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Dipharma
Better Together.
Improved Solutions for Inborn Metabolic Diseases.

DIPHARMA ANNOUNCES THE VALIDATION OF DISANIT® (NITISINONE) MARKETING AUTHORIZATION APPLICATION IN EUROPE

The first stable capsule formulation of Nitisinone for the treatment of hereditary Tyrosinemia type-1 (HT-1) is now under marketing authorization (MA) evaluation in the EU.

Chiasso, Switzerland, February 23rd, 2017 – Dipharma S.A. announces the validation of the MA application for its product Disanit® (Nitisinone capsules 2, 5 and 10 mg) in the EU. The Swiss company also announces plans to file an Abbreviated New Drug Application (ANDA) with the US FDA.

Disanit® is the improved generic version of SOBI's Orfadin®, which is indicated for the treatment of hereditary tyrosinaemia type 1 (HT-1), a very rare inborn error of metabolism (IEM). Disanit® is a stable capsule formulation that can be stored at room temperature without the need of refrigeration.

Disanit® is the third product of Dipharma undergoing a regulatory marketing authorization procedure: Diterin® (sapropterin dihydrochloride 100 mg tabs) has already been approved in South Korea and Russia for the treatment of hyperphenylalaninemia (HPA) due to phenylketonuria (PKU) and Miglustat (miglustat 100 mg caps) was submitted in the USA through an abbreviated new drug application (ANDA) in 2016 for the treatment of Gaucher disease. Dipharma also confirms that the development of several other products for the treatment of rare metabolic diseases is on- going.

"Once approved, Disanit® will be the first capsule formulation of Nitisinone in Europe that can be stored at room temperature, simplifying distribution, decreasing cold-chain related wastage and finally improving ease of use for the patients" commented Marc-Olivier Geinoz, CEO of Dipharma S.A. "*This regulatory filing is the perfect exemplification of our mission: in Dipharma we strive to improve the life of patients suffering from inborn errors of metabolism. We aim to provide more value to patients and health professionals, at an affordable cost for payers."*

Dipharma S.A. also announces that international patent applications were filed claiming a stable crystalline form of Nitisinone, stable capsule formulations of Nitisinone and the proprietary technology developed to improve the product stability, without modifying the pharmaceutical form the patients are familiar with.

Dipharma S.A. is currently working with its partners to make Disanit® and all the rest of its product portfolio available to patients worldwide.

About Disanit®

Disanit® (nitisinone 2, 5 and 10 mg) is the first generic oral drug stable at room temperature currently under review by EU authorities for the treatment of hereditary tyrosinaemia type 1 (HT-1).

HT-1 is a rare inborn error of metabolism (IEM) in which the body is unable to completely break down the amino acid tyrosine, and so harmful substances are formed, causing serious liver problems and liver cancer. HT-1 is progressive and can be fatal if untreated. For more information on HT-1, please visit http://www.dipharma.ch/rare-diseases/.

Disanit® is to be used together with a diet that restricts the intake of the amino acids tyrosine and phenylalanine. These amino acids are normally found in proteins in foods and drinks.

Disanit® was developed by Dipharma S.A., Switzerland. Disanit® is a trademark owned by Dipharma S.A.

Disanit® will be entirely manufactured and packaged in the EU.

About Dipharma

Dipharma S.A. is a Swiss pharmaceutical company specialized in developing high quality, improved, medicines for rare diseases. Dipharma S.A. is part of a third generation group of family companies that have grown to a global presence.

With a portfolio of products for the treatment of Phenylketonuria, Gaucher Disease, Niemann Pick Type C, Hereditary Tyrosinemia Type 1, Urea Cycle Disorders and others, Dipharma SA works every day to provide improved solutions for people affected by inborn metabolic diseases at an affordable cost and with a global reach.

For more information, please visit www.dipharma.ch

Orfadin® is a registered trademark of Swedish Orphan Biovitrum