FOOD AND DRUG ADMINISTRATION (FDA)
Center for Biologics Evaluation and Research (CBER)
Cellular, Tissue, and Gene Therapies Advisory Committee (CTGTAC)
June 10, 2022
FINAL AGENDA

TOPIC SUMMARY

June 10, 2022: Topic II:

The committee will meet in open session to discuss the biologic application (BLA) BLA 125717 from bluebird bio, Inc. for betibeglogene autotemcel (autologous CD34+ stem cells genetically modified with a lentiviral vector to contain a gene encoding functional beta-globin); the applicant has requested an indication for the "treatment of patients with β-thalassemia who require regular red blood cell transfusions."

DAY 2: JUNE 10

EDT 10:00 a.m.	Opening Remarks: Call to Order and Welcome	**Lisa Butterfield, Ph.D.** (5 min) Chairperson Vice-President, PICI Research Center University of California, San Francisco
10:05 a.m.	Administrative Remarks, Roll Call, Introduction of Committee, Conflict of Interest Statement	**Christina Vert, M.S.** (15 min) Designated Federal Officer, CTGTAC DSAC, CBER, FDA
10:20 a.m.	FDA Opening Remarks	**Wilson W. Bryan, M.D.** (5 min) Director Office of Tissues and Advanced Therapies

Session 4: beta-thalassemia Efficacy and Safety

10:25 a.m.	**Applicant Presentations**	**bluebird bio, Inc.** (45 min)
	Introduction	**Anne-Virginie Eggimann, MSc.** Chief Regulatory Officer, bluebird bio, Inc.
	Unmet Medical Need	**Sujit Sheth, MD** Chief, Pediatric Hematology/Oncology & Professor of Clinical Pediatrics at Weill Cornell Medical Center
	Efficacy	**Rich Colvin, MD, PhD** Chief Medical Officer, bluebird bio, Inc.
	Safety	**Ajay Singh, MD** Vice President Pharmacovigilance, bluebird bio, Inc.

	Benefit-Risk	**Alexis Thompson, MD, MPH** Chief, Hematology Children's Hospital of Philadelphia
11:10 a.m.	**FDA Presentation** BLA 125717 Betibeglogene autotemcel (beti-cel) Treatment of patients with β-thalassemia who require regular red blood cell (RBC) transfusions	**Karl Kasamon, M.D.** (45 min) OTAT Clinical Reviewer Division of Clinical Evaluation and Pharmacology/Toxicology (DCEPT) OTAT, CBER
11:55 a.m.	Clarifying Questions to Presenters	(30 min)
12:25 p.m.	**LUNCH BREAK**	(35 min)
1:00 p.m.	**OPEN PUBLIC HEARING**	(60 min)

Session 5: beta-thalassemia Discussion and Voting

2:00 p.m.	Questions to the Committee/Committee Discussion/Voting/Member Remarks	(115 min)
3:55 p.m.	Closing Remarks	**Peter Marks, M.D., Ph.D.** (5 min) Director, CBER
4:00 p.m.	**ADJOURNMENT**	**Christina Vert, M.S.** Designated Federal Officer, CTGTAC DSAC, CBER, FDA