News Release

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RIBOMIC Announces No Safety Concerns After Three Months of 1 mg/kg Weekly Treatment with Umedaptanib Pegol in Achondroplasia Phase 2 study

TOKYO, September 28, 2026 - RIBOMIC, Inc. (TYO:4591), a clinical-stage pharmaceutical company specializing in aptamer therapeutics, is conducting a long-term extension study to evaluate the long-term safety and efficacy of umedaptanib pegol, an anti-FGF2 aptamer, in paediatric patients aged 5 to 14 years with achondroplasia (ACH) who completed the phase 2 clinical trial. The Company today announced that no safety concerns have been identified through three months after the first participant transitioned to the regimen used in the ongoing phase 3 clinical trial in Japan (the WAKABA study), namely 1 mg/kg administered once weekly by subcutaneous injection.

Of the 12 participants who completed the Japanese Phase 2 clinical trial, 11 enrolled in this study, and safety and efficacy are currently being evaluated in seven participants who remain in the study. Three participants have transitioned to the same regimen as that used in the WAKABA study, namely 1 mg/kg administered once weekly. This announcement provides an update on the first such participant through three months after the transition.

The no safety concerns through three months after the transition in the first participant provides additional information regarding the long-term safety profile of umedaptanib pegol and offers early supportive safety data for the regimen used in the WAKABA study. The Company will continue to progress both the phase 2 long-term extension study and the WAKABA study with the aim of developing a new treatment option for patients with ACH.

There is no change to the full-year earnings forecast for the fiscal year ending March 2027 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.

Please visit the RIBOMIC website for more information.
https://www.ribomic.com/eng/

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