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To Whom It May Concern

Company: KYORIN Holdings, Inc.  
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## KYORIN Signs Joint Development and License Agreement for Genetically Modified Adipocyte, Treatment Agent for Fabry Disease

KYORIN Holdings, Inc. announced today that KYORIN Pharmaceutical Co., Ltd. (Head office: Chiyoda-ku, Tokyo; President & CEO: Shigeru Ogihara, hereinafter KYORIN), a subsidiary of KYORIN Holdings, Inc., signed a joint development and license agreement with CellGenTech Inc. (Head office: Chiba-shi, Chiba; President & CEO: Masayuki Aso, hereinafter CellGenTech) for genetically modified human adipocytes (hereinafter GMAC) of Fabry disease that has been developed by CellGenTech (hereinafter this Agreement).

Under this Agreement, KYORIN has acquired an exclusive license to develop and commercialize a new product in the treatment of rare and refractory disease known as Fabry disease using GMAC and CellGenTech's technology. KYORIN will pay CellGenTech for an upfront payment and milestone payments depending on the progress in the development and commercialization of the product as well as tiered royalties on the sales of the product. KYORIN has also acquired the option to develop and commercialize GMAC for another disease.

CellGenTech, a biotech venture founded in 2003 with an origin in Chiba University, is making efforts to develop treatment agents using its unique technology, GMAC, to improve quality of life of patients in intractable or rare diseases and their family (hereinafter QOL).

GMAC is the genetically modified human adipocytes with an ability to produce insufficient enzyme or protein in patients by inducing gene encoding functional treatment enzyme or protein pre-adipocytes isolated from their own subcutaneous fat. GMAC is expected to contribute to the treatment of Fabry disease and the improvement of QOL by supplementing insufficient enzyme over years by one dose.

KYORIN has set forth the priority strategy "Enhance pipeline to support medium-term growth" in the medium-term management plan "HOPE100-stage 3-" and has been actively involved in in-licensing product for intractable or rare diseases.

Under this Agreement, KYORIN actively promotes development to provide the treatment agents for patients of Fabry diseases, which is high unmet medical needs, as soon as possible and aims to become globally recognized company by creating innovative new drugs.

The impact of the execution of this Agreement on the consolidated financial forecast for the fiscal year ending on March, 2022 will be minor.

**【Reference data】**

**About KYORIN Pharmaceutical Co., Ltd.**

Foundation: December 1923

President & CEO: Shigeru Ogihara

Annual Sales: JPY92,010 million (fiscal year ending on March 31, 2022)

Employees: 1,441 (as of March 31, 2022)

Overview: Trusted among patients and professionals in the medical industry, KYORIN strives to be the pharmaceutical company recognized as a one with social significance by improving its presence in the specific therapeutic areas and through its global discovery of novel drugs. KYORIN uses franchise customer strategy where its marketing efforts are focused on respiratory medicine, otolaryngology and urology. In drug discovery, it is deploying 'selection and concentration' and promoting activities aimed at first-in-class drug discovery, such as actively searching for and introducing external drug discovery themes as well as multi-tiered program development.

**About CellGenTech, Inc.**

Foundation: October 2003

President & CEO: Masayuki Aso

Employees: 24 (as of March 31, 2022)

Overview: CellGenTech, Inc., as a pioneer of cell-based medicine, is making efforts to develop "transduced adipocyte-based medicine" which is prepared by introducing a therapeutic gene into human adipocytes, especially for patients and their families suffering from intractable diseases caused by inheritably deficient or impaired protein factor. Considering high potential of "transduced adipocyte-based medicine", our technology may apply to other medical research field. We are open to discuss any opportunity of collaboration whatever it contributes to the benefit of patients suffering from intractable diseases over the world.

**About Fabry Disease**

Fabry disease is a progressive X-linked hereditary disorder, which occurred due to gene mutation in the gene encoding  $\alpha$ -galactosidase (GALA) a type of lysosome hydrolase. Deposition of glycosphingolipids, mainly globotriaosylceramide, which is substrate of  $\alpha$ -GALA cause a varied symptoms and disorder, like limb pain, dyshidrosis, angiokeratoma, progressive renal insufficiency and cardiomegaly etc. The reported prevalence ranges 1 per 40,000 but higher in recent newborn screening study\*. In Japan, as from January 1, 2015, Fabry disease is classified as designated intractable diseases and specified chronic pediatric disease

\* Practical guideline for the management of Fabry disease 2020 Japanese Society for Inherited Metabolic Diseases