

News Release



October 31, 2022

MEDIPAL HOLDINGS CORPORATION
JCR Pharmaceuticals Co., Ltd.

Translation

MEDIPAL HOLDINGS and JCR Pharmaceuticals Agree on the Granting of Exclusive Negotiation Rights for Global Commercialization Targeting Four Ultra-Rare Diseases and Conclude a Licensing Contract for the Commercialization of a Fucosidosis Therapeutic

October 31 -- MEDIPAL HOLDINGS CORPORATION (HQ: Chuo-ku, Tokyo; Representative Director, President and CEO: Shuichi Watanabe; hereinafter, "MEDIPAL") and JCR Pharmaceuticals Co., Ltd. (HQ: Ashiya, Hyogo Prefecture; Chairman and President: Shin Ashida; hereinafter, "JCR") announced today that their Boards of Directors resolved an execution of Memorandum on granting of exclusive negotiation rights for the global commercialization outside Japan of four new drug candidates being developed by JCR for ultra-rare lysosomal storage diseases (LSDs) (hereinafter, "the Memorandum"). Furthermore, in accordance with the execution of Memorandum, the two companies have concluded a licensing agreement for a therapeutic candidate for the treatment of fucosidosis.

Fucosidosis, neuronal ceroid lipofuscinosis type 1 (CLN1), neuronal ceroid lipofuscinosis type 2 (CLN2), and galactosialidosis are scheduled to be disease focus for the development of therapeutics under the Memorandum. Each of these diseases comprise only a very small patient population. Through the Memorandum, MEDIPAL will acquire an exclusive right to negotiate to obtain an exclusive license with sublicensing rights for the global commercialization of the therapeutic candidates outside Japan, which includes research, development, manufacturing and marketing. In return, JCR will receive a designated monetary payment in consideration for granting exclusive negotiation rights.

Furthermore, through a separate agreement, MEDIPAL will acquire an exclusive license with sublicensing rights for the global commercialization of a drug substance that targets fucosidosis (blood-brain barrier (BBB) penetrating human α -L-fucosidase) outside Japan, including research, development, manufacturing, and marketing. JCR is scheduled to receive an upfront payment for the agreement and royalties on post-development sales.

JCR is focused on the research and development of several products for the treatment of LSDs, which are rare diseases, that use J-Brain Cargo[®], JCR's proprietary BBB penetration technology. Among these, indications known as ultra-rare diseases have very small patient populations, which generally is associated with certain clinical development challenges. However, to serve patients who are eagerly awaiting the development of new drugs, MEDIPAL and JCR, which have maintained a good relationship and have consistently produced results through collaboration over many years, have decided to undertake new drug development in these ultra-rare disease fields as a fulfillment of their social responsibilities as companies engaged in healthcare. The two companies will work to deliver novel therapeutic drugs to patients and their families all over the world as expeditiously as possible.

Explanation of Terminology

About Fucosidosis

Fucosidosis is a lysosomal storage disorder that is inherited in an autosomal recessive manner. Mutations result in malfunction of a glycoprotein-metabolizing enzyme (α -fucosidase) which causes glycans and glycoproteins to accumulate throughout the body. Patients with fucosidosis display a variety of symptoms, including psychomotor symptoms, muscle hypotonia, visceromegaly, and skeletal abnormalities. Fucosidosis is classified as type 1 and type 2, based on the age of onset. The prevalence of fucosidosis is extremely rare, affecting less than 1 in 2 million people. There is no standard therapy available for this disease.

About Neuronal ceroid lipofuscinosis (NCL)

NCL is an LSD that is also referred to as Batten disease. It is a neurodegenerative genetic disease and inherited in an autosomal recessive pattern. NCL is mainly characterized by progressive motor dysfunction, convulsions, and vision impairment. In general, the prognosis is extremely poor when the disease first appears in childhood. NCL can be classified into CLN1 to CLN14 based on the causative gene mutation.

CLN1 is caused by low activity of an enzyme that removes palmitic acid (palmitoyl-protein thioesterase 1: PPT1). It is classified into four types based on the age of onset: infantile, late infantile, juvenile, and adult. There is no standard therapy available for this disease.

CLN2 is caused by low activity in a proteolytic enzyme (tripeptidyl peptidase 1: TPP1). It is classified into infantile and juvenile forms. TPP1 enzyme replacement therapy by intracerebroventricular administration has been approved in Europe and the U.S. in 2017 and Japan in 2019 as a standard of care for this disease.

About Galactosialidosis

Galactosialidosis is an LSD and is caused by a genetic mutation of the protective protein cathepsin A (PPCA). PPCA abnormality results in accumulation of glycolipids and GM1 gangliosides, a glycoprotein. Patients with Galactosialidosis display a variety of symptoms including bone, coarse facies, and CNS signs and symptoms. There is no standard therapy available for this disease.

About MEDIPAL HOLDINGS CORPORATION (As of March 31, 2022)

Representative: Shuichi Watanabe, Representative Director, President and CEO

Headquarters: 2-7-15, Yaesu, Chuo-ku, Tokyo, Japan

Established: May 6, 1923

Employees (consolidated): 12,801

Paid-in capital: 22,398 million yen

Scope of business: As a holding company, MEDIPAL HOLDINGS CORPORATION controls, administers, and supports the operating activities of companies in which it holds shares in the Prescription Pharmaceutical Wholesale Business; the Cosmetics, Daily Necessities and OTC Pharmaceutical Wholesale Business; and the Animal Health Products and Food Processing Raw Materials Wholesale Business, and conducts business development for the MEDIPAL Group.

About JCR Pharmaceuticals Co., Ltd. (As of March 31, 2022)

Representative: Shin Ashida, Chairman and President

Headquarters: 3-19 Kasuga-cho, Ashiya, Hyogo, Japan

Established: September 13, 1975

Employees (consolidated): 810

Paid-in capital: 9,061 million yen

Scope of business: Manufacture, selling, buying, import/export of pharmaceutical products, Regenerative Medicines and drug substances. Selling, buying, import/export of medical devices and laboratory instruments.

Cautionary Statement Regarding Forward-Looking Statements

This document contains forward-looking statements that are subject to known and unknown risks and uncertainties, many of which are outside our control. Forward-looking statements often contain words such as “believe,” “estimate,” “anticipate,” “intend,” “plan,” “will,” “would,” “target” and similar references to future periods. All forward-looking statements regarding our plans, outlook, strategy and future business, financial performance and financial condition are based on judgments derived from the information available to us at this time. Factors or events that could cause our actual results to be materially different from those expressed in our forward-looking statements include, but are not limited to, a deterioration of economic conditions, a change in the legal or governmental system, a delay in launching a new product, impact on competitors’ pricing and product strategies, a decline in marketing capabilities relating to our products, manufacturing difficulties or delays, an infringement of our intellectual property rights, an adverse court decision in a significant lawsuit and regulatory actions.

This document involves information on pharmaceutical products (including those under development). However, it is not intended for advertising or providing medical advice. Furthermore, it is intended to provide information on our company and businesses and not to solicit investment in securities we issue.

Except as required by law, we assume no obligation to update these forward-looking statements publicly or to update the factors that could cause actual results to differ materially, even if new information becomes available in the future.

Investors & Media:

MEDIPAL HOLDINGS CORPORATION
Public Relations Department
ir@mediceo-gp.com

JCR Pharmaceuticals Co., Ltd.
Corporate Communications / IR Office, Administration Division
ir-info@jcrpharm.co.jp

###