

MiNA Therapeutics Presents New Data for its HbF Program at the Keystone Symposia's Delivery of Nucleic Acid Therapeutics Conference

Pre-clinical studies confirm in vivo delivery of small activating RNA therapeutics to erythroid progenitor cells at clinically meaningful levels

Compelling pre-clinical data supports advancement of MiNA's HbF program for development in sickle cell disease and beta thalassemia

London, United Kingdom, 24 January 2024 – MiNA Therapeutics Limited, the pioneer in small activating RNA (RNAa) therapeutics, announced today that it will present new preclinical data for its lead RNA activation program targeting fetal hemoglobin (HbF) at the Keystone Symposia's Delivery of Nucleic Acid Therapeutics Conference in Banff, Alberta, Canada. Pre-clinical studies confirmed *in vivo* delivery of MiNA's RNAa therapeutics to erythroid progenitor cells in bone marrow at levels that are clinically meaningful for the treatment of beta-hemoglobinopathies. The data will be presented on January 24, 2024, at 7:30 pm MST during a poster presentation.

MiNA's HbF program is designed to increase transcription of the gamma globin (HBG) gene, enabling patients with beta-hemoglobinopathies to produce enhanced levels of HbF. HbF is a compensatory form of hemoglobin which has the potential to achieve a functional cure in patients with severe inherited blood disorders such as sickle cell disease and beta thalassemia.

"These results demonstrate for the first time efficient *in vivo* delivery of RNAa therapeutics to erythroid progenitor cells with a delivery technology that is well-suited for safe and effective treatment in the clinic," said Robert Habib, CEO of MiNA Therapeutics. "We are excited by this compelling evidence, which strongly supports advancement of our lead program for the treatment of sickle cell disease and beta thalassemia, which together affect more than five million people around the world."

MiNA's HbF program uses a liposomal delivery technology, NOV340, which efficiently delivers RNAa therapeutics *in vivo* without the need for harmful pre-conditioning or complex cell engineering. NOV340 formulations have established safety and pharmacodynamic activity in clinical trials involving more than 290 patients, including 130 patients treated with MiNA's first RNAa development candidate, MTL-CEBPA. MiNA anticipates advancing its HbF program, the first program to emerge from its genetic medicine portfolio, into pre-clinical development in 2024.

In the presentation, entitled "NOV340 Liposome Encapsulating Nucleic Acid Payload Achieves Efficient Biodistribution to Erythroid Progenitor Cells," data from animal models confirmed *in vivo* delivery to erythroid progenitor cells at levels that met accepted benchmarks for impactful treatment of beta-hemoglobinopathies. In non-human primates, intravenous administration of encapsulated liposomes resulted in delivery of MiNA's RNAa compound to over 60% of committed colony-forming unit erythroid (CFU-E) cells and proerythroblasts (Pro-E) in bone marrow. Equivalent levels of delivery efficiency were observed in peripheral blood monocytes, a cell type in which pharmacodynamic activity of NOV340formulated RNAa therapeutics has previously been shown in clinical studies.

Following the symposia, the poster detailing full study results will be available on the MiNA website: <u>www.minatx.com</u>.

MiNA has previously achieved clinical proof of concept for its RNAa therapeutics platform with MTL-CEBPA. MiNA is exploring out-licensing opportunities for MTL-CEBPA and its



immuno-oncology portfolio, which uniquely combines the capability to specifically restore or boost any dysregulated gene target with clinically validated *in vivo* delivery to myeloid immune cells.

About MiNA Therapeutics

MiNA Therapeutics is the global leader in small activating RNA therapeutics or RNAa. Harnessing innate mechanisms of gene activation, RNAa therapeutics are a revolutionary new class of medicines that can restore or boost normal function of genes and thereby protein-modulated pathways in cells. We are advancing a proprietary pipeline of new medicines with an initial focus on genetic medicine, while collaborating with leading pharmaceutical companies to apply our technology platform across a broad range of other therapeutic areas. Based on our unique know-how in RNA activation, we are expanding the possibilities of RNA-based medicine. www.minatx.com.

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