News Release

2025/10/7

RIBOMIC Announces Positive Phase 2 Cohort 2 Results for Umedaptanib Pegol in Achondroplasia, Demonstrating Clinical Proof of Concept

TOKYO, October 7, 2025 - RIBOMIC, Inc. (TYO:4591), a clinical-stage pharmaceutical company specializing in aptamer therapeutics, has conducted a Phase 2 clinical trial of umedaptanib pegol (anti-FGF2 aptamer) in pediatric patients (ages 5-14) with achondroplasia (ACH). The company announced today that the administration of the high-dose (0.6 mg/kg) subcutaneous injection (once biweekly) group (Cohort 2 Note 1) has completed the trial and demonstrated a positive impact of the test drug on the patient growth rate.

In this Phase 2 clinical trial, both Cohort 1 and Cohort 2 received the same total dose of the investigational drug. Cohort 1 Note 2, which was completed earlier, consisted of a group receiving weekly subcutaneous injections of a low dose (0.3 mg/kg). In contrast, Cohort 2 consists of a high dose (0.6 mg/kg), administered every other week. The trial's objective is to confirm the efficacy, safety and durability of umedaptanib pegol.

In Cohort 1, three of the five subjects who completed the dosing period showed an increased height growth rate compared to pre-treatment levels (see Observation Study Note 3), with two showing growth rates of +4.6 cm/year and +3.3 cm/year (Disclosure dated November 5, 2024). In Cohort 2, all 6 subjects completed the trial, and 5 subjects showed increased height growth rate, with 2 showing significant increases of +5.0 cm/year and +2.0 cm/year. The results for these 4 subjects with markedly increased height growth exceeded the average height growth rate of +1.7 cm/year for Voxzogo® (vosoritide, manufactured by BioMarin, administered subcutaneously daily), which is currently approved as an ACH treatment. Furthermore, the mean height growth rates for Cohorts 1 and 2 were +1.5 cm/year and +1.4 cm/year, respectively, which are comparable to those of Voxzogo®. No safety concerns arose throughout the Phase 2 clinical trial.

These results demonstrate the efficacy, safety and durability of umedaptanib pegol in Phase 2 clinical trials, successfully establishing proof-of-concept (POC) as an ACH treatment. The study demonstrated that increasing the total dose allows for extending the dosing interval. Umedaptanib pegol is expected to achieve results comparable to those of Voxzogo® with biweekly or weekly dosing. This should significantly benefit pediatric ACH patients, who are expected to require long-term treatment.

Based on the successful completion of the Phase 2 trial, we are planning a Phase 3 clinical

trial to further verify the efficacy of umedaptanib pegol as an ACH treatment. Previous nonclinical studies using ACH animal models demonstrated that bone elongation rates increase proportionally with the dose of the drug. Therefore, the Phase 3 trial will increase the weekly subcutaneous dose of umedaptanib pegol to approximately 1 mg/kg and lower the participating pediatric patients' ages to around two years. This approach is expected to yield significantly improved treatment outcomes compared to those of currently approved drugs.

The Phase 3 trial is scheduled to begin in the first quarter of fiscal year 2026 and conclude within fiscal year 2027. Since this drug has received orphan drug designation from the Ministry of Health, Labor and Welfare in Japan, our goal is to utilize the associated preferential measures to obtain approval by the end of fiscal year 2028.

Note1 Test group receiving high-dose (0.6 mg/kg) subcutaneous injections once every 4 weeks for 8 weeks (total of 2 doses). After confirming safety and tolerability, the dosing interval will be changed to once every 2 weeks for 26 weeks (total dosing period: 34 weeks).

Note2 Test group receiving low-dose (0.3 mg/kg) subcutaneous injections once every two weeks for 8 weeks (total of 4 doses). After confirming safety and tolerability, the dosing interval will be changed to once weekly for 26 weeks (total dosing period: 34 weeks).

Note3 A study to obtain baseline clinical data, including pre-treatment height growth, for pediatric ACH patients. This data will enable efficacy evaluation in the Phase 2 clinical trial and facilitate appropriate subject selection (total observation period: 26 weeks).

Note4 Kimura T, --- Nakamura Y, Ozono K, Krejci P. An RNA aptamer restores defective bone growth in FGFR3-related skeletal dysplasia. Sci. Transl. Med., May 5;13(592):eaba4226 (2021)

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.

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/

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 News Release discontinuation of clinical trials, v) infringement of Company's intellectual property rights by third parties.
Information on pharmaceutical products (including products currently in development), which is included in this press release is not intended to constitute an advertisement or medical advice.

Contacts for inquiries or additional information:
RIBOMIC Inc.
ir.inquiry@ribomic.com