Pivotal year for gene therapy: Slew of CRISPR treatments will hit the market in 2023

Currently, there are no gene editing–based treatments on the market, but the technology continues its march toward potential FDA approval, with several products in mid- and late-stage trials. As these programs mature, 2023 could be a pivotal year for companies in the space. Here are some highlights to look forward to as the year progresses.

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CRISPR Therapeutics and Vertex Pharmaceuticals completed the submission of a rolling Biologics License Application (BLA) to the FDA for their product, exa-cel, as a potential treatment for sickle cell disease (SCD) and transfusion-dependent beta thalassemia (TDT).

In March 2023, Intellia Therapeutics received a Regenerative Medicine Advanced Therapy (RMAT) designation from the FDA for NTLA-2002, an in vivo CRISPR-based treatment for hereditary angioedema (HAE).

Beam Therapeutics’ specialty is in base editing, and the company intends to use its CRISPR-based technology to edit point mutations. Beam’s most advanced pipeline product is BEAM-10, which is in Phase I/II BEACON trials. BEAM-101 serves as an ex vivo treatment for SCD and beta thalassemia via activation of fetal hemoglobin.

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