July 29, 2025

Acuitas Therapeutics' Scientists Collaborate with the University of Zurich and the University of Pennsylvania to Develop a Novel Deep Learning Model for Efficient Adenine Base Editing

Vancouver, B.C. – Acuitas Therapeutics today announced the publication of a new study in *Genome Biology*, titled "Predicting adenine base editing efficiencies in different cellular contexts by deep learning," demonstrating a significant advancement in genome editing. Co-authored by Acuitas scientists Ying Tam, Jennifer Moon, Paulo Lin, and Steven Fan, along with partners from the University of Zurich and the University of Pennsylvania, the study shows that adenine base editing – a precise method of DNA modification – can be accurately predicted across different biological contexts using a new machine learning model, BEDICT2.0.

The team screened 12,000 guide RNAs targeting over 2,100 pathogenic mutations using six adenine base editor (ABE) variants delivered by either adeno-associated virus vectors or Acuitas' lipid nanoparticles. The study revealed that base editing efficiencies observed *in vitro* using plasmid delivery did not reliably predict outcomes *in vivo*. However, editing outcomes *in vitro* using mRNA electroporation strongly correlated with *in vivo* results, likely due to mRNA delivery better recapitulating the physiological expression of the editor after *in vivo* delivery. Based on this insight, the authors developed BEDICT2.0, a deep learning model trained on mRNA-delivered base editing data. BEDICT2.0 accurately predicts editing efficiencies and bystander effects in *in vitro* and *in vivo* contexts, specifically outperforming previous models, including DeepABE and BE-HIVE for *in vivo* modelling.

This study describes an invaluable tool for selecting optimal ABE-sgRNA pairs to maximize editing efficiency, while minimizing bystander effects, advancing the clinical viability of base editing therapies that can be efficiently delivered as AAV or as a mRNA/gRNA combination in lipid nanoparticles.

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.

-END-