EffRx Pharmaceuticals Signs Exclusive License Agreement with Dipharma SA and Launches Miglustat Dipharma in Switzerland

FREIENBACH, Switzerland – EffRx Pharmaceuticals SA, a commercial-stage company that commercializes niche and orphan medicines in Switzerland and Europe, today announced it has recently entered into an exclusive license agreement with Dipharma SA, a Swiss specialty pharmaceutical company, developing high quality, improved medicines for rare diseases. Under the terms of the agreement, EffRx has received exclusive rights to commercialize Miglustat Dipharma in Switzerland.

Miglustat Dipharma is a generic equivalent to Actelion's (Johnson & Johnson's) Zavesca®[1]. It is indicated for the oral treatment of adult patients with mild to moderate type 1 Gaucher disease for whom enzyme replacement therapy is unsuitable, as well as for the treatment of progressive neurological manifestations in adult and pediatric patients with Niemann-Pick type C disease.
Gaucher disease and Niemann-Pick disease belong to a larger group of more than 50 disorders known as lysosomal storage disorders. They are inherited metabolic diseases that are characterized by an abnormal build-up of various toxic materials in the body's cells as a result of enzyme deficiencies.

Patients with mild to moderate type 1 Gaucher disease lack an enzyme called glucocerebrosidase, which results in a glycosphingolipid called glucosylceramide building up in different parts of the body, such as the spleen, liver and bones. Miglustat Dipharma is used in patients who cannot receive the standard treatment of enzyme replacement therapy. Niemann-Pick type C is a potentially fatal disease in which glycosphingolipids build up within cells in the brain and elsewhere in the body. Miglustat Dipharma is used to treat the neurological symptoms of the disease.

The addition of Miglustat Dipharma marks another milestone for EffRx Pharmaceuticals' growing portfolio of niche and orphan medicines in Switzerland. After having secured the rights for innovative therapies to treat conditions like cystic fibrosis and pediatric adrenal insufficiency, EffRx is thrilled to be able to launch a product which has an established place in the treatment of two major lysosomal storage disorders.

Miglustat Dipharma is reimbursed in Switzerland as of 1 November 2020.[2] It provides a therapeutic alternative for patients and important cost savings for the Swiss healthcare system.

About EffRx Pharmaceuticals

EffRx Pharmaceuticals is a commercial-stage pharmaceutical company focused on the late stage development and commercialization of prescription medications for niche and orphan indications. The business model is centered around providing superior clinical and commercial value propositions for physicians, payers and patients.
EffRx pro-actively seeks in-licensing opportunities for Europe in niche therapeutic areas, with a primary interest for rare diseases, where EffRx has received an orphan drug designation (ODD) from the FDA for a pipeline asset.

EffRx's go-to-market competence is proven by the development, launch and lucrative expansion of Binosto® in a highly competitive European market. Our lead commercialized product, Binosto® for the treatment of osteoporosis, is marketed in the US as well as selected European and Asian countries.

About Dipharma SA

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

With a portfolio of generic orphan products for the treatment of Phenylketonuria, Gaucher Disease, Hereditary Tyrosinemia Type 1, Urea Cycle Disorders and others, Dipharma S.A. provides improved solutions for patients affected by inborn metabolic diseases at an affordable cost and with a global reach.

For more information, please visit www.dipharma.ch
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1 Zavesca® is a registered trademark of Actelion Pharmaceuticals Ltd.
2 Reimbursed for the treatment of adult patients with mild to moderate type 1 Gaucher disease for whom enzyme replacement therapy is not possible.