News Release

RIBOMIC

RIBOMIC Resumes Trial Recruitment Suspended by Coronavirus

TOKYO, May 7, 2020 - RIBOMIC, Inc., a clinical stage pharmaceutical company specializing in aptamer therapeutics and traded on the Mothers Market of the Tokyo Stock Exchange (TYO:4591), today announced enrollment of new subjects has resumed in the phase 2 trial of RBM-007 for the treatment of wet age-related macular degeneration (AMD) being conducted in the United States. The enrollment in the trial had been temporarily paused in March in response to COVID-19. It will resume at sites where trial activities are allowed by the local, state and federal regulations and where investigators have the capacity to ensure patient safety.

About RBM-007 and development background

RBM-007 is a novel oligonucleotide-based aptamer with potent anti-FGF2 (fibroblast growth factor 2) activity. Currently approved therapies for wet AMD, intravitreal injections of anti-VEGF drugs, have shown dramatic visual benefits for wet AMD patients. However, a significant portion of wet AMD patients exhibit incomplete response to therapy, and over the extended management course can lose vision, with the formation of submacular fibrosis as one risk factor. RIBOMIC investigated a novel therapy for wet AMD targeting FGF2, which is implicated in not only angiogenesis but also fibrosis in several diseases, and created RBM-007, a novel oligonucleotide-based aptamer with potent anti-FGF2 activity. RBM-007 is chemically synthesized, and pharmacokinetic studies of RBM-007 in the rabbit vitreous revealed high and relatively long-lasting profiles, which are superior to the other approved anti-VEGF drugs. The dual action of RBM-007 (anti-angiogenic and anti-scarring) holds promise as an additive or alternative therapy to anti-VEGF treatments for wet AMD.

About TOFU study

A Multi-Center, Randomized, Double Masked and Active Controlled Phase II Study Assessing the Efficacy and Safety of Intravitreal Injections of RBM-007 monotherapy and RBM-007 in Combination with Eylea® Compared to Eylea® Monotherapy in Subjects with Wet Age-related Macular Degeneration (TOFU Study) is Phase 2 Study assessing the safety, efficacy and durability of RBM-007.

Study Design	Multicenter, active-controlled, double masked study
Patient Population	Patients with wet AMD who are non or low responders to existing anti-VEGF drugs
Administration	Four monthly intravitreal injections of RBM-007. Eylea® dosed every other month as per label.
Primary Endpoints	Mean change in Best Corrected Visual Acuity from Baseline and safety

Study Arms	RBM-007 (monotherapy) RBM-007 and Eylea® (Anti-VEGF drug) (combination) Eylea® (Anti-VEGF drug) (monotherapy)
Number of Subject	81 (27 per arm)
Duration	5 months (primary endpoint at month 1 after last injection)
Location	10 or more sites across the United States

See ClinicalTrials.gov for more information.
https://clinicaltrials.gov/ct2/show/NCT04200248

About wet Age-related Macular Degeneration
Wet (exudative) age-related macular degeneration, is the leading cause of blindness in the United States and Europe. It is caused by the formation of abnormal and leaky new blood vessels under the retina, termed choroidal neovascularization. The leakage of fluid from the vessels causes retinal thickening and retinal degeneration including fibrotic scar formation, and leads to severe and rapid loss of vision.

ABOUT RIBOMIC
RIBOMIC is a bio-venture company centered on drug discovery. The company develops aptamer therapeutics, within the fast-growing field of nucleic acid medicine. The RiboART system, the company's core drug discovery platform, can be used for the discovery of many types of aptamer drugs. RIBOMIC is dedicated to the discovery and development of drugs that target the broad field of unmet medical needs, which encompasses eye disorders, pain and many other problems.
See 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 discontinuation of clinical trials, v) infringement of Company's intellectual property rights by third parties.
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