News Release

RIBOMIC Announces Completion of its Phase I Trial of RBM-007 for the Treatment of Achondroplasia

TOKYO, May 12, 2021 - RIBOMIC, Inc., a clinical stage pharmaceutical company specializing in aptamer therapeutics (TYO:4591), today announced the completion of its Phase I study of RBM-007 for the treatment of achondroplasia. The study results will be reported after a detailed analysis of the trial data.

Study Design

Phase	Phase I
Allocation	Non-Randomized; Open label
Subjects	24 healthy male volunteers
Method of Administration	Single or two subcutaneous injections of RBM-007 Sequential titration of three escalating dose groups
Purpose	Evaluation of safety, tolerability and pharmacokinetics
Location	Single site in Japan

The outline of this study can also be found at JapicCTI.
https://www.clinicaltrials.jp/cti-user/trial/ShowDirect.jsp?directLink=0p52SZqEULFxxBQ0QuD.Vg--

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 bio-venture company centered on drug discovery. The company is engaged in the field 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, pain and many other conditions.
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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