LEADER3D: Docket Description

Title:

Opportunity for Public Comment on Rare Disease Educational Materials from the Center for Drug Evaluation and Research's Accelerating Rare disease Cures Program and the Rare Disease Innovation Hub

Background

Rare diseases affect an estimated 25 to 30 million individuals in the United States, approximately half of whom are children. Despite scientific advances and existing incentives, the majority of rare diseases lack approved therapies. Drug development for rare diseases presents unique scientific, clinical, and regulatory challenges that require tailored educational resources for multiple stakeholder communities.

FDA has undertaken multiple efforts to support rare disease drug development through education, outreach, and regulatory science. In December 2022, as part of CDER's Accelerating Rare disease Cures (ARC) Program, FDA published a public docket (FDA-2022-N-3226-0001) seeking input on knowledge gaps and priority topics in rare disease drug development and related regulatory considerations. Input received through that docket informed a landscape assessment published as a public report in 2024, and the subsequent development of publicly available educational materials, including case studies and videos addressing scientific, clinical, and regulatory topics relevant to rare disease drug development.

Building on the 2022–2023 LEADER 3D public docket and the educational materials developed as a result of that engagement, CDER ARC is now seeking targeted input through a new public docket focused on feedback on Learning and Education to ADvance and Empower Rare Disease Drug Developers (LEADER 3D) educational materials.

Request for Feedback on Existing LEADER 3D Educational Materials

In this docket, CDER ARC is seeking feedback on the resulting educational materials developed under the LEADER 3D Program. This request represents a next phase of engagement focused on evaluating existing LEADER 3D materials, to inform future iterations of program-specific educational resources for stakeholders engaged in rare disease drug development. FDA invites comments from stakeholders involved in rare disease drug development, including industry, academia, and other federal partners, on topics such as:

- The relevance and clarity of existing LEADER 3D educational materials
- The effectiveness of current formats, such as case studies and videos
- Suggestions for additional topics or formats for potential future materials that would further support rare disease drug development

With input from stakeholders actively engaged in the design and conduct of rare disease drug development programs, including industry, academia, and other federal partners, FDA intends to

evaluate and refine the educational materials developed under the LEADER 3D Program. Feedback received will be used to identify areas where existing materials could be expanded, or supplemented, as well as to inform the prioritization and development of the next phase of publicly available LEADER 3D educational resources. Through this iterative approach, FDA seeks to build on prior engagement and support the ongoing development of safe and effective therapies for rare diseases.

Additional Opportunity for Public Comment from the Rare Disease Innovation Hub

The FDA Rare Disease Innovation Hub (Hub) works across FDA medical product centers and programs to promote coordination and alignment to advance safe and effective rare disease therapies. In 2022, the Food and Drug Administration (FDA) issued a request for public input on scientific, clinical, and regulatory challenges and priority topics in rare disease drug development (Docket No. FDA-2022-N-3226). FDA appreciates the input received through that effort. Accordingly, The Hub is seeking information to better understand what educational resources are currently available for patients/patient organization and developers of CBER-regulated rare disease biologics. FDA is also interested in understanding where gaps in available resources may exist.

Educational Resources for Patients and Patient Organizations

The Hub invites stakeholders to identify existing educational resources intended for patients and patient organizations that relate to rare disease product development. Submissions should identify specific resources (for example, by name and where they can be accessed) and briefly describe the aspects of rare disease product development they address. The Hub is also asking patients and patient organizations to highlight areas of rare disease product development where desired educational resources for patients may not exist.

Educational Resources for Rare Disease Biologics Developers

The Hub invites stakeholders to identify existing educational resources intended to support developers engaged in rare disease biologics development. Feedback should identify specific resources and where they can be accessed and may address any stage of development.

The Hub is also interested in understanding areas of rare disease biologics development where educational resources are limited or absent.

Feedback should focus on identifying existing resources and highlighting areas where there are gaps in educational materials. FDA is not seeking proposals for new programs or initiatives through this request.

Submission of Comments

Responses to both queries may be submitted within one document. Comments submitted in response to this notice should clearly indicate whether they pertain to:

- CDER ARC's LEADER 3D program educational materials for rare disease drug developers, or

- The Rare Disease Innovation Hub's request for input on educational resources that support patients and patient advocates, and those related to CBER regulated biologics in the development of rare disease therapies.

For comments to be considered, please submit them by April 3, 2026.