



Vertex to Present New Data on Exagamglogene Autotemcel (exa-cel) at the American Society of Hematology (ASH) Annual Meeting and Exposition

November 2, 2023

- New data including longer duration follow-up from SCD and TDT pivotal trials accepted for oral presentation –
- Five additional abstracts accepted for poster presentation -

BOSTON--(BUSINESS WIRE)--Nov. 2, 2023-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today announced that seven abstracts have been accepted for presentation at the 2023 American Society of Hematology (ASH) Annual Meeting and Exposition. This includes two presentations of updated clinical data describing additional patients with longer follow-up duration from pivotal Phase 3 trials investigating exagamglogene autotemcel's (exa-cel) potential as a one-time functional cure for sickle cell disease (SCD) and transfusion-dependent beta thalassemia (TDT).

- Abstract #1052 entitled "Exagamglogene Autotemcel for Severe Sickle Cell Disease" will be an oral presentation on Monday, December 11 at 4:45 p.m. PST.
- Abstract #1053 entitled "Exagamglogene Autotemcel for Transfusion-Dependent Beta-Thalassemia" will be an oral presentation on Monday, December 11 at 5:00 p.m. PST.

Five additional abstracts have been accepted, including two on the improvements in patients' quality of life following treatment with exa-cel.

- Abstract #4997 entitled "Improvements In Health-Related Quality of Life After Exagamglogene Autotemcel in Patients with Transfusion-Dependent Beta Thalassemia" will be a poster presentation on Monday, December 11 from 6:00-8:00 p.m. PST.
- Abstract #4999 entitled "Improvements In Health-Related Quality of Life After Exagamglogene Autotemcel in Patients With Severe Sickle Cell Disease" will be a poster presentation on Monday, December 11 from 6:00-8:00 p.m. PST.
- Abstract #3674 entitled "VOC-free Status Among Patients with Sickle Cell Disease Following Allogeneic Hematopoietic Stem Cell Transplant: A Cohort Study of Medicaid Enrollees" will be a poster presentation on Sunday, December 10 from 6:00-8:00 p.m. PST.
- Abstract #3678 entitled "The Impact of Recent Vaso-Occlusive Crisis on Health-Related Quality of Life in Adults with Sickle Cell Disease" will be a poster presentation on Sunday, December 10 from 6:00-8:00 p.m. PST.
- Abstract #3900 entitled "Estimating Sickle Cell Disease Prevalence by State: A Model Using US-born and Foreign-born State-specific Population Data" will be a poster presentation on Monday, December 11 from 6:00-8:00 p.m. PST.

The accepted abstracts are available online on the [ASH website](#).

About exagamglogene autotemcel (exa-cel)

Exa-cel is an investigational, autologous, ex vivo CRISPR/Cas9 gene-edited cell therapy that is being evaluated for patients with SCD or TDT, in which a patient's own hematopoietic stem cells are edited to produce high levels of fetal hemoglobin (HbF; hemoglobin F) in red blood cells. HbF is the form of the oxygen-carrying hemoglobin that is naturally present during fetal development, which then switches to the adult form of hemoglobin after birth. The elevation of HbF by exa-cel has the potential to reduce or eliminate painful and debilitating VOCs for patients with SCD and alleviate transfusion requirements for patients with TDT. Earlier results from these ongoing trials were published in The New England Journal of Medicine in January of 2021 and presented at the European Hematology Association Congress in 2023.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. The company has multiple approved medicines that treat the underlying cause of cystic fibrosis (CF) — a rare, life-threatening genetic disease — and has several ongoing clinical and research programs in CF. Beyond CF, Vertex has a robust clinical pipeline of investigational therapies across a range of modalities in other serious diseases where it has deep insight into causal human biology, including sickle cell disease, beta thalassemia, APOL1-mediated kidney disease, acute and neuropathic pain, type 1 diabetes and alpha-1 antitrypsin deficiency.

Vertex was founded in 1989 and has its global headquarters in Boston, with international headquarters in London. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia and Latin America.

Vertex is consistently recognized as one of the industry's top places to work, including 14 consecutive years on Science magazine's Top Employers list and one of Fortune's 100 Best Companies to Work For. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on [LinkedIn](#), [Facebook](#), [Instagram](#), [YouTube](#) and [Twitter/X](#).

(VRTX-GEN)

Vertex Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements regarding the plans to present abstracts at the ASH Annual Meeting and Exposition and our expectations for the benefits of exa-cel. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of risks and uncertainties that could cause actual events or results to differ materially from those expressed or implied by such forward-looking statements. Those risks and uncertainties include, among other things, that data from the company's studies may not be indicative of final clinical trial results, that data from the company's development programs may not support registration or further development of its compounds due to safety, efficacy, or other reasons, that data may not be available on the anticipated timeline, or at all, that our development programs may experience delays, and other risks listed under the heading "Risk Factors" in Vertex's most recent annual report and subsequent filings filed with the Securities and Exchange Commission at www.sec.gov and available through the company's website at www.vrtx.com. You should not place undue reliance on these statements or the scientific data presented. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

View source version on [businesswire.com](https://www.businesswire.com/news/home/20231102249827/en/): <https://www.businesswire.com/news/home/20231102249827/en/>

Vertex Pharmaceuticals Incorporated

Investors:

InvestorInfo@vrtx.com

Susie Lisa, CFA: +1 617-341-6108

or

Manisha Pai: +1 617-961-1899

Media:

mediainfo@vrtx.com

or

U.S.: +1 617-341-6992

or

Heather Nichols: +1 617-839-3607

Source: Vertex Pharmaceuticals Incorporated