MiNA Therapeutics Presents New Pre-Clinical Data from Lead Sickle Cell Disease Program at the American Society of Hematology Annual Meeting

MTL-HBG drug candidate demonstrated best-in-class activity and safety in pre-clinical models

Data supports therapeutic potential in sickle cell disease without necessitating gene editing

London, United Kingdom, December 7, 2024 – MiNA Therapeutics Limited, the pioneer in small activating RNA (RNAa) therapeutics, today presented new pre-clinical data for its lead RNA activation program for the treatment of sickle cell disease, which demonstrated best-in-class activity and safety. The data shared at the 2024 American Society of Hematology (ASH) Annual Meeting in San Diego will also be highlighted at a poster walk session on December 9, 2024, at 12:30 pm PST.

MTL-HBG is an RNAa medicine designed to increase transcription of the gamma globin gene (HBG), enabling people with sickle cell disease to produce enhanced levels of fetal hemoglobin (HbF). HbF is a compensatory form of hemoglobin which, when induced to sufficient levels, protects people with sickle cell disease from a range of symptoms including recurrent vaso-occlusive crises and progressive organ damage.

“The pre-clinical data establish MTL-HBG as a promising drug candidate in sickle cell disease. The levels of fetal hemoglobin safely induced by MTL-HBG underscore the potential to protect from severe symptoms without the need for gene editing,” said Robert Habib, CEO of MiNA Therapeutics. “We are excited by this compelling evidence, which strongly supports advancement of MTL-HBG into IND-enabling studies.”

MTL-HBG is administered *in vivo* without the need for harmful pre-conditioning or complex cell engineering. MTL-HBG comprises an RNAa payload, which directly targets the HBG gene and is encapsulated in NOV340 liposomes. A previous RNAa medicine formulated in NOV340 liposomes has demonstrated biodistribution to greater than 60% of erythroid progenitor cells in bone marrow in non-human primates, and preliminary safety and activity in clinical trials. MiNA anticipates advancing MTL-HBG into IND-enabling studies in 2025.

The poster entitled, “Small Activating RNA-Mediated Induction of HBG Via Liposome Delivery for *In Vivo* Treatment of Sickle Cell Disease and Beta-Thalassemia,” highlights how MTL-HBG induced HbF by 3.6-fold as a proportion of total hemoglobin (% HbF) in erythroid progenitor cells derived from human bone marrow tissue. HbF levels induced by MTL-HBG exceeded 20%, a threshold which is widely recognized to protect people with sickle cell disease from vaso-occlusive crises. The activity in erythroid progenitor cells was demonstrated to be durable, pancellular and highly specific. *In vivo* delivery of MTL-HBG was confirmed in non-human primates as evidenced by an increase in red blood cells containing HbF (F-cells). Monthly administration of MTL-HBG was predicted to achieve 20%-30% HbF in sickle cell patients in an industry-standard model simulation.

MTL-HBG is the first drug candidate to emerge from MiNA’s genetic medicine portfolio.

The poster presentation is available on the MiNA website: www.minatx.com.

About MiNA Therapeutics
MiNA Therapeutics is the leader in small activating RNA therapeutics. Harnessing innate mechanisms of gene activation, small activating RNA therapeutics are a revolutionary new class of medicines that can restore normal function to patients’ cells. We are advancing a

proprietary pipeline of new medicines with an initial focus on cancer and genetic diseases, while collaborating with leading pharmaceutical companies to apply our technology platform across a broad range of therapeutic areas. Based on our unique know-how in RNA activation we are expanding the possibilities of RNA-based medicine for patients. For more information, visit www.minatx.com.

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