revenue amounted to SEK 14.2 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. You can find more information about Sobi at www.sobi.com.

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[2] van Spronsen FJ, Thomasse Y, Smit GP, et al. Hepatology. 1994;20(5): 1187-1191

[3] Orfadin EPAR: Product information 26/01/2017