News Release

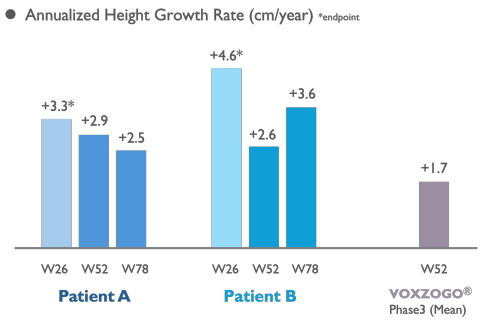
2025/8/20

RIBOMIC Announces the Sustained Efficacy in a Long-Term Administration Trial of Umedaptanib Pegol for Achondroplasia

TOKYO, August 20, 2025 - RIBOMIC, Inc. (TYO:4591), a clinical-stage pharmaceutical company specializing in aptamer therapeutics, has been conducting a Phase IIa clinical trial of umedaptanib pegol (anti-FGF2 aptamer) in pediatric patients (5-14 years old) with achondroplasia (ACH), and today announces the sustained efficacy in a long-term administration trial.

The Company previously announced that in a Phase IIa clinical trial conducted in November 2024 for patients with achondroplasia (ACH) using umedaptanib pegol (an anti-FGF2 aptamer), the height growth rate of two pediatric patients in the low-dose (0.3 mg/kg) subcutaneous injection group (Cohort 1, once weekly) showed a significant increase. Following this, the continued administration under the same conditions for one year (long-term administration study) demonstrated that the growth-promoting effects of umedaptanib pegol were sustained.

The figure shows the positive change from baseline in annualized height growth rate of the two pediatric patients who responded to treatment, compared to pre-treatment levels (observation study), at six months (W26), one year (W52), and one and a half years (W78) after treatment initiation. The sustained efficacy of the drug was confirmed. The annual height growth rate of the two patients exceeded the average height growth rate (+1.7 cm/year) of Voxzogo® (vosoritide, manufactured by BioMarin), which is currently approved as a treatment for ACH.



If this pace continues, with an annual height increase of 3 cm over six years, the average height of adult males with ACH is expected to increase from 130 cm to 148 cm, and that of adult females from 124 cm to 142 cm, which is anticipated to significantly contribute to improved quality of life (QOL).

There have been no safety concerns in the ongoing Phase II clinical trials, including this long-term administration trial.

ABOUT umedaptanib pegol

umedaptanib pegol is a novel oligonucleotide-based aptamer formerly designated RBM-007, with potent anti-FGF2 (fibroblast growth factor 2) activity and is expected to be a fundamental treatment that directly targets the pathogenic mechanism of achondroplasia.
The drug has demonstrated clinical POC in exudative age-related macular degeneration.

ABOUT Achondroplasia

Achondroplasia is disease in which a genetic mutation of the fibroblast growth factor receptor type 3 (FGFR3) causes FGFR3 to be activated, resulting in an excessive influx of FGF signals that inhibit the normal growth of cartilage and other tissues, causing short stature with limb shortening and other symptoms. It is a rare disease with an incidence of 1 in 25,000 newborns and is considered intractable. The development of effective new drugs is required.

ABOUT RIBOMIC

RIBOMIC is a clinical-stage biopharmaceutical company specializing in the discovery and development of aptamer therapeutics, a type of nucleic acid medicine with great potential for the development of next-generation drugs. The RiboART system, the company's core drug discovery platform, can be used to discover many types of aptamer drugs. RIBOMIC is dedicated to the discovery and development of drugs targeting the broad field of unmet medical needs, which includes eye disease, rare childhood disease of short stature, and many other diseases.
Please visit the RIBOMIC website for more information.
https://www.ribomic.com/eng/

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