

## **MiNA Therapeutics Announces Publication of Pre-Clinical Data Supporting Therapeutic Potential of Clinical Candidate MTL-CEBPA in Liver Cancer and Liver Disease**

--Data Published in Oncogene Demonstrate Improvement of Liver Function and Increased Survival in Multiple Disease Models--

**London, United Kingdom, March 9, 2018** – MiNA Therapeutics, the pioneer in RNA activation therapeutics, today announced the publication of pre-clinical data on its MTL-CEBPA program in which the compound was shown to promote disease reversal in several models of liver disease and to reduce tumour burden in a model of liver cancer. MTL-CEBPA consists of CEBPA-51 small activating RNAs encapsulated in SMARTICLES® nanoparticles. It 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 underscore the potential of this compound to address both severe liver disease indications as well as earlier disease stages to enhance chances of survival.

“The combination of beneficial effects observed across a range of liver disease models supports a unique and promising role for MTL-CEBPA therapy in the treatment of liver diseases and liver cancer,” commented Robert Habib, CEO of MiNA Therapeutics. “This exciting pre-clinical data highlights the potential of saRNAs to up-regulate gene expression and treat disease in radically new ways compared to conventional medicines.”

In the publication, researchers investigated the potential benefits of MTL-CEBPA in a set of *in vivo* severe liver disease models representing advanced liver cirrhosis, non-alcoholic steatohepatitis (NASH) and liver cancer. Overall, MTL-CEBPA was shown to restore the expression of CEBPA, a master regulator of liver function. In the advanced liver cirrhosis model, MTL-CEBPA significantly reversed liver fibrosis and liver dysfunction and enhanced survival. In a model of NASH, MTL-CEBPA reversed liver steatosis. Improved liver function as well as a large reduction in tumour burden was also seen in a model of primary liver cancer. Together, these findings validate the beneficial role of up-regulating CEBPA expression in liver disease and liver cancer and are consistent with externally published data using genetic models of liver disease.

The publication titled “*Gene activation of CEBPA using saRNA: Preclinical studies of the first in human saRNA drug candidate for liver cancer*”, was published in the latest issue of *Oncogene* by researchers at MiNA Therapeutics in collaboration with several well-regarded academic institutions including scientists at Imperial College London and National Taiwan University Hospital. 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](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 and other diseases. [www.minatx.com](http://www.minatx.com)

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