FOOD AND DRUG ADMINISTRATION (FDA)
Center for Biologics Evaluation and Research (CBER)
Cellular, Tissue, and Gene Therapies Advisory Committee (CTGTAC)
June 9, 2022
FINAL AGENDA

TOPIC SUMMARY

June 9, 2022: Topic I:

The committee will meet in open session to discuss the biologics license application (BLA) BLA 125755 from bluebird bio, Inc. for elivaldogene autotemcel [autologous CD34+ stem cells genetically modified with a lentiviral vector to contain an adenosine triphosphate binding cassette, sub-family D, member 1(ABCD1) gene which encodes a functional adrenoleukodystrophy protein (ALDP)]. The applicant has requested an indication for the treatment of patients less than 18 years of age with early cerebral adrenoleukodystrophy (CALD) who do not have an available and willing HLA-matched sibling hematopoietic stem cell (HSC) donor

DAY 1: JUNE 9

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 (OTAT), CBER

Session 1: Early CALD Efficacy and Safety

10:25 a.m.	**Applicant Presentations**	**bluebird bio, Inc.** (50 min)
	Introduction	**Anne-Virginie Eggimann, M.Sc.** Chief Regulatory Officer, bluebird bio, Inc.
	Cerebral Adrenoleukodystrophy	**Florian Eichler, MD** Director, Leukodystrophy Service, Massachusetts General Hospital, Associate Professor of Neurology, Harvard Medical School
	Efficacy	**Jakob Sieker, MD** Senior Medical Director, Clinical Research and Development, bluebird bio, Inc.

	Safety and Benefit/Risk	**Laura Demopoulos, MD** Vice President, Pharmacovigilance, bluebird bio, Inc.
	Clinical Perspective: The Role of eli-cel	**Christine Duncan, MD** Sr. Physician, Dana-Farber/Boston Children's Hospital Cancer and Blood Disorders Center Medical Director of Clinical Research & Development, Gene Therapy, Boston Children's Hospital Associate Professor of Pediatrics, Harvard Medical School
11:15 a.m.	**FDA Presentation** Elivaldogene Autotemcel (Eli-cel): BLA 125755 Clinical Considerations for Efficacy and Specific Safety in Early Cerebral Adrenoleukodrystrophy	**Shelby Elenburg, M.D. and Leah Crisafi, M.D., FASA, CDR, USPHS** (50 min) OTAT Clinical Reviewers Division of Clinical Evaluation and Pharmacology/Toxicology (DCEPT) OTAT, CBER
12:05 p.m.	Clarifying Questions to Presenters	(30 min)
12:35 p.m.	**LUNCH BREAK**	(25 min)
1:00 p.m.	**OPEN PUBLIC HEARING**	(60 min)

Session 2: Safety, including vector integration

2:00 p.m.	**Invited Speaker Presentation** Integration of HIV Proviruses in Oncogenes Can Cause Clonal Expansion of T Cells and Contribute to the Development of T Cell Lymphomas	**Stephen Hughes, Ph.D.** (25 min) National Cancer Institute, National Institutes of Health
2:25 p.m.	**Applicant Presentation**	**bluebird bio, Inc.** (30 min)
	Introduction	**Anne-Virginie Eggimann, M.Sc.** Chief Regulatory Officer, bluebird bio, Inc.
	Lentiviral Vector Safety (relevant to both eli-cel and beti-cel)	**Melissa Bonner, PhD** Senior Vice President, Head of Research, bluebird bio, Inc.

2:55 p.m.	**BREAK**	(10 min)
3:05 p.m.	**FDA Presentation** Risk of Insertional Oncogenesis with Eli-cel, Lovo-cel, and Beti-cel	**Leah Crisafi, M.D., FASA, CDR, USPHS** (30 min) OTAT Clinical Reviewer Division of Clinical Evaluation and Pharmacology/Toxicology (DCEPT) OTAT, CBER
3:35 p.m.	Clarifying Questions to Presenters	(30 min)

Session 3: Early CALD Discussion and Voting

4:05 p.m.	Questions to the Committee/Committee Discussion/Voting/Member Remarks	(110 min)
5:55 p.m.	Closing Remarks	**Wilson Bryan, M.D.** (5 min) Director, OTAT, CBER
6:00 p.m.	**ADJOURNMENT**	**Christina Vert, M.S.** Designated Federal Officer, CTGTAC DSAC, CBER, FDA