PRESS RELEASE

August 23, 2013

OrphanPacific, Inc.
Orphan Europe SARL

"Normosang® Infusion 250 mg (nonproprietary name: human hemin)" for the treatment of acute hepatic porphyria; an uncommon but life-threatening medical emergency

On August 23, 2013 OrphanPacific, Inc. (OP) launched, "Normosang® Infusion 250 mg (nonproprietary name: human hemin)" (R&D Code: CMK-001) for the treatment of acute hepatic porphyria, in Japan.

The porphyrias are a hereditary group of disorders, each of which is related to a deficiency in one of the enzymes involved in the biosynthesis of heme. The acute porphyrias [acute intermittent porphyria (AIP), variegate porphyria (VP) and hereditary coproporphyria (HCP)] are rare, autosomal dominant, and characterised by episodic acute neurovisceral attacks which may be life threatening. Symptoms of an acute attack may include severe abdominal pain, constipation, nausea, hypertension, tachycardia and convulsion. Based on European studies, the annual incidence of the most common acute porphyria, acute intermittent porphyria (AlP), is 0.13 per million and the prevalence of patients with current or past symptoms is 5.9 per million. The nationwide epidemiological survey on hereditary porphyria in 2010 reported that there are an estimated 36 patients currently hospitalized in Japan.

Normosang is an established treatment in Europe since 1985. It suppresses production of intermediate metabolites of porphyrin that leads to acute porphyria. It should be given, soon after the onset of an attack, to any patient with severe symptoms (severe pain, vomiting), or who shows complications such as seizures, hyponatraemia, or incipient neuropathy, and also to any patient with a history of porphyria attacks complicated by neuropathy. Most patients improve within 5 days.

An agreement between Orphan Europe SARL and CMIC HOLDINGS Co., Ltd. was established in 2008 for the development of this much awaited treatment in Japan. In June 2013, the Marketing Authorisation of Normosang was transferred to OP.

End

Glossary

【Orphan Drug】

Treatment drug for a rare and severe disease affecting fewer than 50,000 patients in Japan, defined in the Article 77-2 of the Pharmaceutical Affairs Law

【Orphan Pacific, Inc. 】

The company was established in 2012, as a joint venture between Japan's largest CRO, CMIC Holdings Co., and Japan's largest wholesale distributor of ethical drugs, Medipal Holdings Co. The joint venture was formed to advance the development of orphan drugs in Japan, and the company began marketing its lead compound, Buphenyl ® (generic name: sodium phenylbutyrate) for the treatment of urea cycle disorders in January of 2013.
For more information, please visit http://www.orphanpacific.com/e/index.html

【CMIC HOLDINGS Co., Ltd.】

The CMIC group started its business as the first Contract Research Organization (CRO) in Japan, providing services that contribute to increasing efficiency and speed of clinical research. Building on this experience and the Pharmaceutical Value Creator business model, the group provides a wide range of services: pharmaceutical research and development, manufacturing, sales and marketing.
For more information about the CMIC Group, please visit http://www.cmic-holdings.co.jp/e/

【Orphan Europe SARL】

Orphan Europe was founded in 1990 to provide treatments for people with a rare disease. The company has brought, to the market, seven medicinal products for the treatment of rare diseases. Orphan Europe was acquired by Recordati in 2007. Recordati is an international pharmaceutical company listed on the Italian stock exchange and has over 3,300 staff dedicated to the research, development, manufacturing and marketing of pharmaceuticals.
For more information about Orphan Europe, please visit http://www.orphan-europe.com/

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