MiNA Therapeutics Announces Publication of Pre-Clinical Data Supporting On-Target Mechanism of Action of Clinical Candidate MTL-CEBPA

--Data Published in Molecular Therapy--

London, United Kingdom, September 6, 2017 – MiNA Therapeutics, the pioneer in RNA activation therapeutics, today announced the publication of new pre-clinical data supporting the on-target mechanism of action of drug candidate MTL-CEBPA. MTL-CEBPA is the first development candidate to emerge from MiNA's RNA activation platform, consisting of CEBPA-51 small activating RNAs encapsulated in SMARTICLES® nanoparticles, and is currently being evaluated in a Phase I clinical study in patients with liver cancer.

The paper, entitled "*Development and mechanism of small activating RNA targeting CEBPA, a novel therapeutic in clinical trials for liver cancer*", was published in Molecular Therapy by researchers at MiNA Therapeutics in collaboration with scientists at Imperial College London, Norwegian University of Science and Technology, City of Hope and the Scripps Research Institute.

A number of scientific findings of CEBPA-51 were reported including a direct gene activation mechanism evidenced by physical interaction of CEBPA-51 at the CEBPA gene and increased transcriptional activity of the CEBPA gene. These findings are consistent with externally published mechanisms of small activating RNA including the presence and dependence of a cell's endogenous gene regulation machinery. CEBPA-51 was shown to be a highly precise activator of CEBPA, supported by a sequence specific mechanism. In addition, a favourable safety profile of CEBPA-51 was evidenced by the absence of potential off-target effects.

"These comprehensive research findings elegantly support an on-target gene activation mechanism of CEBPA-51, supporting its selection as a component of our first clinical candidate MTL-CEBPA" commented Robert Habib, CEO of MiNA Therapeutics.

The paper is available on the Company's website in the publications section under "Media".

About MTL-CEBPA

MTL-CEBPA consists of a double stranded RNA formulated into a SMARTICLES® liposomal nanoparticle and is designed to activate the CEBPA gene. By restoring CEBPA expression to normal levels, MTL-CEBPA has been demonstrated to attenuate or reverse liver disease in a range of pre-clinical studies including models of liver cancer, liver cirrhosis, non-alcoholic fatty liver disease (NAFLD) and non-alcoholic steatohepatitis (NASH). MTL-CEBPA is currently under evaluation in OUTREACH, a first-in-human Phase I clinical study in patients with severe liver cancer. The multi-centre Phase I study is assessing the safety and tolerability of MTL-CEBPA in patients with advanced liver cancer who are ineligible or resistant to standard therapies. To learn more about the OUTREACH clinical study, please visit our listing at **clinicaltrials.gov**

About MiNA Therapeutics

Harnessing the innate mechanism of gene activation, MiNA Therapeutics' platform enables the development of new medicines that restore normal function to patients' cells. We are applying our technology and clinical know-how to transform the therapy landscape of severe liver and other diseases. www.minatx.com

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