News Release

RIBOMIC Announces RBM-007 Phase 1 Clinical Trial Results for Achondroplasia

TOKYO, November 10, 2021 - RIBOMIC, Inc., a clinical stage pharmaceutical company specializing in aptamer therapeutics (TYO:4591), today announced the results from its Phase 1, healthy volunteer clinical study using RBM-007 for the planned treatment of Achondroplasia, which was completed in May this year.

This Phase 1 study assessed the safety, tolerability and pharmacokinetics of RBM-007 administered subcutaneously in 24 healthy volunteers. The study used an open label, dose-escalating, sequential-cohort design and was conducted in Japan. Subjects each were given a dose of RBM-007 (0.1, 0.3, 0.6 mg/kg) on one or two occasions.

Overall, subcutaneous injection of RBM-007 at 0.1-0.6 mg/kg up to two times with an interval of one or two weeks was well tolerated without safety concerns.

One SAE of anaphylactic reaction occurred in one subject administered the highest dose of 1.0 mg/kg. The subject was treated with medications and the event resolved the same day that it occurred. Because of this event, the maximal dose was decreased to 0.6 mg/kg and the study was continued.

The blood concentration of RBM-007 measured in the study provided basic pharmacokinetic data necessary for the next early Phase 2 trial, which the company is currently discussing with the Pharmaceuticals and Medical Devices Agency (PMDA).

About RBM-007
RBM-007 is a novel oligonucleotide-based aptamer with potent anti-FGF2 (fibroblast growth factor 2) activity. RBM-007 has been shown to have potent effects in limiting excessive interactions between FGF2 and FGF receptor 3 activating variant, which are known to cause Achondroplasia.

See JapicCTI (Japic Clinical Trials Information) for more information of the trial.
https://www.clinicaltrials.jp/cti-user/trial/ShowDirect.jsp?directLink=0p52SZqEULFxxBQ0QuD.Vg--

About Achondroplasia
Achondroplasia is a rare disease with a form of 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 variant 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 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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