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US manufactures world's first mRNA-based personalised CRISPR therapy

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N of 1 therapy uniquely developed, on demand, for infant with life-threatening rare metabolic disorder in six months



Aldevron, a global leader in the production of DNA, RNA and protein, together with Integrated DNA Technologies (IDT), a global leader in genomics solutions, have announced the successful manufacture of the world's first personalised CRISPR gene editing drug product to treat an infant with urea cycle disorder (UCD).

With no current cure for UCDs, the Children's Hospital of Philadelphia (CHOP) and the University of Pennsylvania (Penn) engaged Aldevron and IDT, both part of Danaher Corporation, to manufacture a novel mRNA-based personalised CRISPR therapy in six months—three times faster than the standard timeline for gene editing drug products.

The technically complex, N of 1 therapy required a new guide RNA (gRNA) sequence, new mRNA-encoded base editor, custom off-target safety services and a clinically validated lipid nanoparticle (LNP) formulation, marking an industry milestone that demonstrates how the US continues to lead the way in mRNA gene editing therapies to improve human health for all.

Aldevron provided the mRNA and worked with Acuitas Therapeutics, a private biotechnology company specialising in the development of LNP delivery systems for nucleic acid therapeutics. Together with the gRNA and safety services provided by IDT, the companies delivered a customised *in vivo* base-editing therapy, in a significantly compressed timeline, as a transformational therapy for the infant patient. Collaboration between all partners' quality and regulatory teams also led to successful Emergency Investigational New Drug (EIND) approvals.