

**FOR IMMEDIATE RELEASE**

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

**--Data Presented at Oligonucleotide Therapeutics Society Annual Meeting--**

**London, United Kingdom, September 27, 2016** – MiNA Therapeutics, the pioneer in RNA activation therapeutics, today announced the presentation of 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 and is currently being evaluated in a Phase I clinical study in patients with liver cancer.

The data were presented at the 2016 Annual Meeting of the Oligonucleotide Therapeutics Society on September 26 in Montreal, Canada, in a poster titled "Development and mechanism of a small activating RNA targeting CEBPA, a novel therapeutic in clinical trials for patients with liver cancer".

"Through a range of methods MTL-CEBPA has been validated to up-regulate the CEBP- $\alpha$  protein through an on-target and highly specific mechanism of transcriptional activation" commented Robert Habib, CEO of MiNA Therapeutics. "This data supports the potential of our RNA activation platform to up-regulate therapeutically 'undruggable' proteins in a highly specific manner."

MTL-CEBPA is a SMARTICLES<sup>®</sup> liposomal formulation of CEBPA-51, a small activating RNA targeting the CEBPA gene. In the experiments covered by the presentation, CEBPA-51 was shown in cell lines to transcriptionally activate expression of CEBPA gene resulting in increased levels of CEBPA mRNA as well as CEBP- $\alpha$  protein. Mutations and modifications to the sequence of CEBPA-51 demonstrated this mechanism to be sequence specific as well as strand specific. Incorporation of 2'O-Me modifications in CEBPA-51 was shown to abrogate immune stimulation without loss of on-target activity. In addition, CEBPA-51 was shown to co-localise with, and require for activity, Argonaute 2 – an enzyme involved in a cell's innate regulation of gene expression.

The poster presented at the Oligonucleotide Therapeutics Society Annual Meeting is available on the Company's website in the publication section under "Media".

### **About MTL-CEBPA**

MTL-CEBPA consists of a double stranded RNA formulated into a SMARTICLES<sup>®</sup> 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 will assess the safety and tolerability of MTL-CEBPA in patients with advanced primary or metastatic 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](https://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 diseases. Our initial product candidate will achieve clinical proof of concept in 2017.

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