News Release

July 15, 2020

RIBOMIC Announces Dosing of First Subject in RBM-007 Phase 1 Clinical Trial for Achondroplasia

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- The first subject was administered with RBM-007 in Phase 1 Clinical Trial for the treatment of Achondroplasia

TOKYO, July 15, 2020 - RIBOMIC, Inc., a clinical stage pharmaceutical company specializing in aptamer therapeutics and traded on the Mothers Market of the Tokyo Stock Exchange (TYO: 4591), today announced that the first subject in cohort 1 was administered with RBM-007 subcutaneously in Phase 1 clinical trial in Japan.

This represents the second indication for the innovative molecule, which is currently under phase 2 trial in the United States for the treatment of age-related macular degeneration.

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 factor 2 (FGF2) and FGF receptor 3 activating variant, which are known to cause Achondroplasia.

About Phase 1 study

Study Design	An open label, unblinded, noncomparative Phase 1 study
Patient Population	Healthy, male, adult Japanese volunteers
Administration	Single or two-time subcutaneous administrations with RBM-007, dose-escalating with sequential cohorts of 0.1 mg/kg, 0.3 mg/kg and 1.0 mg/kg. For the single-injection cohorts, each cohort is divided into two steps (one subject will receive RBM-007 as the first step, and then the other three subjects will receive it as the next step).
Primary Endpoints	Safety, tolerability and pharmacokinetics
Cohort	Cohort 1: RBM-007, 3 dose levels (0.1, 0.3, 1.0 mg/kg), once Cohort 2: RBM-007, 3 dose levels (0.1, 0.3, 1.0 mg/kg), twice with an interval of 1 week to 2 weeks
Number of Subject	24 subjects (Cohort 1: 4 subjects in each dose, total 12 subjects,

	Cohort 2: 4 subjects in each dose, total 12 subjects)
Duration	June 2020 – March 2021
Location	Single site in Japan

See JapicCTI (Japic Clinical Trials Information) for more information.
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 bio-venture company centered on drug discovery. The company develops aptamer therapeutics, within the fast-growing field of nucleic acid medicine. 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, pain and many other problems.

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.
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