2018 Current Fiscal Year Report: Endocrinologic and Metabolic Drugs Advisory Committee

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1. Department or Agency	2. Fiscal Year
Department of Health and Human Services	2018

3. Committee or Subcommittee	3b. GSA Committee No.
Endocrinologic and Metabolic Drugs Advisory Committee	855

4. Is this New During Fiscal Year?	5. Current Charter	6. Expected Renewal Date	7. Expected Term Date
No	08/27/2018	08/27/2020	

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 2 **17b. Closed** 0 **17c. Partially Closed** 0 **Other Activities** 0 **17d. Total** 2

Meetings and Dates

Purpose	Start	End
The committee discussed the safety and efficacy of new drug application (NDA) 209637 for semaglutide injection, submitted by Novo Nordisk, as an adjunct to diet and exercise to improve glycemic control in adults with type 2 diabetes mellitus	10/18/2017	10/18/2017
The committee discussed the safety and efficacy of new drug application (NDA) 210645, for volanesorsen solution for subcutaneous injection, submitted by Akcea Therapeutics, Inc. The proposed indication is as an adjunct to diet for the treatment of patients with familial chylomicronemia syndrome. Majority of the committee voted “Yes”, agreeing based on the information included in the briefing materials and presented, the applicant provided sufficient efficacy and safety data to support approval of volanesorsen. The committee members who voted “Yes” noted that the study met its primary endpoint of decreasing TG. Eight of the voting committee members voted “No”. Majority of those committee members who voted "No", voiced concerns with the data presented by the applicant and agreed that the data did not demonstrate a favorable risk/benefit ratio.	05/10/2018	05/10/2018

Number of Committee Meetings Listed: 2

	Current FY	Next FY
18a(1). Personnel Pmts to Non-Federal Members	$6,810.00	$16,405.00
18a(2). Personnel Pmts to Federal Members	$0.00	$3,281.00

18a(3). Personnel Pmts to Federal Staff	$158,202.00	$153,545.00
18a(4). Personnel Pmts to Non-Member Consultants	$8,905.00	$16,405.00
18b(1). Travel and Per Diem to Non-Federal Members	$10,605.00	$22,826.00
18b(2). Travel and Per Diem to Federal Members	$1,910.00	$2,876.00
18b(3). Travel and Per Diem to Federal Staff	$0.00	$0.00
18b(4). Travel and Per Diem to Non-member Consultants	$12,133.00	$15,908.00
18c. Other(rents,user charges, graphics, printing, mail, etc.)	$53,473.00	$59,482.00
18d. Total	$252,038.00	$290,728.00
19. Federal Staff Support Years (FTE)	1.10	1.10

20a. How does the Committee accomplish its purpose?

The Committee reviews and evaluates data concerning the safety and effectiveness of marketed and investigational human drug products for use in the treatment of endocrine and metabolic disorders, and makes appropriate recommendations to the Commissioner of Food and Drugs.

20b. How does the Committee balance its membership?

Members are experts in internal medicine, endocrinology, pediatric endocrinology, metabolism, diabetes, bone and mineral disorders, adrenal and gonadal conditions, geriatrics, growth disorders, clinical pharmacology, statistics, obesity and epidemiology. The committee includes one technically qualified voting member who is identified with consumer interests. The Committee has one non-voting member identified with industry interests.

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

The committee led two meetings during the FY-18.On October 18, 2017, the committee discussed the safety and efficacy of new drug application (NDA) 209637 for semaglutide injection, submitted by Novo Nordisk, as an adjunct to diet and exercise toimprove glycemic control in adults with type 2 diabetes mellitus. The committee members overwhelmingly voted “Yes”, agreeing thatthe available efficacy and safety data support approval of semaglutide 0.5 mg and 1 mg, administered subcutaneously once weekly, as an adjunct to diet and exercise to improve glycemic control in adults with type 2 diabetes mellitus. The committee agreed that theprimary endpoint was met, and benefits of semaglutide included better glycemic control and favorable findings for weight. Agency Action: The Agency is still reviewing all recommendations discussed during the meeting.On May 10, 2018, the committee discussed the safety and efficacy of new drug application (NDA) 210645, for volanesoren solution for subcutaneous injection, submitted by Akcea Therapeutics, Inc. The proposed indication is as an adjunct to diet for the treatment of patients with familial chylomicronemia syndrome. Twelve of the 20 voting

members voted “Yes”, agreeing that based on the information included in the briefing materials and presented, the applicant provided sufficient efficacy and safety data to support approval of volanesorsen. Thecommittee members who voted “Yes” noted that the study met its primary endpoint of decreasing TG. Agency Action: The Agency is still reviewing all recommendations that were made at the meeting. It is expected that this committee will meet four times in FY-19.

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

Members of the committee are from academia and clinical practice. This broadens the base of experience from which final decisions are made. The advice leads to strong regulatory discussions, which are able to withstand intense public scrutiny.

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

There were no closed meetings to reporting during FY-18.

21. Remarks

No reports are required for this committee.

Designated Federal Officer

LaToya Ann Bonner DFO

Committee Members	Start	End	Occupation	Member Designation
Blaha, Michael	07/01/2016	06/30/2020	Assistant Professor, Cardiology and Epidemiology, Department of Medicine and Epidemilogy, Johns Hopkins University, Baltimore, MD	Special Government Employee (SGE) Member
Budnitz, Daniel	07/01/2015	06/30/2019	Director, CDC Medication Safety Program	Regular Government Employee (RGE) Member
Burman, Kenneth	07/01/2016	06/30/2020	Chief, Endocrine Section, MedStar Health, Washington, DC	Special Government Employee (SGE) Member
Ellenberg, Susan	07/01/2018	06/30/2022	Professor of Medical Ethics and Health Policy, University of Pennsylvania	Special Government Employee (SGE) Member
Everett, Brendan	10/30/2014	06/30/2018	Assistant Professor of Medicine, Harvard Medical School; Director of General Cardiology Inpatient Service, Brigham and Women's Hospital	Special Government Employee (SGE) Member
Heckbert, Susan	10/30/2014	06/30/2018	Professor, Department of Epidemiology, University of Washington	Special Government Employee (SGE) Member

Kewalramani, Reshma	02/29/2016	10/31/2019	Vice President, US Med Organ, Amgen	Representative Member
Konstam, Marvin	07/01/2018	06/30/2022	Chief Physician Executive, The Cardiovascular Center, Tufts University School of Medicine	Special Government Employee (SGE) Member
Low Wang, Cecilia	07/01/2016	06/30/2020	Associate Professor of Medicine, University of Colorado, Anschutz Medical Campus	Special Government Employee (SGE) Member
McCollister-Slipp, Anna	06/19/2018	06/30/2022	Consumer Representative; Founder, VitalCrowd	Special Government Employee (SGE) Member
Neaton, James	10/30/2014	06/30/2018	Professor of Biostatistics, Coordinating Centers of Biometric Research, Univ. Minnesota, School of Public Health	Special Government Employee (SGE) Member
Weber, Thomas	07/01/2016	06/30/2020	Associate Professorof Medicine, Duke University Medical Center, Durham, NC	Special Government Employee (SGE) Member
Wilson, Peter	07/01/2014	06/30/2018	Director, Epidemiology and Genomic Medicine, Atlanta Veterans Administration Medical Center; Professor of Medicine and Public Health, Emory University, Emory Clinical Cardiovascular Research Institute	Regular Government Employee (RGE) Member
Yanovski, Susan	12/22/2015	06/30/2019	Co-director, Office of Obesity Research, Natinal Institute of Diabetes and Digestive and Kidney Diseases, NIH, Bethesda, MD	Regular Government Employee (RGE) Member
de Lemos, James	07/01/2018	06/30/2022	Cardiology/Professor of Medicine UT Southwestern Medical Center	Special Government Employee (SGE) Member

Number of Committee Members Listed: 15

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 Endocrinologic and Metabolic Drugs Advisory Committee supports FDA's strategic priorities by reviewing and evaluating available data concerning the safety and effectiveness of marketed and investigational human drug products for use in the treatment of endocrinologic and metabolic diseases and makes appropriate recommendations to the Commissioner of the Food and Drug

Administration. This supports the development of safe and effective new medical technologies, and advances the status of the Agency as a science-based and science-led regulatory agency, providing global leadership in the protection of public health.

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

N/A

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 Endocrinologic and Metabolic Drugs 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 bases rather than on a full time basis. The service 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?
47

Number of Recommendations Comments
The committee made 47 recommendations from FY-03 through FY-18 - see question 20a of the annual report for specific accomplishments.

What is the approximate Percentage of these recommendations that have been or will be Fully implemented by the agency?
79%

% 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?
9%

% 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
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 new medical products.

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

No

Grant Review Comments

N/A

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