FOOD AND DRUG ADMINISTRATION (FDA)
Center for Biologics Evaluation and Research (CBER)
76th Meeting of the Cellular, Tissue, and Gene Therapies Advisory Committee (CTGTAC)
October 31, 2023
FINAL AGENDA

The committee will meet in open session to discuss and make recommendations on biologics license application (BLA) 125787 from Vertex Pharmaceuticals, Inc. for exagamglogene autotemcel (exa-cel). The applicant has requested an indication for the treatment of sickle cell disease in patients 12 years and older with recurrent vaso-occlusive crises.

Time EDT	Presentation/Presenter
9:00 a.m.	**Opening Remarks: Call to Order and Welcome (5 Min)** Tabassum (Taby) Ahsan, Ph.D., Acting Chairperson, CTGTAC Vice President, Cell Therapy Operations City of Hope, Duarte, CA **Administrative Announcements, Roll Call, Introduction of Committee, Conflict of Interest Statement (20 Min)** LCDR Cicely Reese, Pharm.D., Designated Federal Officer Division of Scientific Advisors and Consultants (DSAC), OM, CBER, FDA
9:25 a.m.	**FDA Introduction (5 Min)** **Introductory Remarks** • **Nicole Verdun, M.D.** Director Office of Therapeutic Products (OTP), CBER, FDA
9:30 a.m.	**Guest Speaker Presentations (55 Min including Q & A)** **Genetic Editing** • **Fyodor Urnov, Ph.D. (20 Min)** Professor, Department of Molecular and Cell Biology University of California, Berkeley Director of Technology and Translation Innovative Genomics Institute Berkeley, California

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	Off-Targets of Genetic Editing • **Daniel E. Bauer, M.D., Ph.D. (20 Min)** Principal Investigator and Staff Physician Dana-Farber/Boston Children's Cancer and Blood Disorders Center Boston Children's Hospital Boston, MA **Q & A (15 Min)**
10:25 a.m.	**BREAK (10 min.)**
10:35 a.m.	**<u>Applicant Presentations</u> (75 Min including Q & A)** **Exa-cel for the Treatment of Sickle Cell Disease (SCD) in Patients ≥ 12 Years With Recurrent Vaso-Occlusive Crises (VOCs).** Introduction — **Stephanie Krogmeier, Ph.D.** Vice President, Global Regulatory Affairs Vertex Pharmaceuticals Incorporated Unmet Need — **Alexis Thompson, M.D., M.P.H.** Division Chief, Hematology Children's Hospital of Philadelphia Efficacy — **William Hobbs, M.D., Ph.D.** Vice President Clinical Development, Hematology Vertex Pharmaceuticals Incorporated Non-Clinical Safety — **David Altshuler, M.D., Ph.D.** Executive Vice President and Chief Scientific Officer, Vertex Pharmaceuticals Incorporated Clinical Safety — **Christopher Simard, M.D.** Vice President, Global Patient Safety Vertex Pharmaceuticals Incorporated

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	Clinical Perspective **Haydar Frangoul, M.D.** Medical Director, Pediatric Hematology/Oncology Tristar Centennial Medical Center **Q & A (15 Min)**
11:50 a.m.	**LUNCH (45 Min)**
12:35 p.m.	**Open Public Hearing (60 Min)**
1:35 p.m.	**BREAK (10 Min)**
1:45 p.m.	**FDA Presentations (75 Min including Q & A)** **BLA 125787 Exagamglogene autotemcel (exa-cel)** • **Karl Kasamon, M.D.** Reviewer, Officer of Clinical Evaluation Division of Hematology, Benign Hematology Branch, OTP, CBER. FDA • **Komudi Singh, Ph.D**. Bioinformatics Reviewer, Office of Cellular Therapy and Human Tissue OTP, CBER, FDA **Q & A (15 Min)**
3:00 p.m.	**Committee Discussion (110 min)** • Discussion Question
4:50 p.m.	**Closing Remarks (10 Min)** • **Nicole Verdun**
5:00 p.m.	**ADJOURNMENT**