10-year CRISPR anniversary: How gene editing revolutionized medicine, and what lies ahead

Ten years ago, a little-known Science paper authored by Jennifer Doudna, Ph.D., and Emmanuelle Charpentier, Ph.D., proposed using CRISPR/Cas9 for gene editing. As the first wave of gene-editing-based therapies post clinical data and head to the FDA, biopharma executives at the forefront of the burgeoning field highlighted the innovations and challenges before gene editing is ready for prime time.

Now, some of the early efforts of turning CRISPR gene editing technology into viable therapies are coming to fruition. Vertex and partner CRISPR Therapeutics just announced their plan to file their CRISPR/Cas9-edited cell therapy exagamglogene autotemcel (exa-cel) for a rolling review at the FDA in sickle cell disease and beta thalassemia starting in November. If approved, exa-cel could be the first CRISPR-based therapy available.

Meanwhile, Intellia Therapeutics, after being the first to show that systemic infusion of CRISPR inside the human body could treat disease, recently reported more positive early data for its in vivo gene editing candidates for transthyretin amyloidosis (ATTR) and hereditary angioedema.

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For now, most gene editing projects are focused on rare diseases and some blood or cancer indications with well-established genetic drivers. Those diseases have clear clinical endpoints and risk-benefit understanding to allow for a quick drug development path.

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