News Release

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RIBOMIC Announces Summary Report on Primary Endpoint for Phase II Clinical Trial of Umedaptanib Pegol in the Treatment of Achondroplasia

TOKYO, March 10, 2026 - RIBOMIC, Inc. (TYO:4591), a clinical-stage pharmaceutical company specializing in aptamer therapeutics, has announced today the summary report on Phase 2 clinical trial of umedaptanib pegol (anti-FGF2 aptamer) in paediatric patients with achondroplasia.

This trial is a Phase II clinical trial in Japan targeting paediatric ACH patients (aged 5 to 14 years) and aims to evaluate the efficacy and safety of this drug. All subjects underwent a 26-week observation period prior to dosing initiation. Following this, dosing commenced. The design included an 8-week lead-in period to confirm safety and tolerability at the start of dosing, followed by 26 weeks of dosing at the specified dosing interval. The trial consisted of two cohorts, with 6 subjects enrolled in each cohort, totaling 12 subjects.

In Cohort 1 (low-dose group), subcutaneous administration of 0.3 mg/kg was performed every 2 weeks for 8 weeks (4 doses total) to confirm safety and tolerability. Subsequently, the dosing interval was changed to every 1 week for 26 weeks. In Cohort 2 (high-dose group), after confirming safety and tolerability with subcutaneous administration of 0.6 mg/kg every 4 weeks for 8 weeks (2 doses total), the dosing interval was changed to every 2 weeks for 26 weeks. Both cohorts were designed to have the same total dose administered over the treatment period. The primary efficacy endpoint was the change (ΔAHV) in annualized height velocity (AHV) from the pre-treatment period (observation period) after 26 weeks of dosing following the induction period.

The clinical trial results are summarized below.

- Regarding the primary endpoint, Annualized Height Velocity (AHV), excluding one subject who discontinued treatment early, the analysis included 11 subjects: 5 from Cohort 1 and 6 from Cohort 2 who completed treatment. The mean ΔAHV after treatment compared to the pre-treatment (observation period) was +1.4 cm/year, showing a statistically significant difference in the exploratory analysis (p=0.04).

- In Cohort 1, 3 out of 5 subjects showed increased post-treatment AHV compared to pre-treatment AHV. Similarly, in Cohort 2, 5 out of 6 subjects demonstrated increased AHV. Among these, 4 subjects exhibited marked increases in ΔAHV: +2.0, +3.3, +4.6, and +5.0 cm/year. These increases in AHV have persisted in a long-term administration study conducted under identical conditions.

- Improvements in AHV were also observed in subjects with Voxzogo® (vosoritide) administration history (2 out of 3 subjects).

- Exploratory evaluation of secondary endpoints—including height Z-score, serum bone metabolism markers, physical measurements (head circumference, chest circumference, upper arm length, forearm length, thigh length, lower leg length, and arm span), and body shape balance—revealed no consistent trends suggesting efficacy within the scope of this trial. Furthermore, no events that would preclude progression to the next trial were observed in bone age, the formation status of the femoral growth plate, findings at the elbow and hip joints based on clinical examination, or the Tanner stage of development.

- Adverse events judged to be related to the drug included injection site pain (1 case), injection site swelling (1 case), headache (1 case), joint pain (1 case), and hypersensitivity (1 case). All were short-term and mild in nature. No other serious adverse events leading to trial discontinuation were observed.

Based on these results, the Phase II clinical trial of umedaptanib pegol confirmed efficacy and provided important safety findings for continued development. These findings support the proof-of-concept (POC) of umedaptanib pegol for ACH treatment. Detailed analysis results will be submitted for publication in a medical journal.

Based on these trial results, the company plans to submit an application for a pivotal Phase III clinical trial.

Please note that these trial results represent findings regarding the efficacy and safety of this drug at the present time and do not guarantee future manufacturing and marketing approval.

There is no change to the full-year earnings forecast for the fiscal year ending March 2026 due to this matter.

[About Umedaptanib Pegol]

Umedaptanib pegol is the international nonproprietary name (INN) for RBM-007. As an aptamer (nucleic acid medicine) that powerfully inhibits the function of fibroblast growth factor 2 (FGF2), it is expected to become a fundamental treatment directly targeting the pathogenesis of achondroplasia. This drug has received orphan drug designation from the Ministry of Health, Labour and Welfare (MHLW) of Japan.

[About Achondroplasia]

Achondroplasia is a disease caused by a mutation in the fibroblast growth factor receptor 3 (FGFR3) gene. This mutation makes FGFR3 more easily activated, leading to excessive influx of FGF signals. This inhibits 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.