2024 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	2024

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
0	0	0	0	0

Meetings and Dates

No Meetings

	Current FY	Next FY
18a(1). Personnel Pmts to Non-Federal Members	$0.00	$0.00
18a(2). Personnel Pmts to Federal Members	$0.00	$0.00
18a(3). Personnel Pmts to Federal Staff	$0.00	$0.00
18a(4). Personnel Pmts to Non-Member Consultants	$0.00	$0.00
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.)	$0.00	$0.00
18d. Total	$0.00	$0.00
19. Federal Staff Support Years (FTE)	0.00	0.00

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. 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 May 12, 2023, the Committee met by web conference in open session to discuss biologics license application (BLA) 125781 from Sarepta Therapeutics, Inc. for delandistrogene moxeparvovec (SRP-9001) for the treatment of ambulatory patients with Duchenne Muscular Dystrophy (DMD) with a confirmed mutation in the DMD gene. The FDA considered the recommendations of CTGTAC in its review of BLA 125781 and granted accelerate approval of Elevidys (delandistrogene moxeparvovec) for the treatment of pediatric patients 4 through 5 years of age with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the DMD gene who do not have a pre-existing medical reason preventing treatment with this therapy. On September 27, 2023, the Committee met in open session to discuss biologics license application (BLA) 125782 from BrainStorm Cell Therapeutics, Inc. for debamestrocel (autologous bone marrow-derived mesenchymal stromal cells induced to secrete neurotrophic factors) for the treatment of amyotrophic lateral sclerosis (ALS). The FDA is considering the recommendations of CTGTAC in its review of BLA 125782.

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 2 advisory committee meetings, on May 12, 2023 and September 27, 2023, and no site visits, in FY 2023. It is anticipated the committee may have 4 advisory committee meetings and no site visit meetings in FY 2024.

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 no closed meetings in FY 2023.

21. Remarks

The Agency considered the recommendations of the Committee from the May 12, 2023 CTGTAC

meeting, and is in the process of considering the recommendations of the Committee from the September 27, 2023 meeting to implement the necessary regulatory actions. The Agency is in the process of approving the minutes for the September 27, 2023 meeting, so they are not available at this time, they will be posted as soon as possible.

Designated Federal Officer

Marie DeGregorio Designated Federal Officer

Committee Members	Start	End	Occupation	Member Designation
Ahsan, Tabassum	05/17/2021	03/31/2024	Vice President, Cell Therapy Operations, City of Hope, Duarte, CA	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	Director of Tissue Engineering Program and Surgical Research Director, Center for Regenerative Medicine, Nationwide Children's Hospital, Columbus, OH	Special Government Employee (SGE) Member
Crombez, Eric	09/19/2022	03/31/2024	Industry Representative - Chief Medical Officer, Ultragenyx Gene Therapy, Cambridge, MA	Representative Member

Kohn, Donald	08/23/2022	03/31/2025	Distinguished Professor Departments of Microbiology, Immunology, and Molecular Genetics; Pediatrics; Molecular and Medical Pharmacology; David Geffen School of Medicine at UCLA University of California, Los Angeles, Los Angeles, CA	Special Government Employee (SGE) Member
London, Wendy	04/01/2023	03/31/2027	Associate Professor of Pediatrics, Boston Children's Hospital/Dana-Farber Cancer Institute, Harvard Medical School	Special Government Employee (SGE) Member
Morrison, Sean	06/07/2018	03/31/2026	Director, Children's Medical Research Institute, University of Texas Southwestern Medical Center, Dallas,TX	Special Government Employee (SGE) Member
O'Sullivan-Fortin, Kathleen	08/23/2022	03/31/2025	Consumer Representative, Founder, 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
Shah, Nirali	02/04/2022	03/31/2026	Head, Hematologic Malignancies Section, Pediatric Oncology Branch, National Cancer Institute, Bethesda, MD	Regular Government Employee (RGE) Member

Wolfe, Gil	02/04/2022	03/31/2026	Professor and Chairman of Neurology, Irvin and Rosemary Smith Chair, University at Buffalo Distinguished Professor, Department of Neurology, Jacobs School of Medicine and Biomedical Sciences, University at Buffalo/SUNY, Buffalo, NY	Special Government Employee (SGE) Member
Wu, Joseph	11/28/2016	03/31/2025	Director, Stanford Cardiovascular Inst. and Professor of Medicine and Radilogy, Stanford University, Stanford, CA	Special Government Employee (SGE) Member

Number of Committee Members Listed: 12

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 tabacco 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?
99

Number of Recommendations Comments
The Committee made 99 recommendations from FY 2003 through FY 2023.

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