News Release

RIBOMIC Announces Publication of Research Paper on Aptamer Drug Discovery for Achondroplasia

TOKYO, May 6, 2021 - RIBOMIC, Inc., a clinical stage pharmaceutical company specializing in aptamer therapeutics (TYO:4591), today announced research results that were published online in Science Translational Medicine. The research was conducted in collaboration with the Osaka University Graduate School of Medicine (Keiichi Ohzono, MD, PhD) and School of Medicine, Masaryk University, Czech Republic (Pavel Krejčí, PhD), and under the support of AMED (Japan Agency for Medical Research and Development).

Journal Name	Science Translational Medicine
Article Title	RNA aptamer restores defective bone growth in FGFR3-related skeletal dysplasia
Article URL	https://stm.sciencemag.org/content/13/592/eaba4226/tab-pdf
Article Summary	RBM-007 has proven efficacy in studies of ACH patient-derived iPSC (artificial pluripotent stem cells) and animal models that recapitulate disease.

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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