Draft Guidance for Industry, Researchers, Patient Groups, and Food and Drug Administration Staff

Meetings with the Office of Orphan Products Development

DRAFT GUIDANCE

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You should submit comments and suggestions regarding this draft document within 90 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852. Submit electronic comments to http://www.regulations.gov. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

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U.S. Department of Health and Human Services
Food and Drug Administration
Office of Orphan Products Development (OOPD)

Additional copies are available from:

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I. INTRODUCTION

This guidance provides recommendations to industry, researchers, patient groups, and other stakeholders (collectively referred to in this guidance as "stakeholders") interested in requesting a meeting, including a teleconference, with the Food and Drug Administration's (FDA's) Office of Orphan Products Development (OOPD) on issues related to orphan-drug designation requests, humanitarian use device (HUD) designation requests, rare pediatric disease designation requests, funding opportunities through the Orphan Products Grants Program and the Pediatric Device Consortia Grants Program, and orphan product[1] patient-related topics of concern. It is also intended to assist OOPD staff in addressing such meeting requests. This guidance describes procedures for requesting, preparing, scheduling, conducting, and documenting such meetings.

FDA guidance documents, including this guidance, do not establish legally enforceable responsibilities. Instead, these guidances describe the Agency's current thinking on a topic and should be viewed only as recommendations unless specific regulatory or statutory requirements are cited. The use of the word *should* in Agency guidances means that something is suggested or recommended but not required.

II. BACKGROUND

OOPD staff regularly participate in meetings with stakeholders who seek input from OOPD relating to orphan-drug designation requests, HUD designation requests, rare pediatric disease designation requests, funding opportunities through the Orphan Products Grants Program and

[1] As used in this guidance, "orphan product" may include a drug, device, biological product, or medical food for a rare disease or condition.

the Pediatric Device Consortia Grants Program, and orphan product patient-related issues. These meetings vary from general information-gathering meetings, where stakeholders ask basic questions about the designation or grants process, to more specific and complicated discussions involving, for example, questions related to orphan drug exclusivity.

In some cases, these meetings may represent a critical point in the orphan product development process and may even have an impact on the eventual availability of products for patients with rare diseases and conditions. It is therefore important that these meetings be scheduled within a reasonable time, conducted effectively, and documented where appropriate. This guidance is intended to provide consistent procedures to promote well-managed meetings between OOPD and stakeholders.

III. MEETING TYPES: INFORMAL AND FORMAL

A. Definitions

Meetings between OOPD and stakeholders can be "informal" or "formal."

1. *Informal Meetings*

Informal meetings provide an opportunity for OOPD to address:

- general questions about OOPD policies and procedures;
- standard designation and grant process questions (e.g., how to submit a designation request or grant application, what information to include);
- definitions of basic designation terms (e.g., rare disease or condition, orphan subset of a non-rare disease or condition, prevalence vs. incidence);
- general advice on calculating the population estimate of a disease or condition;
- patient group initiatives related to orphan products; and
- other general questions.

In some instances, an informal meeting may be appropriate to discuss a designation deficiency letter if the stakeholder's questions can be readily addressed and do not require extensive analysis within OOPD and/or extensive discussion between OOPD and the stakeholder.

Informal meetings usually take the form of a brief telephone conversation between OOPD and the stakeholder. For informal meetings, stakeholders do not need to provide a meeting package to OOPD; the information contained in the meeting request and any subsequent communications should suffice (see Section IV). If an informal meeting fails to resolve the stakeholder's questions, OOPD may recommend that the next meeting be a formal meeting. In some instances, if a stakeholder requests an informal meeting, OOPD may advise that the matter be addressed through a formal meeting. Other times, OOPD may be able to resolve the stakeholder's questions through e-mail without having to schedule an informal meeting.

2. *Formal Meetings*

Formal meetings provide an opportunity for OOPD to address:

- specific and often complicated questions related to designation requests such as questions arising from a deficiency letter related to a complex orphan subset issue;
- denied designation requests;
- questions related to orphan-drug exclusivity; and
- other matters that require preparation by OOPD and extensive discussion between OOPD and the stakeholder.

Formal meetings can take the form of an in-person meeting or teleconference and usually involve more participants than an informal meeting. Before a formal meeting occurs, stakeholders should provide a meeting package to OOPD that includes detailed background information and a proposed meeting agenda, among other information (see Section V). In some instances, when a stakeholder requests a formal meeting, OOPD may advise that the matter be addressed through an informal meeting.

It is important to understand that formal meetings described in this guidance are not pre-submission or interactive review meetings.[2] That is, they are not a forum for stakeholders to obtain feedback from OOPD on product development protocols or planned studies or for OOPD to determine whether information in a pending or forthcoming designation request or grant application is complete and adequate. Rather, formal meetings are a forum for stakeholders to obtain clarification from OOPD, and discuss disagreements with OOPD about OOPD policies, positions, and statements, including previous correspondence (e.g., a deficiency letter on an orphan-drug designation).

[2] For more information on pre-submission meetings, see FDA's guidance "Requests for Feedback on Medical Device Submissions: The Pre-Submission Program and Meetings with Food and Drug Administration Staff" (http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM311176.pdf). For more information on interactive review, see FDA's draft guidance "Types of Communication During the Review of Medical Device Submissions" (http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM341948.pdf). FDA's draft guidance represents FDA's proposed approach on this topic. When final, this guidance will supersede FDA's guidance "Interactive Review for Medical Device Submissions: 510(k)s, Original PMAs, PMA Supplements, Original BLAs, and BLA Supplements" http://www.fda.gov/OHRMS/DOCKETS/98fr/07d-0492-gdl0001.pdf). For more information on formal meetings for drugs or biological products, see FDA guidance, "Formal Meetings Between the FDA and Sponsors or Applicants" (http://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm153222.pdf).

B. OOPD Program Areas

Stakeholders may request meetings with OOPD about any of the following orphan product programs:

OOPD Designation Programs
Orphan-Drug Designation (including Orphan-Drug Exclusivity)
HUD Designation
Rare Pediatric Disease Designation (for Priority Review Voucher Program)

Many stakeholder questions about orphan product designations can be addressed through informal meetings, with the exception of orphan-drug exclusivity questions. In OOPD's experience, most exclusivity-related questions are best addressed through formal meetings. Formal meetings are also appropriate for complicated designation questions that require extensive analysis within OOPD and/or extensive discussion between OOPD and the stakeholder (e.g., an orphan subset question that has resulted in several rounds of deficiency letters).

Orphan Product Grants
Orphan Products Grants Program
Pediatric Device Consortia Grants Program
Orphan Product Outreach Program
Patient-related Topics

In OOPD's experience, questions about Orphan Products Grants, Pediatric Device Consortia Grants, and patient-related topics generally can be addressed through informal meetings. For example, informal grant topics may include general questions regarding how to apply for a grant (e.g., what types of products qualify) and the grant review process, as well as questions about ongoing funded grants (e.g., how to handle enrollment issues or reporting requirements). Patient-related topics may include, for example, questions about various orphan product incentives for rare disease product development.

IV. MEETING REQUESTS FOR INFORMAL AND FORMAL MEETINGS

Before requesting a meeting with OOPD, stakeholders should consult OOPD's website, which contains general information about orphan product designations and grants: www.fda.gov/orphan. This website includes links to relevant FDA regulations and guidance documents, as well as responses to Frequently Asked Questions (FAQs).[3] The information on this website may provide the answers and clarification the stakeholder seeks.

[3] These regulations include 21 CFR Part 316 ("Orphan Drugs") and 21 CFR Part 814, Subpart H ("Humanitarian Use Devices"). Relevant guidance documents include FDA's guidance "Humanitarian Use Device (HUD) Designations" (http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/DesignatingHumanitarianUseDevicesHUDS/LegislationRelatingtoHUDsHDEs/ucm283517.htm).

If, after reviewing this publicly available information, the stakeholder still seeks a meeting (including a teleconference) with OOPD, the stakeholder can request a meeting in one of several ways:

- If the requested meeting concerns a designation request or grant application that has already been submitted to OOPD, the stakeholder should request a meeting by e-mailing the identified point of contact for the designation request or grant application, with the subject heading "Meeting Request." This point of contact will be identified in the latest correspondence from FDA, whether an acknowledgment letter, deficiency letter, or other correspondence about the request or application.
- If the requested meeting does not concern a designation request or grant application that has already been submitted, the stakeholder can request a meeting by e-mailing the general OOPD inbox at orphan@fda.hhs.gov, with the subject heading "Meeting Request." Alternatively, the stakeholder can send an e-mail to the point of contact for each OOPD Program Area listed in the "Contact FDA" section of OOPD's website (www.fda.gov/orphan), again with the subject heading "Meeting Request."
- OOPD strongly encourages that meeting requests be sent by e-mail to readily enable back-and-forth exchanges with the stakeholder about scheduling and other details. However, a stakeholder who prefers not to request a meeting by e-mail can submit a written request to:

  Office of Orphan Products Development
  Attention: "Program Contact Name – Meeting Request"
  Food and Drug Administration
  10903 New Hampshire Avenue
  Building 32, Room 5271
  Silver Spring, MD 20993

At a minimum, all meeting requests should include:

- a brief statement of the meeting purpose, including identification of the product to be discussed and any applicable designation request or grant application number;
- whether the stakeholder prefers an informal or formal meeting (see Section III);
- suggested dates and times for the meeting;
- preferred format of the meeting (i.e., teleconference or in-person meeting); and
- the e-mail address(es) to which OOPD should send a response to the meeting request (if different from the e-mail address from which the request was sent) and telephone number for the primary contact for the stakeholder.

OOPD will aim to respond to a meeting request within 5 working days of receipt; OOPD will determine the appropriate meeting type and will work with the stakeholder on identifying a convenient date and time. Before scheduling the meeting, OOPD may ask the stakeholder for more information about the proposed meeting to help determine whether an informal or formal meeting is most appropriate and who from OOPD should attend. This additional information may include a request for a brief list of questions that the stakeholder hopes to have answered.

Once a meeting is scheduled, OOPD will notify the stakeholder by e-mail or phone of the meeting type, date, time, duration, place or call-in number, and expected OOPD participants. If the meeting is to be a formal meeting, stakeholders should submit a meeting package to OOPD that includes detailed background information and a proposed meeting agenda, among the other information described in Section V. For informal meetings, the information in the meeting request may suffice, although OOPD may ask for supplemental information via e-mail or phone. This supplemental information may include a request for a list of questions that the stakeholder hopes to have answered (if not already provided) and a list of individuals, with their titles and affiliations, who are expected to participate on behalf of the stakeholder.

V. MEETING PACKAGE CONTENT FOR FORMAL MEETINGS

If a formal meeting is scheduled, OOPD should receive the meeting package at least 2 weeks before the meeting. This meeting package is intended to help OOPD prepare for the meeting and enable a productive discussion between OOPD and the stakeholder. We encourage stakeholders to submit the meeting package electronically by e-mail to the OOPD program contact who scheduled the meeting, with the subject heading "Meeting Package." If a stakeholder chooses to submit a paper copy, the stakeholder should send the meeting package to the following address:

Office of Orphan Products Development
Attention: "Program Contact Name – Meeting Package"
Food and Drug Administration
10903 New Hampshire Avenue
Building 32, Room 5271
Silver Spring, MD 20993

We expect the length of the meeting package to vary depending on the issues to be discussed, the product, the indication, and the phase of product development. The meeting package should contain the following information:

- The date, time, and subject of the meeting;
- An explanation of the meeting purpose (this explanation should generally be more detailed than the meeting purpose described in the meeting request);
- Basic information about the product to be discussed, including:
    - Product name or identifier;
    - Designation or application number, if applicable;
    - The relevant rare disease or condition;
    - Brief background about the product, including how it may be used for the rare disease or condition;
- A proposed meeting agenda, including a detailed list of questions that the stakeholder hopes to have answered along with any supplemental explanation or context that may help OOPD address these questions (this list should generally be more extensive and/or detailed than any list provided as part of a meeting request);
- Any data, information, or presentation materials to support the discussion, if needed; and

- A list of all individuals, with their titles and affiliations, who are expected to participate in the meeting on behalf of the stakeholder. Note that a stakeholder may include patients and/or other subject-matter experts at the meeting with OOPD.

If a formal meeting is scheduled and OOPD fails to receive a meeting package at least 2 weeks before the meeting, OOPD may choose to postpone the meeting given the lack of adequate preparation time to review the meeting package. OOPD will notify the stakeholder of this postponement and will work with the stakeholder to reschedule the formal meeting for a later date.

VI. MEETING PROTOCOL

Meetings will be chaired by an OOPD staff member and will begin with introductions and a statement of the meeting purpose. The stakeholder will then take the lead, for example, by summarizing the meeting agenda, giving a presentation, and asking questions. Presentations should generally be kept brief to maximize the time available for discussion. If a stakeholder requires any audio-visual requirements for the meeting, this should be discussed ahead of time. OOPD advises that stakeholders limit their presentations and questions to the information previously submitted to OOPD (whether as part of the meeting request for informal meetings or in the meeting package for formal meetings), with added explanation and clarification as necessary. If any new information or questions are presented, OOPD may not be in a position to substantively engage with the stakeholder on the new topics.

At the close of the meeting, OOPD and stakeholder attendees should summarize the important discussion points, meeting outcomes, and any action items. If meeting minutes are warranted (see Section VII), OOPD will remind the stakeholder to prepare and submit a draft summary of meeting minutes within 15 working days after the meeting.

VII. DOCUMENTATION OF MEETINGS

Documentation of meeting outcomes, agreements, disagreements, and action items is critical to ensuring that this information is preserved for meeting attendees and future reference. Summary meeting minutes should be prepared for all formal meetings and certain informal meetings, if appropriate (e.g., where disagreements arise between OOPD and the stakeholder or where the stakeholder and OOPD agree on specific action items). Within 15 working days following the meeting, the stakeholder should provide a draft of summary meeting minutes to the OOPD program contact by e-mail, with the subject heading "Draft Meeting Minutes." This draft should summarize the meeting discussion points, agreements, disagreements, and action items. OOPD will review the draft and provide revisions to the stakeholder via e-mail in a timely manner (generally within 15 working days). The stakeholder will then either accept the version as final and notify OOPD to that effect or follow up with questions and/or further revisions.

Once the meeting minutes are finalized, OOPD will provide copies by e-mail to all OOPD attendees and to the stakeholder. These minutes will serve as OOPD's record of the meeting and be included in all relevant files.