2022 Current Fiscal Year Report: Cellular Tissue and Gene Therapies Advisory Committee

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1. Department or Agency	2. Fiscal Year
Department of Health and Human Services	2022

3. Committee or Subcommittee	3b. GSA Committee No.
Cellular Tissue and Gene Therapies Advisory Committee	127

4. Is this New During Fiscal Year?	5. Current Charter	6. Expected Renewal Date	7. Expected Term Date
No	10/28/2022	10/28/2024	

8a. Was Terminated During FiscalYear?	8b. Specific Termination Authority	8c. Actual Term Date
No		

9. Agency Recommendation for Next FiscalYear	10a. Legislation Req to Terminate?	10b. Legislation Pending?
Continue	Not Applicable	Not Applicable

11. Establishment Authority Authorized by Law

12. Specific Establishment Authority	13. Effective Date	14. Commitee Type	14c. Presidential?
21 U.S.C. 394	11/28/1990	Continuing	No

15. Description of Committee Scientific Technical Program Advisory Board

16a. Total Number of Reports No Reports for this FiscalYear

17a. Open	17b. Closed	17c. Partially Closed	Other Activities	17d. Total
2	0	1	0	3

Meetings and Dates

Purpose	Start	End

The Committee met by web conference in open session to hear an overview of the research programs of the Gene Transfer and Immunogenicity Branch (GTIB), Division of Cellular and Gene Therapies (DCGT), Office of Tissues and Advanced Therapies (OTAT), Center for Biologics Evaluation and Research (CBER).	03/10/2022 - 03/10/2022
The committee met to discuss and make recommendations on two biologics license applications (BLAs) from bluebird bio, Inc. On June 9, 2022 the topic was the BLA 125755 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)) for patients less than 18 years of age with early cerebral adrenoleukodystrophy who do not have an available and willing HLA-matched sibling hematopoietic stem cell (HSC) donor. On June 10, 2022 the topic was BLA 125717 for betibeglogene autotemcel (autologous CD34+ stem cells genetically modified with a lentiviral vector to contain a gene encoding functional beta-globin) for patients with -thalassemia who require regular red blood cell transfusions.	06/09/2022 - 06/10/2022
The Committee met by web conference in open session to discuss regulatory expectations for xenotransplantation products. The discussion topics include human cells that have had ex vivo contact with animal cells, and animal organs and cells for transplantation into human subjects, both of which are xenotransplantation products.	06/29/2022 - 06/30/2022

Number of Committee Meetings Listed: 3

	Current FY	Next FY
18a(1). Personnel Pmts to Non-Federal Members	$22,520.00	$32,984.00
18a(2). Personnel Pmts to Federal Members	$2,815.00	$8,245.00
18a(3). Personnel Pmts to Federal Staff	$314,749.00	$292,290.00

18a(4). Personnel Pmts to Non-Member Consultants	$28,832.00	$35,340.00
18b(1). Travel and Per Diem to Non-Federal Members	$0.00	$0.00
18b(2). Travel and Per Diem to Federal Members	$0.00	$0.00
18b(3). Travel and Per Diem to Federal Staff	$0.00	$0.00
18b(4). Travel and Per Diem to Non-member Consultants	$0.00	$0.00
18c. Other(rents,user charges, graphics, printing, mail, etc.)	$101,457.00	$96,075.00
18d. Total	$470,373.00	$464,934.00
19. Federal Staff Support Years (FTE)	2.00	1.75

20a. How does the Committee accomplish its purpose?

The Committee reviews and evaluates available data relating to the safety, effectiveness, and appropriate use of human cells, human tissues, gene transfer therapies and xenotransplantation products which are intended for transplantation, implantation, infusion and transfer in the prevention and treatment of a broad spectrum of human diseases and in the reconstruction, repair or replacement of tissues for various conditions. On Dec 9-10, 2021, a web conference was held for the Gene Transfer and Immunogenicity Branch (GTIB) Site Visit Meeting to evaluate their research program and to make recommendations to the committee. On March 10, 2022, the Committee met by web conference in open session to hear an overview of the research

programs of the Gene Transfer and Immunogenicity Branch (GTIB), Division of Cellular and Gene Therapies (DCGT), Office of Tissues and Advanced Therapies (OTAT), Center for Biologics Evaluation and Research (CBER). After the GTIB open session, the meeting was closed to the public to make recommendations to the Agency on specific research programs. The recommendations of the committee were utilized by FDA as part of its independent intramural program review of research priorities. On June 9, 2022, the Committee met by web conference in open session to discuss and make recommendations on biologics license application BLA 125755 from bluebird bio, Inc. for elivaldogene autotemcel to treat patients younger than 18 years of age with early cerebral adrenoleukodystrophy. The FDA considered the recommendations of CTGTAC in its review of BLA 125755, including recommendations on post-licensure studies and granted accelerated approval for Skysona (elivaldogene autotemcel). On June 10, 2022, the Committee met by web conference in open session to discuss biologics license applications BLA 125717 from bluebird bio, Inc. for betibeglogene autotemcel for the treatment of patients with -thalassemia who require regular red blood cell transfusions. The FDA considered the recommendations of CTGTAC in its review of BLA 125717, including recommendations on post-licensure studies and granted approval for Zynteglo (betibeglogene autotemcel). On June 29-30, 2022, the Committee met by web conference in open session to discuss regulatory expectations for xenotransplantation products. The discussion topics include human cells that have had ex vivo contact with animal cells, and animal organs and cells for transplantation into human subjects, both of which

are xenotransplantation products. The FDA will consider the recommendations of CTGTAC in evaluating xenotransplantation products.

20b. How does the Committee balance its membership?

Members have clinical or preclinical and product experience in the fields of cellular therapies, tissue transplantation, gene transfer therapies, and xenotransplantation including biostatistics, bioethics, hematology/oncology, human tissues and transplantation, reproductive medicine, general medicine and various medical specialties including surgery and oncology, immunology, virology, molecular biology, cell biology, developmental biology, tumor biology, biochemistry, rDNA technology, nuclear medicine, gene therapy, infectious diseases, and cellular kinetics. One member is technically qualified and identified with consumer interests and one non-voting member represents the point of view of industry.

20c. How frequent and relevant are the Committee Meetings?

The committee held 3 advisory committee meetings on March 10, 2022, June 9-10, 2022, and June 29-30, 2022, and 1 site visit on Dec 9-10, 2021, in FY 2022. It’s anticipated the committee may have 3 advisory committee meetings and 1 site visit meeting in FY23.

20d. Why can't the advice or information this committee provides be obtained elsewhere?

Members of the Committee are drawn from academia, research, and/or clinical practice. Their advice and input lends credibility to regulatory decisions. The alternate means of obtaining this advice would involve the recruitment of large

numbers of scientists on a full-time basis at maximum rates of compensation.

20e. Why is it necessary to close and/or partially closed committee meetings?

The committee held one closed sessions in FY2022.

21. Remarks

The Agency is in the process of considering the recommendations of the Committee from each meeting held in 2022 (March through June) to implement the necessary regulatory actions

Designated Federal Officer

Christina Vert Designated Federal Officer

Committee Members	Start	End	Occupation	Member Designation
Ahsan, Tabassum	05/17/2021	03/31/2025	Bioengineering, Therapeutic Discovery at MD Anderson Cancer Center, Houston, TX	Special Government Employee (SGE) Member
Anspach, Sylvia	06/09/2022	06/09/2022	Patient Representative for Childhood cerebral adrenoleukodystrophy	Special Government Employee (SGE) Member
Auchincloss, Hugh	06/29/2022	06/30/2022	Deputy Director, National Institute of Allergy and Infectious Diseases, Bethesda, MD	Regular Government Employee (RGE) Member
Basavaraju, Sridhar	06/29/2022	06/30/2022	Director, Office of Blood, Organ, & Other Tissue Safety, Division of Healthcare Quality Promotion, National Center for Emerging & Zoonotic Infectious Diseases, Centers for Disease Control & Prevention, Atlanta, GA	Regular Government Employee (RGE) Member
Berns, Kenneth	09/27/2018	03/31/2022	Distinguished Professor Emeritus, University of Florida	Special Government Employee (SGE) Member

Bloom, Marshall	04/01/2022	03/31/2026	Chief, Biology of Vector-borne Viruses Section, Laboratory of Virology, Rocky Mountain Laboratories, National Institute of Allergy and Infectious Diseases, Hamilton, MT	Regular Government Employee (RGE) Member
Breuer, Christopher	06/07/2018	03/31/2026	Co-Director, Nationwide Children's Hospital, Columbus, OH	Special Government Employee (SGE) Member
Butterfield, Lisa	11/28/2016	03/31/2023	Professor, Surgery and Immunology, U. Pittsburgh, Pittsburgh, PA	Special Government Employee (SGE) Member
Coffin, John	06/09/2022	06/09/2022	Professor, Tufts University, Boston, MA	Special Government Employee (SGE) Member
Conway, Paul	06/29/2022	06/30/2022	Patient Representative	Special Government Employee (SGE) Member
Cooper, Matthew	06/29/2022	06/30/2022	Director, Kidney and Pancreas Transplantation Medical Director, Transplant QAPI Medstar Georgetown Transplant Institute Professor of Surgery Georgetown University School of Medicine Washington, DC	Special Government Employee (SGE) Member
Crombez, Eric	06/09/2022	06/09/2022	Alternate Industry Representative, Chief Medical Officer, Ultragenyx Gene Therapy, Cambridge, MA	Representative Member
DiPersion, John	06/09/2022	06/09/2022	Deputy Director, Siteman Cancer Center, Washington University School of Medicine, St. Louis, MO	Special Government Employee (SGE) Member
Dueck, AmyLou	06/09/2022	06/09/2022	Consultant and Associate Professor of Biostatistics, Mayo Clinic, Scottsdale, AZ	Special Government Employee (SGE) Member

Fishman, Jay	06/29/2022	06/30/2022	Associate Director, MGH Transplant Center Director, Transplant Infectious Disease & Compromised Host Program Massachusetts General Hospital Professor of Medicine, Harvard Medical School Boston, MA	Special Government Employee (SGE) Member
Fox, Bernard	01/27/2020	03/31/2023	Providence Portland Medical Center Portland, OR	Special Government Employee (SGE) Member
Godley, Lucy	04/02/2022	07/10/2022	Professor, University of Chicago, Chicago, IL	Special Government Employee (SGE) Member
Gordeuk, Victor	06/09/2022	06/09/2022	Professor of Medicine, University of Illinois, Chicago, IL	Special Government Employee (SGE) Member
Hawkins, Randy	04/01/2017	03/31/2022	Consumer Representative, Private Practice,Inglewood, CA	Special Government Employee (SGE) Member
Keller, Stephanie	06/09/2022	06/09/2022	Associate Professor, Emory University and Children's Healthcare, Atlanta, GA	Special Government Employee (SGE) Member
Kimmel, Paul	06/29/2022	06/30/2022	Senior Advisor, National Institute of Diabetes and Digestive and Kidney Diseases, Bethesda, MD	Regular Government Employee (RGE) Member
Kohn, Donald	08/23/2022	03/31/2025	Director, UCLA Human Gene and Cell Therapy Program UCLA Broad Stem Cell Research Center, University of California, LA	Special Government Employee (SGE) Member
Lee, Jeannette	05/30/2019	03/31/2023	Professor of Biostatistics, University of Arkansas for Medical Science, Little Rock, AR	Special Government Employee (SGE) Member

Maciejewski, Jaroslaw	06/09/2022	06/09/2022	Professor of Medicine, Cleveland Clinic, Cleveland, OH	Special Government Employee (SGE) Member
Maragh, Samantha	06/29/2022	06/30/2022	Leader, Genome Editing Program National Institute of Standards & Technology Gaithersburg, MD	Regular Government Employee (RGE) Member
Morrison, Sean	06/07/2018	03/31/2026	Director, Children's Research Institute, University of Texas Southwestern Medical Center, Dallas,TX	Special Government Employee (SGE) Member
Nichol, Geoffrey	12/26/2019	04/21/2022	Industry Representative, Senior Vice President, Global Clinical Research and Chief Medical Officer, BioMarin Pharmaceutical, Novato, CA	Representative Member
O'Sullivan-Fortin, Kathleen	08/23/2022	03/31/2025	Consumer Representative, ALD Connect, Inc., Middleton, MA	Special Government Employee (SGE) Member
Ott, Melanie	04/01/2022	03/31/2026	Director, Gladstone Institute of Virology, Department of Medicine, University of California San Francisco, School of Medicine, San Francisco, CA	Special Government Employee (SGE) Member
Palevsky, Paul	06/29/2022	06/30/2022	Professor, University of Pittsburgh and Chief, Kidney Medicine Section, VA Pittsburgh Healthcare System, Pittsburgh, PA	Regular Government Employee (RGE) Member
Roberts, Donna	06/09/2022	06/09/2022	Professor, Medical University of South Carolina, Charleston, SC	Special Government Employee (SGE) Member
Shah, Nirali	02/04/2022	03/01/2026	Head, Hematologic Malignancies Section Pediatric Oncology Branch National Cancer Institute, Bethesda MD	Regular Government Employee (RGE) Member

Shapero, Steven	06/09/2022	06/09/2022	Patient Representative Childhood cerebral adrenoleukodystrophy	Special Government Employee (SGE) Member
Singh, Navdeep	06/09/2022	06/09/2022	Patient Representative beta thalassemia	Special Government Employee (SGE) Member
Trieu, Janelle	06/09/2022	06/09/2022	Patient Representative beta thalassemia	Special Government Employee (SGE) Member
Walters, Mark	09/27/2018	03/31/2022	Jordan Family Director, USCF Benioff Children's Hospital Oakland, Oakland, CA	Special Government Employee (SGE) Member
Wolfe, Gil	02/04/2022	03/01/2026	Professor and Chairman, Department of Neurology, University of Buffalo, State University of New York, Buffalo, NY	Special Government Employee (SGE) Member
Wu, Joseph	11/28/2016	03/31/2023	Director, Stanford Cardiovascular Inst. and Professor of Medicine and Radilogy, Stanford University, Stanford, CA	Special Government Employee (SGE) Member
Zaia, John	06/07/2018	03/31/2022	Director, Center for Gene Therapy, Beckman Research Institute of City of Hope, Duarte, CA	Special Government Employee (SGE) Member
Zeiss, Caroline	06/29/2022	06/30/2022	Professor of Comparative Medicine Chief of Pathology Comparative Medicine Professor, Ophthalmology and Visual Science, Yale University School of Medicine, New Haven, CT	Special Government Employee (SGE) Member

Number of Committee Members Listed: 40

Narrative Description

FDA's strategic priorities in responding to the public health challenges of the 21st century are to advance regulatory science

and innovation; strengthen the safety and integrity of the global supply chain; strengthen compliance and enforcement activities to support public health; expand efforts to meet the needs of special populations; advance medical countermeasures and emergency preparedness; advance food safety and nutrition; promote public health by advancing the safety and effectiveness of medical products; establish an effective tobacco regulation, prevention, and control program; and manage for organizational excellence and accountability. The Cellular, Tissue, and Gene Therapies Advisory Committee supports FDA's mission and strategic action by reviewing and evaluating available data relating to the safety and effective use of cellular therapies, tissue transplantation, gene transfer therapies and xenotransplantation, which are intended for the use in the prevention and treatment of a broad spectrum of human diseases. The Committee also considers the quality and relevance of FDA's research program, which provides scientific support for the regulation of these products. The Committee supports FDA's mission by using science-based risk management in all of its activities. The Committee recommendations provide the most health promotion and protection at the least cost for the public. This Committee assists the Agency in ensuring timely, high quality, cost-effective processes for review of new technologies/pre-market submissions, effective communication and working relationships with stakeholders to enhance U.S. and global health outcomes, accurately analyzing risks associated with medical products, facilitating the development and availability of medical countermeasures to limit the effects of a terrorist attack on the civilian and military populations, protecting the safety and security of biologics (gene therapy, human tissues, and cellular therapies) all key components of FDA's strategic plan objectives.

What are the most significant program outcomes associated with this committee?

	Checked if Applies
Improvements to health or safety	✓
Trust in government	✓
Major policy changes	✓
Advance in scientific research	✓

Effective grant making	☐
Improved service delivery	☐
Increased customer satisfaction	☑
Implementation of laws or regulatory requirements	☑
Other	☐

Outcome Comments

NA

What are the cost savings associated with this committee?

	Checked if Applies
None	☐
Unable to Determine	☑
Under $100,000	☐
$100,000 - $500,000	☐
$500,001 - $1,000,000	☐
$1,000,001 - $5,000,000	☐
$5,000,001 - $10,000,000	☐
Over $10,000,000	☐
Cost Savings Other	☐

Cost Savings Comments

The utilization of the Cellular, Tissue and Gene Therapies Advisory Committee enables the Agency to obtain required and frequently scarce professional services from medical and scientific experts not otherwise available to the Agency; and to obtain the services of these experts only on an as needed basis rather than on a full time basis. The services of the Committee resulted in advice for the improvement of the public health, for which it is difficult to assign a financial value.

What is the approximate Number of recommendations produced by this committee for the life of the committee?

97

Number of Recommendations Comments

The Committee made 97 recommendations from FY2003 through FY2022.

What is the approximate Percentage of these recommendations that have been or

will be Fully implemented by the agency?
84%

% of Recommendations Fully Implemented Comments
The function of an advisory committee is purely advisory in nature. Although the FDA most often accepts the recommendations from its committees, the advice is purely advisory in nature, and therefore, the Agency has the option of not implementing the advice.

What is the approximate Percentage of these recommendations that have been or will be Partially implemented by the agency?
10%

% of Recommendations Partially Implemented Comments
The function of an advisory committee is purely advisory in nature. Although the FDA most often accepts the recommendations from its committees, the advice is purely advisory in nature, and therefore, the Agency has the option of not implementing the advice.

Does the agency provide the committee with feedback regarding actions taken to implement recommendations or advice offered?
Yes ☑ No ☐ Not Applicable ☐

Agency Feedback Comments
The Agency usually does. Product approval issues are first released to the sponsor. When appropriate, information is made available to the public. Actions related to guidance documents or other general matters issues are available publicly when implemented.

What other actions has the agency taken as a result of the committee's advice or recommendation?

	Checked if Applies
Reorganized Priorities	☑
Reallocated resources	☐
Issued new regulation	☑
Proposed legislation	☐
Approved grants or other payments	☐
Other	☑

Action Comments

FDA approves or chooses not to approve an investigational medical product.

Is the Committee engaged in the review of applications for grants?

No

Grant Review Comments

NA

How is access provided to the information for the Committee's documentation?

	Checked if Applies
Contact DFO	✓
Online Agency Web Site	✓
Online Committee Web Site	✓
Online GSA FACA Web Site	✓
Publications	✓
Other	

Access Comments

N/A