



Chugai and BioMarin Enter Exclusive Sublicense Agreement on the Patent of C-type Natriuretic Peptide Intended for Treatment of Achondroplasia

TOKYO, March 3, 2016 --Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced today that it has concluded an exclusive sublicense agreement with BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) on the patent of Chugai's C-type natriuretic peptide (CNP) intended for treatment of achondroplasia in the regions of U.S. and Japan.

CNP is an endogenous peptide discovered in Japan. It induces bone growth by inhibiting the fibroblast growth factor receptor 3, which inhibits the growth of the cartilage that controls bone growth. This peptide is expected to be beneficial for treatment of patients with achondroplasia, the intractable disease, in whom bone growth is systemically impaired.

In the U.S., EU and Australia, BioMarin is currently conducting a phase II study of a CNP derivative, vosoritide (formerly called BMN 111), in children with achondroplasia. The interim analysis of this study demonstrated favorable safety and efficacy of vosoritide subcutaneously administered once daily. The phase II study added a fourth cohort and increased the dose. The company is also planning to initiate a pivotal phase III study, as well as another study in children under the age of five.

Meanwhile, Chugai acquired an exclusive license to use the patent on CNP and has been engaging in research for commercializing the drug for treatment of achondroplasia. However, Chugai has decided to discontinue the program on its own since it has fallen behind the initial schedule.

This time, in order to make vosoritide available for patients as a new treatment for achondroplasia as quickly as possible, Chugai held discussion with BioMarin and decided to sublicense to BioMarin the exclusive rights to use the patent for CNP, which belongs to Chugai.

“We hope that this agreement will enable prompt launch of vosoritide and meet the needs of people with achondroplasia as a new treatment where none exists.” said Chugai’s head of Business Development Department, Mr. Tetsuya Yamaguchi.

This agreement gives BioMarin the rights to commercialize vosoritide as a drug for treatment of achondroplasia. Chugai will receive an upfront payment, as well as milestone payments in line with the progress of the development, and after the launch, a royalty in line with the amount of sales.