News Release

2024/7/1

RIBOMIC Announces Completion of Patient Enrollment in Early Phase II Study of umedaptanib pegol for Achondroplasia and Initiation of Dosing in Cohort 2 of Early Phase II Clinical Study

TOKYO, July 1, 2024 – RIBOMIC Inc. (TYO:4591), a clinical-stage pharmaceutical company specializing in aptamer therapeutics, today announced that 13 patients have been enrolled in an observational study [1] of umedaptanib pegol (anti-FGF2 aptamer) in pediatric patients (5-14 years old) with Achondroplasia in an early phase II study.
A low-dose subcutaneous study (Cohort1 [2]) to evaluate the efficacy and safety of umedaptanib pegol in six patients who have completed the observational study is ongoing and is expected to be completed in November of this year.
These patients will continue in the extension study and three have already completed Cohort 1 and entered the extension study.
RIBOMIC also announced that the first patient in a high-dose subcutaneous study (Cohort 2 [3]) of umedaptanib pegol has been dosed in patients who have completed the observational study has been administered. No safety concerns have been identified in the ongoing early phase II clinical study and extension study, including this case.

Please see the following for a summary of early Phase II study in Japan.
Early phase II observational study: https://jrct.niph.go.jp/latest-detail/jRCT2031220113
Early phase II clinical study: https://jrct.niph.go.jp/latest-detail/jRCT2031220291
Early phase II extension study: https://jrct.niph.go.jp/latest-detail/jRCT2031220338

References

[1] The objective is to obtain baseline clinical data, including height growth, in pediatric patients with Achondroplasia and to compare them with these data to enable the evaluation of efficacy and a safety in the ongoing early phase II clinical study and at the same time to appropriately select subjects for the early phase II clinical study (total observation period: 26 weeks).

[2] After confirming the safety and tolerability of subcutaneous administration at a dose of 0.3 mg/kg once every 2 weeks for 8 weeks (total of 4 doses), the dosing interval is changed to once weekly for 26 weeks (total dosing period: 34 weeks).

[3] After confirming the safety and tolerability of subcutaneous administration at a dose of 0.6 mg/kg once every 4 weeks for 8 weeks (total of 2 doses), the dosing interval is changed to once every 2 weeks for 26 weeks (total dosing period : 34 weeks).

There are no revisions to the full-year earnings guidance for the fiscal year ending March 31, 2025 that was announced on May 14, 2024.

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 discovery 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.

"RIBOMIC," "RiboART system" and the RIBOMIC logo are registered trademarks or trademarks of RIBOMIC Inc. in various jurisdictions.

Contacts for inquiries or additional information:

RIBOMIC Inc.

ir.inquiry@ribomic.com