**July 3rd, 2025**

# Acuitas Therapeutics Collaborates on mRNA-LNP Prime Editing Study for Genetic Liver Disease

**Vancouver, B.C.** – Acuitas Therapeutics today announced its collaboration on a landmark study published in *Nature Biomedical Engineering*, titled "Treatment of a metabolic liver disease in mice with a transient prime editing approach," which highlights the therapeutic potential of lipid nanoparticle (LNP)-mediated mRNA delivery for prime editing *in vivo*. The study – co-authored by Acuitas scientists Ying Tam, Jennifer Moon, Paulo Lin, and Steven Fan, along with partners from the University of Zurich, ETH Zurich, and the University of Pennsylvania – presents a novel approach to correct a disease-causing mutation in a mouse model of phenylketonuria (PKU), an inherited metabolic liver disorder.

The research team used two distinct strategies for liver-targeted prime editing, both of which delivered the prime editor (PE) as nucleoside-modified mRNA encapsulated in Acuitas' LNP. In the first strategy, the guide RNA (pegRNA) was stably expressed from an adeno-associated virus while in the second, the pegRNA was delivered as synthetic RNAs using LNP delivery. Using these approaches, they achieved up to 47.4% editing at the *Dnmt1* locus and up to 20.7% correction of the pathogenic *Pahenu2* mutation in PKU mice with the enhanced PE7 variant – successfully reducing blood phenylalanine concentrations below the therapeutic threshold of 360 µmol/L.

The transient nature of mRNA-LNP delivery allowed for high editing efficiency, while minimizing prolonged editor expression and the resultant potential off-target effects, which are concerns with viral delivery systems. These findings highlight the promise of non-viral, mRNA-LNP-based prime editing as a scalable and clinically relevant platform for treating monogenic liver diseases.

Click here to read the full publication.

**About Acuitas Therapeutics**

Acuitas Therapeutics is a global leader in lipid nanoparticle (LNP) technology and partners with pharmaceutical and biotechnology companies, as well as non-governmental organizations and academic institutions, to advance nucleic acid therapeutics into clinical development and commercialization. Acuitas' clinically validated LNP technology has enabled COMIRNATY® (Pfizer-BioNTech), the first approved mRNA vaccine, which has been deployed globally, and ONPATTRO® (Alnylam), the first approved RNAi therapeutic. Acuitas has also enabled k-abe, the first LNP enabled personalized CRISPR gene editing therapy, in addition to the first in-human genome base editing trial.

Current efforts focus on enhancing LNP to advance novel gene therapies, in addition to the identification of potent new lipids to enable partners to develop vaccines for infectious diseases, multivalent vaccines, and novel therapeutic vaccines against cancer, including personalized cancer vaccines.

For more information, visit www.acuitastx.com.

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