Regulation of C/EBPα Inhibits Suppressive Activity of Myeloid Cells and Potentiates Antitumor Response in Mice and Cancer Patients

**Background**

Transcriptional factors regulating the function of myeloid cells represent an attractive targeting opportunity because of their broad effect on the function of these cells. The transcription factor CCAAT/enhancer-binding protein alpha (C/EBPα) is involved in differentiation of myeloid cells, red blood cell metabolism, and immunity. Deregelation of C/EBPα has been reported in several solid tumors including liver, breast, and lung. We have developed a first-in-class small activating RNA therapeutics comprising a SMARTICLES® liposomal nanoencapsulating CEBPα-S1, a 2′-O-Me RNA oligonucleotide duplex designed to specifically target and upregulate the transcription of the CEBPA gene. The mechanism of antitumor effect of MTL-CEBPA, as well as its possible effect in cancer patients remains unclear.

**Mechanism of MTL-CEBPA Regulation in Myeloid Cells in Mice**

**Efficacy of MTL-CEBPA in combination with Sorafenib in Patients with Liver Cancer**

**Clinical activity of MTL-CEBPA in advanced HCC patients treated in combination with sorafenib.**

**Author Affiliation**

- **MiNA Therapeutics**
- **National University Cancer Institute Singapore, Singapore**
- **Wistar Institute, Philadelphia, PA, US**
- **Therapeutics Ltd, London, UK**
- **HalioDx, Marseille, France**
- **University of Cambridge, Cambridge, UK**
- **Pan Colorectal Cancer Center, London, UK**
- **Lewis Cancer Institute, Birmingham, AL**
- **Department of Gastrointestinal and Medical Oncology, MD Anderson Cancer Center, Houston, TX**
- **National Cancer Center, Singapore, Singapore**
- **Institute of Molecular and Cell Biology, Bioinformatics Research Institute of Telecommunications Public Authority, Singapore**

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