

FDA STAFF MANUAL GUIDES, VOLUME III - GENERAL ADMINISTRATION

FDA OFFICIAL COUNCILS AND COMMITTEES

FDA ORPHAN DRUG PRODUCTS POLICY COUNCIL

Effective Date: 11/20/2017

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1. PURPOSE.

As part of the Food and Drug Administration's modernization efforts related to the regulation of orphan products, the FDA establishes a policy council to address scientific and regulatory issues pertaining to drugs, biologics, and medical devices for rare disease populations (herein referred to as "Council" and "orphan products," respectively). This charter describes the duties and responsibilities of the Council, the organization of its membership, and its operating procedures.

2. POLICY

The Council will function as a forum for developing guiding principles, where necessary, to ensure a consistent approach on the regulation of orphan products, including orphan product designation and review, orphan exclusivity, and rare pediatric disease priority review vouchers (RPD PRVs). In addition, the Council will provide guidance, and, where necessary, render decisions on novel and precedent-setting product-specific RPD designation requests. The Council will also prospectively identify complex scientific and regulatory issues related to orphan products. If needed, the Council can serve as a forum to resolve disagreements among centers, the Office of Orphan Products Development (OOPD), and/or sponsors on activities and policies related to these issues. The Council does not directly meet with sponsors.

The Council will promote and coordinate communication both internally and externally on policy decisions made by the Council.

3. RESPONSIBILITIES

A. Responsibilities of the Council

1. Facilitates the development of orphan product policy to achieve a unified, timely, and consistent FDA position.
2. Coordinates timely decisions regarding novel and precedent-setting RPD designations.
3. Reports directly to the FDA Deputy Commissioner for Medical Products and Tobacco.
4. Promotes and coordinates internal and, when necessary, external communication related to orphan product policies.

B. Responsibilities of the Council members related to their organizational unit

1. Represent their organizational unit's views on issues under consideration by the Council.
2. Serve as the point of contact for communications with their organizational unit, including their senior managers, about Council deliberations and obtain their input in a timely manner.
3. Identify relevant stakeholders and their concerns to the orphan product policy issue under discussion.
4. Attend meetings regularly in person or by teleconference. If a member cannot attend a meeting, an alternate may be designated by the Council member. The members (and alternates) must be able to represent the consensus recommendation or position of their organization. If deemed appropriate by the Chairperson, email communication may be used to obtain Council input.

C. Responsibilities of the Council Chair

1. Directs the activities of the Council.
2. Facilitates discussion on issues within the Council.
3. Reviews proposed issues for the Council and determines when issues are appropriate for Council review and discussion. If a proposal is not selected for Council review by the date specified, the Chairperson (through the Executive Secretary) provides an explanation for this

decision. Reconsideration by the Council of such decisions can be requested.

D. Responsibilities of the Executive Secretary

1. Maintains the Council roster and any working groups formed (including information such as the creation date, Council-assigned responsibilities, and sunset of working groups).
2. Prepares documents and papers as requested by the Chairperson.
3. Distributes documents relevant to the activities of the Council.
4. Schedules pre-meetings as necessary and assists in preparing materials for scheduled meetings.
5. Arranges and organizes meeting logistics.
6. Notes action items generated during Council meetings and follows up on action items.
7. Maintains a repository, organized by year and including reports, summaries of meeting discussions, a log and status of issues discussed and actions assigned, and copies of the Council decisions and actions that can be accessible to all staff across Centers.
8. Confirms meeting agendas and conduct meetings.

E. Council Organization

1. The Council resides in the Office of Medical Products and Tobacco (OMPT).
2. The Council Chairperson will be the Deputy Commissioner for Medical Products and Tobacco or his/her designee.
3. The Executive Secretary is appointed by the Chairperson.
4. The Council is composed of:
 - a. Two representatives each from the medical product centers (Center for Biologics Evaluation and Research, Center for Drug Evaluation and Research, Center for Devices and Radiological Health). The two representatives will include the Center Director and a named representative.

- b. Two representatives from OSMP: OSMP and OOPD Directors
 - 1) For issues related to RPD designations, one additional representative from OSMP: OPT Director
- c. Other participants and consultants from within the agency (e.g., Center for Food Safety and Applied Nutrition to address issues related to medical foods, Office of the Chief Counsel) and from other Federal government organizations (e.g., NIH) may participate, when appropriate, in the activities of the Council at the discretion of the Chairperson. The Council will follow applicable policies and procedures if information is to be shared outside FDA.
- d. The Council may establish working groups as needed.

F. Responsibility of the Individual/Office/Center Seeking Council Evaluation:

1. A proposal for a Council meeting may be submitted by any individual involved with the regulation of orphan products.
2. The requesting individual should submit a meeting request form (provide link) to the Executive Secretary for consideration by the Chairperson. This request form includes the following information: the issue to be resolved, the trigger that raised the issue, the date by which a response is needed, and concurrence of his/her Center or Office Director.
3. Issues appropriate for Council deliberation include precedent setting regulatory decision(s), when there are disagreements within the Agency and/or with sponsors, and when one center/office would like to change an existing practice or identifies a novel policy question.
4. If the proposal is accepted, the requesting individual should provide a meeting background document (provide link) to the executive secretary for review at least two weeks before the Council meeting. The background document should provide all the necessary information to understand the issues to be discussed.

4. PROCEDURES.

- A. The Council will be scheduled to meet on a monthly basis. Meetings will be cancelled if the Chairperson concludes there are no issues ripe for Council discussion.
- B. Ad hoc meetings may be scheduled at the discretion of the Chairperson.

- C. A request for Council deliberation will not delay review deadlines and a Council decision does not replace the existing formal appeals process for sponsors.
- D. Experts from Agency staff will be sought and invited to participate in the discussion at the Chairperson’s discretion. Suggested attendees may be provided in the meeting background document.
- E. The individual/office/center seeking Council evaluation will provide a brief (e.g., 10-minute) overview of the issues at the beginning of the meeting.
- F. Decisions will be reached through consensus, after deliberation among all parties attending the Council meeting. If consensus cannot be achieved, the Chairperson has discretion to raise the matter further to reach a decision. For time-sensitive RPD designations, the Chairperson will be responsible for resolving any outstanding issues, and where necessary, will render a decision.

5. RECORDS OF COUNCIL.

- A. The Chairperson and the executive secretary will assure that the activities of the Council, including recommendations, decisions, issues, action items, and other pertinent materials attributable to the Council, are documented and communicated to senior management and relevant staff, as appropriate.
- B. The Council will review this charter at least annually based on experience gained by the Council and revise it as needed.

6. EFFECTIVE DATE.

The effective date of this charter is November 20, 2017.

7. Document History - SMG 2010.19, “FDA Orphan Drug Products Policy Council”

STATUS (I,R,C)	DATE APPROVED	LOCATION OF CHANGE HISTORY	CONTACT	APPROVING OFFICIAL
Initial	11/17/2017	N/a	FDA/OMPT	Scott Gottlieb, M.D., Commissioner of Food and Drugs

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