News Release

RIBOMIC Announces the First Case Registration of observational study for RBM-007 early Phase II development project in Achondroplasia patients

TOKYO, November 28, 2022 - RIBOMIC, Inc., a clinical stage pharmaceutical company specializing in aptamer therapeutics (TYO:4591), today announced that the first case has been registered at Tokyo Medical and Dental University Hospital for an observational study to obtain basic clinical data including height growth and to select appropriate subjects for continuously scheduled RBM-007 early phase 2 study in Achondroplasia patients.

Furthermore, RIBOMIC has received approval from the Pharmaceuticals and Medical Devices Agency (PMDA) to conduct an early Phase 2 study of exploratory investigation for the safety and efficacy of RBM-007 in achondroplasia pediatric patients, as well as following extension study. Therefore, RIBOMIC is planning to proceed with those studies following the observational study.
Please see the following for a summary of Phase 2 trials in Japan.
Early phase 2 observational study : https://jrct.niph.go.jp/latest-detail/jRCT2031220113
Early phase 2 study : https://jrct.niph.go.jp/latest-detail/jRCT2031220291
Early phase 2 extension study : https://jrct.niph.go.jp/latest-detail/jRCT2031220338

About RBM-007
RBM-007 is a novel nucleic acid medicine (oligonucleotide-based aptamer) developed in-house at RIBOMIC's research facilities in Tokyo. RBM-007 has been shown to have potent effects in limiting excessive interactions between fibroblast growth factors, which are known to cause achondroplasia.

About Achondroplasia
Achondroplasia is a rare disease characterized by short stature (adult height of approximately 130 cm for males and approximately 125 cm for females) with short limbs. Achondroplasia has no known cure, and is designated as an intractable disease by the Ministry of Health, Labour and Welfare in Japan. This disease results mainly from a genetic defect in FGFR3 (fibroblast growth factor type 3 receptor). This genetic change causes the receptor to be overly active to growth factors such as FGF2, which leads to reduced growth of chondrocytes, resulting in a short stature. Achondroplasia occurs in a frequency of 1 in approximately 25,000 normal live births and is estimated to affect approximately 250,000 people worldwide.
By inhibiting the binding of FGF2 to FGFR3, RBM-007 has demonstrated therapeutic effects in studies using animal models of achondroplasia and patient-derived iPS (induced pluripotent stem) cells.

ABOUT RIBOMIC
RIBOMIC is a clinical stage bio-pharmaceutical company specializing in the discovery and development of aptamer therapeutics, which is one type of nucleic acid medicine, a field with much potential for the development of next-generation drugs. The RiboART system, the company's core drug discovery platform, can be used for the discovery of many types of aptamer drugs. RIBOMIC is dedicated to the discovery and development of drugs that target the broad field of unmet medical needs, which encompasses eye disorders, rare disease of short stature in children and many other diseases.
See RIBOMIC website for more information.
https://www.ribomic.com/eng/

Forward-Looking Statements

This announcement contains forward-looking statements relating to current plans, estimates, strategies, belief and the future performance of Company. These statements are based on Company's current expectations in light of the information and assumptions currently available so that Company does not promise the realization and these expectations may differ materially from those discussed in the forward-looking statements. These factors include, but not limited to, i) changes in general economic conditions and in laws and regulations, relating to pharmaceutical markets, ii) currency exchange rate fluctuations, iii) claims and concerns on the product safety and efficacy, iv) completion and discontinuation of clinical trials, v) infringement of Company's intellectual property rights by third parties.
Information on pharmaceutical products (including products currently in development), which is included in this press release is not intended to constitute an advertisement or medical advice.

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