2024 Current Fiscal Year Report: Peripheral and Central Nervous System Drugs 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.
Peripheral and Central Nervous System Drugs Advisory Committee	990

4. Is this New During Fiscal Year?	5. Current Charter	6. Expected Renewal Date	7. Expected Term Date
No	06/04/2022	06/04/2024	

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 0 **17b. Closed** 0 **17c. Partially Closed** 0 **Other Activities** 0 **17d. Total** 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 data on the safety and effectiveness of marketed and investigational human drugs for use in the treatment of neurological diseases and makes appropriate recommendations to the Commissioner of the Food and Drugs.

20b. How does the Committee balance its membership?

Members are experts in the fields of neurology, neuropharmacology, neuropathology, otolaryngology, epidemiology, statistics or related

specialties. The Committee has one technically qualified member identified with consumer interests. In addition to the voting members, the Committee includes one non-voting member who is identified with industry interests.

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

In FY-23, the Committee held three meetings. At one of these meetings, the Committee met in joint session with other Committees but was not the lead Committee. See the Agency Recommendations, Remarks section for a list of joint meetings in which the Committee was not the lead Committee. On March 22, 2023, the Peripheral and Central Nervous System Drugs Advisory Committee met to discuss new drug application (NDA) 215887, for tofersen (BIIB067) intrathecal injection, submitted by Biogen Inc., for the treatment of amyotrophic lateral sclerosis (ALS) associated with a mutation in the superoxide dismutase 1 (SOD1) gene. The issues the Committee members were asked to discuss included whether neurofilament light chain (NfL) reduction is reasonably likely to predict clinical benefit, the strengths and limitations of the available clinical data regarding the effectiveness of tofersen for SOD1-ALS, and the overall benefit-risk assessment of this drug. The Committee voted unanimously (9 to 0) that a reduction in NFL concentration with tofersen is reasonably likely to predict clinical benefit in the treatment of patients with SOD1-ALS. However, the Committee voted 5-3 (with one abstention), that the available clinical data are not sufficiently convincing to meet the substantial evidence of effectiveness threshold for regular approval. Agency Action: On April 25, 2023, the Agency approved approved Qalsody (tofersen), submitted

by Biogen, Inc., for the treatment of patients with amyotrophic lateral sclerosis (ALS) associated with a mutation in the superoxide dismutase 1 (SOD1) gene (SOD1-ALS). On April 14, 2023, a meeting was held jointly with the Psychopharmacologic Drugs Advisory Committee. Further information regarding this meeting is provided in the Recommendation Remarks section. On June 9, 2023, the Peripheral and Central Nervous System Drugs Advisory Committee met to discuss supplemental biologics license application (sBLA) 761269/s-001, for LEQEMBI (lecanemab) solution for intravenous infusion, submitted by Eisai, Inc., for the treatment of Alzheimer's disease, initiated in patients with mild cognitive impairment or mild dementia stage of disease. This product was approved under 21 CFR 314.500 (subpart H, accelerated approval regulations) for the treatment of Alzheimer’s disease. Confirmatory studies are studies to verify and describe the clinical benefit of a product after it receives accelerated approval. The committee will discuss the confirmatory study, BAN2401-G000-301, conducted to fulfill post-marketing requirement 4384-1 detailed in the January 6, 2023, approval letter, available at https://www.accessdata.fda.gov/drugsatfda_docs/appletter/2023/761269Orig1s000ltr.pdf. The Committee was asked to discuss whether the results from the CLARITY AD study provided evidence of clinical benefit of lecanemab for the treatment of Alzheimer’s disease (AD), as well as the overall benefit/risk assessment of lecanemeb taking into consideration Apolipoprotein E (ApoE) 4 homozygotes, patients requiring concomitant treatment with anticoagulant agents, and patients with cerebral amyloid angiopathy. The Committee voted unanimously (6 to 0) that the results of the CLARITY AD study verified the clinical benefit of lecanemab for the treatment of AD. Agency

Action: On July 6, 2023, the Agency converted Leqembi (lecanemab-irmb), indicated to treat adult patients with Alzheimer's Disease, to traditional approval following a determination that a confirmatory trial verified clinical benefit. It is expected that the Committee will meet 1-2 times during FY-24

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 Committee helps the regulatory decisions withstand intense public scrutiny. The 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 during FY-23.

21. Remarks
There were no reports required for this Committee in FY-23. In FY-23, the Committee held three meetings. At one of these meetings, the Committee met in joint session with other committees but was not the lead Committee. So that joint meetings are not counted twice in the FACA database, they will be reported under the primary or lead Committee. For the purposes of this database, the secondary Committee still reports meeting information and costs associated under this section of the report as well as the cost section. On April 14, 2023, a meeting was held jointly with the Psychopharmacologic Drugs Advisory Committee to discuss supplemental new

drug application (sNDA) 205422 s009, efficacy supplement for REXULTI (brexpiprazole) tablets, submitted by Otsuka Pharmaceutical Company, Ltd., and Lundbeck, Inc., for the proposed treatment of agitation associated with Alzheimer's dementia. The Committees were asked to discuss the overall benefit/risk assessment of bexpiprazole for the treatment of agitation associated with Alzheimer's dementia, as well as whether there is a population of patients for whom the benefit/risk of this drug appears acceptable, and for whom the benefit/risk does not appear to be favorable. The majority of the Committee members (9 to 1) agreed that the Applicant provided sufficient data to allow identification of a population in whom the benefits of treating agitation associated with Alzheimer's dementia with brexpiprazole outweigh its risks. Agency Action: On May 11, 2023, the Agency granted supplemental approval of REXULTI (brexpiprazole) oral tablets for the treatment of agitation associated with dementia due to Alzheimer's disease.

Designated Federal Officer

Jessica Seo Designated Federal Officer

Committee Members	Start	End	Occupation	Member Designation
Alexander, Robert	12/01/2021	01/31/2026	Chief Scientific Officer Alzheimer's Prevention Initiative, Banner Alzheimer's Institute; Research Professor, Department of Psychiatry University of Arizona College of Medicine – Phoenix	Special Government Employee (SGE) Member

Apostolova, Liana	12/01/2021	01/31/2026	Distinguished Professor in Neurology, Barbara and Peer Baekgaard Chair in Alzheimer's Disease Research, Indiana University School of Medicine	Special Government Employee (SGE) Member
Cudkowicz, Merit	12/01/2021	01/31/2025	Julieanne Dorne Professor of Neurology, Harvard Medical School; Chair, Department of Neurology, Director, Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital	Special Government Employee (SGE) Member
Gold, Michael	03/30/2020	10/31/2023	Chief Medical Officer, Neumora Therapeutics	Representative Member
Kryscio, Richard	04/21/2016	01/31/2024	Professor, Statistics and Biostatistics, University of Kentucky	Special Government Employee (SGE) Member
Mielke, Michelle	02/01/2020	01/31/2024	Chair, Department of Epidemiology and Prevention, Professor of Epidemiology and Gerontology and Geriatric Medicine, Wake Forest University School of Medicine, Division of Public Health Services	Special Government Employee (SGE) Member
Montine, Thomas	12/01/2021	01/31/2026	Chair, Department of Pathology, Stanford Medicine Endowed Professor, Stanford University School of Medicine	Special Government Employee (SGE) Member

Number of Committee Members Listed: 7

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 Peripheral and Central Nervous System Drugs Advisory Committee supports FDA's strategic priorities by reviewing and evaluating data concerning the safety and effectiveness of marketed and investigational human drug products for use in the treatment of neurologic diseases and makes appropriate recommendations to the Commissioner of Food and Drugs. 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 the 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 Peripheral and Central Nervous System Drugs Advisory Committee enabled 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 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?

32

Number of Recommendations Comments

The Committee made 32 recommendations from FY-03 through FY-23.

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, therefore, the Agency has the option of not implementing the advice. This number represents an approximation of the percentage of recommendations that the agency has fully implemented or plans to fully implement.

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

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