News Release

RIBOMIC

RIBOMIC Announces Completion of IND submission of RBM-007 for Treatment of Achondroplasia

TOKYO, April 10, 2020 - RIBOMIC, Inc., a clinical stage pharmaceutical company specializing in aptamer therapeutics and traded on the Mothers Market of the Tokyo Stock Exchange (Code Number: 4591), today announced that it submitted an Investigational New Drug Application (IND) to the Pharmaceuticals and Medical Devices Agency (PMDA) in Japan to test its novel drug RBM-007, an anti- Fibroblast Growth Factor 2 (FGF2) aptamer to treat Achondroplasia. 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.

RIBOMIC expects to begin phase 1 clinical trial in Q1(Japan fiscal year) 2020 at a single site in Japan with a total of 24 healthy adult males to assess the safety, tolerability and pharmacokinetics of RBM-007.

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 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 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 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.
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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