MiNA Therapeutics Presents Initial Results from First-in-Human MTL-CEBPA Study in Advanced Liver Cancer Patients

--Well tolerated in patients with healthy and impaired liver function--
--Blood samples demonstrate proof of RNAa mechanism--
--Evidence of anti-tumour activity in heavily pre-treated patients--

London, United Kingdom, June 4, 2018 – MiNA Therapeutics, the pioneer in RNA activation (RNAa) therapeutics, today announced preliminary results from its ongoing Phase I study of small activating RNA (saRNA) candidate MTL-CEBPA in advanced liver cancer. In the study, MTL-CEBPA was generally well tolerated in patients with both healthy and impaired liver function and provided evidence of anti-tumour activity. MTL-CEBPA was also found to mediate RNAa activity in white blood cells. The data are being presented at the 2018 American Society of Clinical Oncology (ASCO) Annual Meeting in a poster titled "Preliminary results of a first-in-human, first-in-class phase I study of MTL-CEBPA, a small activating RNA (saRNA) targeting the transcription factor C/EBP-a in patients with advanced liver cancer" in the Developmental Therapeutics – Clinical Pharmacology and Experimental Therapeutics poster discussion session being held on Monday June 4, 2018 from 3:00pm to 4:15pm CDT.

"Despite recent advances in treatment options, liver cancer remains a significant unmet medical need with numerous hurdles for therapeutic intervention. New treatment options are desperately needed, in particular for those patients with impaired liver function," said Dr. Debashis Sarker, Principal Investigator at the National Institute for Health Research Clinical Research Facility at Guy's and St Thomas' and King's College London, and chief investigator of the study. "These preliminary safety data and the evidence of anti-tumour activity are very promising and I look forward to evaluating MTL-CEBPA in the dose expansion part of this Phase I clinical trial."

"We are extremely pleased with the preliminary results of this first-in-human study which include safety and tolerability of MTL-CEBPA, as well as evidence of anti-tumour activity in this very advanced, heavily pre-treated cancer patient population. In particular we have seen many patients achieve stable disease or better, including a patient with advanced hepatocellular carcinoma who has achieved over 70% tumour regression and has continued on the study for over one and half years," said Robert Habib, CEO of MiNA Therapeutics. "Additionally, analysis of patient blood samples has demonstrated upregulation of target CEBPA mRNA in white blood cells, representing a significant milestone in the development of saRNA medicines and for our platform."

MTL-CEBPA was evaluated in the dose escalation part of a Phase I clinical trial in patients with advanced liver cancer. As of the data cut-off date of March 31, 2018, 23 patients had been treated once weekly at six dose levels (ranging from 28 mg/m2 to 160 mg/m2) and 5 patients had been treated twice weekly at 70 mg/m2.

MTL-CEBPA was well tolerated in patients at all doses and no Maximum Tolerated Dose was identified. The large majority of adverse events (AEs) reported by investigators were mild to moderate in severity. 12 (43%) patients experienced AEs no higher than Grade 2. AEs of Grade 3 or higher included hyperbilirubinaemia (11%), elevated GGT (11%), hypophosphataemia (11%), anaemia (7%) and hypertension (7%). Only 3 (11%) patients discontinued treatment with MTL-CEBPA due to possible drug-related toxicities including acute coronary syndrome, hyperbilirubinaemia, and elevated GGT.

Pharmacokinetic data from this study showed that Cmax (peak plasma concentration of drug) and AUC (area under the curve) were dose proportional with no evidence of drug accumulation.

CEBPA gene expression was analysed in white blood cells of 10 patients across multiple dose levels and timepoints. The level of CEBPA gene expression was significantly higher on treatment than at baseline, supporting target engagement of MTL-CEBPA. Consistent with up-regulation of CEBPA, which has a role in myeloid differentiation, significant and repeated increases in neutrophils were observed after dosing MTL-CEBPA.

Enrollment in the dose escalation part of the Phase I clinical trial has been completed. Enrollment is starting for the dose expansion part of the Phase I clinical trial in multiple sites in the United Kingdom and Asia. For more information, please contact us at outreach@minatx.com.

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 an 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

About the NIHR Clinical Research Facility for Experimental Medicine at Guy's and St Thomas' NHS Foundation Trust
Our Clinical Research Facility provides world-leading facilites and expertise to support the NHS, universities and industry in conducting experimental medicine studies.

Advantages of our Clinical Research Facility include:

- MHRA Phase I accreditation - our Guy's unit is ideally placed to deliver Phase I clinical trials.
- We are the only UK Clinical Research Facility with embedded Advanced Therapies Manufacturing (GMP) and immune monitoring platforms
- Four specialist units including our Imaging core and Children's Facility within Evelina London Children's Hospital

The Facility is part of the NIHR Biomedical Research Centre at Guy's and St Thomas' and King's College London, helping the Centre achieve its aim of turning scientific discoveries into better healthcare for everyone.
http://www.guysandstthomasbrc.nihr.ac.uk/

About the National Institute for Health Research
The National Institute for Health Research (NIHR): improving the health and wealth of the nation through research.

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- funds high quality research to improve health
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