

Bioblast Pharma sells its Trehalose Clinical Development to Seelos Therapeutics

21 February 2019 | News

Under the terms of the agreement, Seelos will pay Bioblast a payment of \$3.5 million in two installments; \$1.5million payable upon closing of the sale and the other \$2.0 million will be paid upon the first anniversary of the closing of the sale.



Bioblast Pharma, a clinical-stage, orphan disease-focused biotechnology company headquartered in Israel, has announced the sale of its Trehalose clinical development programs (including its advanced phase 2 clinical program of Trehalose to treat Oculoharyngeal Muscular Dystrophy (OPMD) to Seelos Therapeutics, Inc, a clinical-stage biopharmaceutical company. Under the terms of the agreement, Seelos will pay Bioblast a payment of \$3.5 million in two installments; \$1.5 million payable upon closing of the sale and the other \$2.0 million will be paid upon the first anniversary of the closing of the sale. Potential additional milestone payments of up to \$17.0 million will be made upon completion of the clinical study and approval of a New Drug Application (NDA) by the Food and Drug Administration (FDA), as well as royalties.

Bioblast announced on February 14, 2019, that it has signed a collaboration agreement with Team Sanfilippo (TSF) for the development of Trehalose for Sanfilippo Syndrome. Under the terms of the collaboration, TSF plans to conduct an open label study in up to 20 Sanfilippo Syndrome patients, which will be followed for safety and efficacy. If successful, based on the results of such study, Seelos, as successor to Bioblast, may determine to continue the development of Trehalose towards a pivotal study for this extra rare disease while maintaining full commercial rights. TSF recently received FDA approval for this study.

All Trehalose programs are currently expected to be further developed by Seelos following Bioblast's previously announced business combination with Enlivex Therapeutics Ltd., which is expected to close in the first quarter of 2019. The current shareholders of Bioblast will have the opportunity to benefit from the potential success of these programs, as well as potentially other programs for the Trehalose technology after the transaction with Enlivex closes and pursuant to the previously announced Contingent Value Rights (CVR) agreement with Enlivex.

"We have great faith in the efficacy of Trehalose for the treatment of several rare diseases" Dalia Megiddo MD, Interim CEO of Bioblast commented: "We are confident that Seelos will continue to develop the clinical programs skilfully for the benefit of the patients as well as for Bioblast's and Seelos' shareholders."

"We view Trehalose as a promising therapy for many patients suffering from rare diseases caused by accumulation of proteins and other large molecules in their cells. , " commented Raj Mehra. Chief Executive Officer of Seelos "We look forward to further developing this therapy for the benefit of patients suffering from rare diseases."

Seelos Therapeutics is a clinical-stage biopharmaceutical company focused on the development and advancement of novel therapeutics to address unmet medical needs for the benefit of patients with central nervous system (CNS) disorders and in rare diseases. Seelos's robust portfolio includes several late-stage clinical assets targeting psychiatric and movement disorders, including orphan diseases. Seelos is based in New York, USA.