Evaluate Vantage

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Gene editing: overhyped or unstoppable tide?

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A new report from Evaluate Vantage looks at recent developments in gene editing and delivery.

Only 10 years since Crispr made a splash as a possible therapeutic approach, the first product using Crispr/Cas9 gene editing is on the verge of approval. Vertex and Crispr Therapeutics' exa-cel, for sickle cell anaemia and beta thalassaemia, has been filed with US and European regulators.

Alongside this breakneck speed of development, however, are doubts about whether gene editing will become mainstream. A just-published report from *Evaluate Vantage*, which features interviews with some of the major players, takes a look at the big questions facing the field.

This includes whether there will still be a place for ex vivo editing, despite the rise of in vivo technology. And are these in vivo projects coming under more scrutiny than previous approaches, or is there just general wariness about anything new?

And what about the way these therapies are delivered? Delivery, once an overlooked piece of the puzzle, is getting increasing attention, and several recently-launched players are developing new technologies that go beyond viral vectors and lipid nanoparticles (LNPs). Some of these groups are also profiled here.

Topics discussed in this free report include:

- The upcoming approval decision on Vertex/Crispr Therapeutics ex vivo edited project exa-cel, and the candidates coming behind;
- The clinical hold for Verve Therapeutics' VERVE-101, which was followed by swift IND clearance for Intellia's NTLA-2002;
- The potential advantages of new modalities like base and prime editing over Crispr/Cas9;
- New delivery modalities including "barcoded" LNPs, protein nanoparticles and virus-like particles;
- Getting to targets other than the liver, including haematopoietic stem cells.

The companies profiled are:

- **Verve Therapeutics**, which is using in vivo base editing to target PCSK9 for high cholesterol;
- **Beam Therapeutics**, a base editing specialist;
- **Prime Medicine**, which is focused on prime editing and went public last year;
- **Life Edit Therapeutics**, a subsidiary of Elevatebio that recently signed a partnership with Moderna;
- **Metagenomi**, another Moderna partner with broad editing efforts;

- **Aera Therapeutics**, a company founded by the Crispr pioneer Feng Zhang that is using human proteins for delivery;
- **Ensoma**, which is targeting gene editing to haematopoietic stem cells using virus-like particles;
- And **Editas Medicine**, which is gunning for a comeback after a torrid couple of years.

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