News Release

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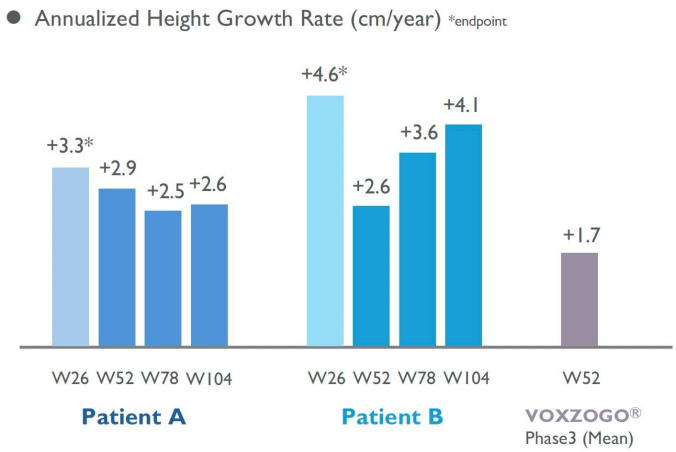
RIBOMIC Announces Continued Growth in Two-Year Follow-Up of Patients Responding to Achondroplasia Treatment Drug, Umedaptanib Pegol

TOKYO, November 20, 2025 - RIBOMIC, Inc. (TYO:4591), a clinical-stage pharmaceutical company specializing in aptamer therapeutics, has completed a Phase 2 clinical trial of umedaptanib pegol (anti-FGF2 aptamer) in pediatric patients (ages 5-14) with achondroplasia. The company announced today that in Cohort 1 (low-dose 0.3 mg/kg weekly subcutaneous injection group), two pediatric patients showed a marked increase in height growth velocity and continuing treatment under the same regimen confirmed that the growth-promoting effect of umedaptanib pegol remained consistent for over two years without attenuation.

The figure shows the change in height velocity over time for the two pediatric patients who responded to treatment, comparing it to the pre-treatment (observational study) period at 6 months (W26), 1 year (W52), 1.5 years (W78), and 2 years (W104) post-administration. This clearly demonstrates the sustained efficacy of the drug. Notably, in one patient (Patient B), the temporary decline in growth-promoting effect recovered over time, resulting in a high growth rate.



The average annual growth rate for the two patients exceeds the average height growth rate (+1.7 cm/year) of the approved drug VOXZOGO® (Vosoritide, manufactured by BioMarin). The two pediatric patients are currently 9 and 10 years old. If they continue growing at this pace for the next five years, they are projected to reach a height of approximately 150 cm.

There are no impact on the results for the fiscal year ending March 2026.

ABOUT UMEDAPTANIB PEGOL

Umedaptanib pegol is a novel oligonucleotide-based aptamer formerly designated RBM-007, with potent anti-FGF2 (fibroblast growth factor 2) activity. This drug has received orphan drug designation from the Ministry of Health, Labour and Welfare in Japan.

ABOUT ACHONDROPLASIA

Achondroplasia is a disease caused by a mutation in the fibroblast growth factor receptor 3 (FGFR3) gene, which makes FGFR3 more easily activated. This leads to excessive influx of FGF signals, inhibiting the normal development of cartilage and other tissues, resulting in short stature accompanied by limb shortening. It is a rare disease with an incidence of approximately 1 in 25,000 newborns and is designated as an intractable disease. The development of effective new drugs is urgently needed.

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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