Harvard Model Congress
Boston 2024

Gene Editing Update

By Cameron Reckard

With a breakthrough in the use of CRISPR as a medicinal product, the landscape for gene editing is continuing to change in the United States and abroad. Casgevy, a product of Vertex Pharmaceuticals designed to treat sickle cell anemia, was approved for general treatment by the Food and Drug Administration (FDA) in early December (Corbley, 2023). This approval, after months of the treatment existing in a trial format, enables doctors to prescribe it without requiring the patient to jump through additional hurdles and application processes like before (Corbley, 2023).

In short, the treatment has been seen as a massive success, including among the patients who have elected to receive it during its trial stages. In the words of one 29-year-old woman, Casgevy enabled her to feel "just like a regular person" and far exceed her doctor's original estimation of an 11-year lifespan (Lovelace and Kopf, 2023). The catch: a 2.2-million-dollar price tag (Lin 2023).

With this approval, other nations are taking a closer look at expanding access to the treatment as well. In fact, the European Union (EU is now considering its move on Casgevy after its Committee for Medicinal Products for Human Use (CHMP) recommended the government to follow the same path as the United States. While a specific decision is not expected till February, and while the EU is not required to follow the CHMP's recommendations, many believe an approval is on the horizon across the Atlantic as well, heralding in a new era in medicine (Shapiro, 2023).

In addition to the new treatment, the medical sector continues to gain new players promising new treatments for existing diseases. For instance, just days after the FDA approval of Casgevy, Tome Biosciences emerged into the industry with a sizeable funding base of over $200 million. Its promise: solutions to "monogenetic liver diseases and cell therapies for autoimmune diseases," with additional details said to come soon (Philippidis, 2023). Still others are looking to the brain, attempting to solve Alzheimer's in the same way Casgevy cures sickle-cell disease: by simply "cutting out" the faulty genetic code. However, these treatments are still years away from receiving the same approval Vertex Pharmaceuticals received, with many clinical trials and studies ahead of any new medical treatment (Thompson, 2023).

Absent during these massive approvals, however, is legislation from Congress on dealing with these new genetic treatment options. Excluding two largely similar drafts on requesting the Secretary of Health and Human Services to provide guidance on gene synthesis (authored by Senators John Hickenlooper (D-CO) and Edward Markey (D-

MA)), no new legislation has been introduced to Congress about gene editing since mid-July 2023. Both pieces also are only in the initial stages for the process, signaling a long road to a chance at approval (S.2356, 2023 and S. 2400, 2023).

Delegates should consider how to react to these recent events when preparing for the conference. With new treatment options using CRISPR technology available to the broader public (although still firmly out of financial reach for some), what new action needs to be taken by Congress to better fit the new landscape in medicine? How might these approvals affect other industries looking to utilize gene editing themselves? While this is far from an exhaustive list, we encourage delegates to ponder these questions and establish answers to them.

BIBLIOGRAPHY

Corbley, Andy. "FDA Approves Cure for Sickle Cell Disease, the First Treatment to Use Gene-Editing Tool CRISPR." *Good News Network*, 16 Dec. 2023, www.goodnewsnetwork.org/fda-approves-cure-for-sickle-cell-disease-the-first-treatment-to-use-gene-editing-tool-crispr/.

Lin, Kenny. "Will the High Price of Gene Therapy for Sickle Cell Disease Put This Cure out of Reach?" *AAFP*, American Academy of Family Physicians, 12 Dec. 2023, www.aafp.org/pubs/afp/afp-community-blog/entry/will-the-high-price-of-gene-therapy-for-sickle-cell-disease-put-this-cure-out-of-reach.html#:~:text=The%20approval%20of%20Casgevy%2C%20which,to%20afford%20it%20is%20unclear.

Lovelace, Berkeley, and Marina Kopf. "FDA Approves Cure for Sickle Cell Disease, the First Treatment to Use CRISPR." *NBC News*, NBCUniversal News Group, 8 Dec. 2023, www.nbcnews.com/health/health-news/fda-approves-cure-sickle-cell-disease-first-treatment-use-gene-editing-rcna127979.

Philippidis, Alex. "The next Chapter in Genome Editing? Tome Emerges from Stealth with $213M." *GEN - Genetic Engineering and Biotechnology News*, Mary Ann Liebert, Inc., 19 Dec. 2023, www.genengnews.com/topics/genome-editing/the-next-chapter-in-genome-editing-tome-emerges-from-stealth-with-213m/.

"S. 2400 — 118th Congress: Securing Gene Synthesis Act." www.GovTrack.us. 2023. December 19, 2023, https://www.govtrack.us/congress/bills/118/s2400

"S. 2356 — 118th Congress: Gene Synthesis Safety and Security Act." www.GovTrack.us. 2023. December 19, 2023, https://www.govtrack.us/congress/bills/118/s2356

Shapiro, Lindsey. "Casgevy, Sickle Cell Gene-Editing Therapy, Favored for EU Approval." *Sickle Cell Disease News*, BioNews, Inc., 19 Dec. 2023,

sicklecellanemianews.com/news/casgevy-sickle-cell-gene-edited-therapy-favored-eu-approval/.

Thompson, Tosin. “HOW CRISPR Gene Editing Could Help Treat Alzheimer’s.” Nature News, Nature Publishing Group, 11 Dec. 2023, www.nature.com/articles/d41586-023-03931-5.