

MiNA Therapeutics Enters Research Collaboration with BioMarin Pharmaceutical to Advance RNAa Platform Research in Rare Genetic Diseases

- Collaboration includes licensing agreement options based on early-stage clinical outcomes

London, United Kingdom, April 25, 2023 -- MiNA Therapeutics, the pioneer of small activating RNA (RNAa) therapeutics, today announced a multi-target research collaboration and option licensing agreement with BioMarin Pharmaceutical Inc. The collaboration will allow for the discovery, and potential development and commercialization of RNAa therapeutic candidates targeting a number of rare genetic diseases.

Under the agreement, BioMarin will employ MiNA Therapeutics' proprietary RNAa algorithm and technology platform to identify and characterize RNAa molecules targeting a number of genetic diseases for which there are currently no or minimal therapeutic options. The agreement does not cover oncology, or other therapeutic areas outside of genetic disease. BioMarin may then elect to license the assets fully for worldwide development and commercialization.

"We are proud to announce this collaboration with BioMarin, a global leader in the development and commercialization of treatments for rare genetic diseases," said Robert Habib, Chief Executive Officer at MiNA. "Our RNAa therapeutic platform has delivered unique and differentiated clinical benefits in other early clinical studies, demonstrating that MiNA's platform may have significant benefits over other genetic medicine approaches."

As part of the deal, MiNA will receive an upfront payment and future royalties will be subject to BioMarin exercising options from the deal. Financial terms of the agreement were not disclosed.

"Activating RNA therapeutics have the potential to change the way we treat certain genetic diseases, particularly those characterized by the limited production of key proteins," said Kevin Eggan, Ph.D., Senior Vice President and Chief Scientific Officer of BioMarin. "For more than two decades, BioMarin has advanced innovative medicines designed to transform the lives of people with genetic conditions. This collaboration with MiNA continues that legacy of innovation, creating a partnership around a novel scientific approach that we believe will allow us to target the root cause of several genetic disorders in a new way."

MiNA's RNAa platform has been clinically validated in more than 120 patients to date. The universally designed platform has the potential to address any gene given its endogenous mechanism of action that ultimately addresses the root cause of the defective gene and enables the body to self-correct. Furthermore, working at the gene level, RNAa medicines are able to restore a cell's own biology. This holds potential to treat currently "undruggable" diseases.

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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