

2025/7/31

## **RIBOMIC Plans to Initiate Phase III Clinical Trial of Umedaptanib Pegol for Achondroplasia in 2026 Following the Ongoing Phase II Clinical Trial**

TOKYO, July 31, 2025 - RIBOMIC, Inc. (TYO:4591), a clinical-stage pharmaceutical company specializing in aptamer therapeutics, has been conducting a Phase IIa clinical trial of umedaptanib pegol (anti-FGF2 aptamer) in pediatric patients (5-14 years old) with achondroplasia (ACH), and today announces that at the Board of Directors meeting held on July 23, 2025, the Company resolved to issue new stock options and establish the following policy for future clinical development.

### **1. Development of umedaptanib pegol for the treatment of achondroplasia**

Currently, the Company is conducting a Phase 2a clinical trial. Considering the results of Cohort 1 (low-dose group trial), which have already been disclosed, and the interim results of Cohort 2 (high-dose group trial), which is currently ongoing, the Company has determined that proceeding directly to a Phase 3 clinical trial is the best course of action for both the company and its shareholders. Accordingly, the Company has resolved to begin preparations to initiate the Phase 3 clinical trial in the first half of the 2026 fiscal year. If the Phase 3 clinical trial progresses as planned, the Company anticipates that a marketing authorization application will be possible by the second half of fiscal year 2028, with approval expected by the middle of fiscal year 2029.

Another reason for this decision is that the drug has been designated as an orphan drug (ODD) by the Ministry of Health, Labour and Welfare. This designation entitles the company to apply for government research and development funding, priority review, and preferential treatment such as an extension of the post-marketing review period. The Company has already been approved to receive research grant funds for the current fiscal year (announced on July 24, 2025). These funds are intended for research and development necessary for submitting an application for marketing approval for orphan drugs, including the development costs associated with preparing the supporting documents for the marketing approval application. Therefore, the Company is required to promptly conduct Phase 3 clinical trial and fulfill the mission to "deliver a treatment to the medical field as soon as possible."

### **2. Regarding the issuance of new share subscription rights**

To realize the above plans, the Company has resolved to issue the 18th to 20th new share subscription rights (with an exercise price adjustment clause) through a third-party allotment and to enter into a share subscription rights repurchase agreement (commitment issue). This capital raising will enable the Company to financially advance the Phase 3 clinical trial of umedaptanib pegol in a seamless manner in line with ODD's mission of realizing a "Japan-originated" new drug.

As disclosed in our financial statements and securities reports, as of March 31, 2025, the Company holds cash and deposits, as well as high-quality securities (hereinafter referred to as "cash and cash equivalents"), totaling approximately 3 billion yen, which are relatively liquid assets. The Company believes that this amount is sufficient to secure funds for approximately two years. However, as disclosed in our earnings

forecast for the fiscal year ending March 2026, even if no operating revenue is expected for the current fiscal year, the Company does not anticipate any concerns regarding the continuation of the Company's business operations. Nevertheless, the Company believes that the risk of significant uncertainty regarding the going concern assumption will increase.

Therefore, in addition to the fact that the exercise of the new share subscription rights will require a certain amount of time, the Company has decided to issue them at this timing with sufficient margin, taking into account the calculation period (January to March 2026) for the market capitalization criteria, one of the listing maintenance criteria of the Tokyo Stock Exchange Growth Market.

### 3. Regarding the collaboration and out-licensing activity of umedaptanib pegol

Regarding the development of umedaptanib pegol, the Company has been continuing collaboration and out-licensing activities since the previous fiscal year. If collaboration negotiations progress by the time the Phase 2 clinical trial is completed in October 2025, the Company anticipates that revenue generation could be achieved as early as the fiscal year ending in 2026. In such a case, several partnership structures (such as a simple license-out or joint development through an option agreement) are envisaged, and the Company aims to select the best option for both the Company and its shareholders.

Additionally, if commercialization is achieved, the exercise of any remaining stock options at that time may be suspended, or the Company may opt to repurchase and cancel the 19th and 20th stock options at its discretion.

Regarding the issuance of new stock subscription rights, although there is time until the scheduled start of expenditure for the intended use of funds in April 2026, the Company plans to issue them at a later, more flexible timing. This will help ensure financial flexibility to achieve our medium- to long-term business objectives and provide a stable and robust business foundation for license-out negotiations, among other secondary benefits.

#### 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 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.

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