
RIBOMIC Inc. Provides Update on RBM-007 Program in Wet Age-Related Macular Degeneration

- Early signs of efficacy in the TEMPURA study provide initial support of clinical benefit in treatment-naïve wAMD

- Further analysis of Phase 2 TOFU data and results from the RAMEN study in previously treated wAMD show no benefit of RBM-007 monotherapy or the combination over Eylea in any of the study outcome measures

- The new data suggests RBM-007 could be more effective in treatment-naïve vs previously treated wAMD

TOKYO, March 23, 2022 - RIBOMIC Inc., a clinical stage pharmaceutical company specializing in aptamer therapeutics (TOKYO:4591), today announced the results from the investigator sponsored trial (IST), TEMPURA, along with updated data from its TOFU and RAMEN studies with RBM-007, an investigational anti-fibroblast growth factor-2 aptamer, in wet age-related macular degeneration (wAMD).

The TEMPURA IST was an open-label, uncontrolled, small study (n=5) designed to evaluate the efficacy and safety of RBM-007 monotherapy in treatment-naïve wAMD subjects and was conducted by Raj K. Maturi, M.D., Midwest Eye Institute, Indianapolis, IN. The patients received an intravitreal injection of 2 mg RBM-007 once monthly for three months. The primary and secondary outcome measures were changes from baseline at month 3 in retinal anatomy and best corrected visual acuity (BCVA), respectively. The retinal anatomy was measured as change in central subfield thickness (CST) using optical coherence tomography (OCT) imaging.

The data demonstrated a positive trend in two clinically relevant endpoints suggesting that RBM-007 has the potential to improve BCVA and retinal anatomy in treatment-naïve wAMD. Majority of the subjects in the study showed improvement in BCVA and/or CST. Most notably, one subject showed strong improvement in visual acuity and retinal anatomy. At month 3, the subject gained 12 letters and the subject's BCVA continued to improve without further treatment. At month 4 (study exit), the subject had a BCVA gain of 15 letters compared to baseline. In this subject, the CST improved by about 200 microns. None of the subjects required rescue with standard of care anti-VEGF injections over the four-month study period except one. This subject showed no improvement in BCVA despite the rescue with Eylea.

"RBM-007 provides a unique approach to the treatment of wet AMD with clinically relevant and meaningful improvements in some subjects with naïve wet AMD." commented Raj Maturi, M.D., the sponsor and principal investigator of the TEMPURA study.

"RIBOMIC's clinical development strategy for RBM-007 was designed to also rationally exploit the molecule's novel and unique activity profile as a monotherapy in treatment-naïve wAMD patients and

we are encouraged by the results even from this small pilot study as the TEMPURA IST. We hope that the new data would generate renewed interest in RBM-007 from potential partners and collaborators,” commented Padma Bezwada, PhD, CEO of RIBOMIC Inc., USA., “Our team would like to thank Dr. Maturi, his staff and the patients for participating in the study”

In tandem, RIBOMIC Inc. provided an update on the secondary outcomes of the TOFU study and the results from the RAMEN study in previously treated wAMD subjects. Further analysis of the TOFU study results shows that RBM-007 alone or as an add-on to Eylea did not demonstrate improvement over Eylea in any of the anatomic endpoints including reduction in retinal thickness or in resolution of subretinal hyper-reflective material/fibrosis. Results of the RAMEN study show no benefit to extended dosing with RBM-007 in pretreated TOFU subjects who rolled over to the open label extension study.

There were no new RBM-007 related safety signals reported in any of the studies.

In summary, based on the three P2 study results, RBM-007 appears effective in improving BCVA and retinal anatomy in treatment-naïve wAMD when compared to eyes previously treated long-term with anti-VEGF agents.

About RBM-007

RBM-007 is a novel oligonucleotide-based aptamer with potent anti-FGF2 (fibroblast growth factor 2) activity. FGF2 is implicated in not only angiogenesis but also fibrosis in several diseases including wAMD. The dual action of RBM-007 (anti-angiogenic and anti-scarring) holds promise as an additive or alternative therapy to anti-VEGF treatments for wAMD. The three P2 studies in wAMD are: 1. Active-controlled, double masked trial, TOFU study (NCT04200248); 2. Single-arm, open-label extension trial, RAMEN (NCT04640272); and 3. Investigator sponsored trial with treatment naïve wAMD patients, the TEMPURA study (NCT04895293).

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 clinical stage bio-pharmaceutical company specializing in the discovery and development of aptamer therapeutics, which is one type of nucleic acid medicine, a field with much potential for the development of next-generation drugs. 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, rare disease of short stature in children and many other diseases.

See RIBOMIC website for more information.

<https://www.ribomic.com/eng/>

Forward-Looking Statements

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