Rare Disease Innovation Hub

Strategic Agenda 2026

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Rare Disease Innovation Hub Mission

FDA created the Rare Disease Innovation Hub (the Hub) to serve as a point of collaboration and connectivity between FDA's medical product Centers and rare disease programs with the goal of improving outcomes for rare disease patients. The Hub enhances collaboration across FDA to pinpoint and address common scientific, clinical, and policy issues related to rare disease product development, including relevant cross-disciplinary approaches related to product review, and promote consistency across offices and Centers. Although the Hub works across all rare diseases, it particularly focuses on challenges within smaller populations or for diseases where the natural history is variable and not fully understood.

2025 Accomplishments

2025 was the Hub's first full year of operation following its launch in fall of 2024. Its launch involved substantial internal and external socialization of the Hub's role in relation to the medical product Centers and the Agency's rare disease initiatives, identification of medical product Center staff to push forward the Hub's ambitious goals for the year, and the securing of resources to support the Hub's new initiatives. With engagement of the medical product Centers concerning these activities, but with no dedicated budget for 2025 and no full-time staff apart from its Director, the Hub made significant progress on its core goals in 2025, the results of which are outlined here. The Hub will continue to build on its 2025 goals and initiatives in 2026.

Goal 1: Further Advance Regulatory Science of Rare Disease Therapies

- The Hub piloted an on-going Rare disease Innovation, Science, and Exploration (RISE) Workshop series, which focuses on issues common to development of therapies for multiple rare diseases and for which innovative methods and evolving regulatory science may offer valuable solutions.

- The Hub held two RISE Workshops in 2025, one focusing on control arm options for rare disease clinical trials with small patient populations and diminishing numbers of eligible trial participants and a second focused on individualized therapies. Registration for these workshops averaged about 2,000 participants per program, both in-person and virtual.

- The Hub further solicited topics for future RISE Workshops via comments in a public docket.

Goal 2: Enhance and Strengthen Coordination and Alignment Between Medical Product Centers

- The Hub launched the Rare Disease Policy and Portfolio Council (RDPPC), a cross-center collaborative body that meets monthly to convene senior medical product leadership and subject matter experts from CBER and CDER to align on regulatory approaches regarding complex challenges in rare disease drug development relevant to both Centers. The RDPPC is a forum for the Centers' subject matter experts to share knowledge regarding product

applications in the same or similar disease states, align on review approaches, and identify areas for development in FDA's regulatory science. RDPPC discussions in 2025 have led to substantive collaborations between medical product Centers, including an internal working group and a public workshop focused on ophthalmology endpoints. The value of the RDPPC has also been extended to a more regularized internal process for cross-center connections between CDER and CBER when the Centers are addressing novel or complex issues in rare disease drug development.

- The Hub led the development of the Rare Disease Evidence Principles (RDEP) to facilitate the approval of drugs to treat rare diseases with very small patient populations with significant unmet medical need and with known genetic defects that are the major pathophysiologic drivers. RDEP offers the assurance that drug review will encompass additional supportive data in the review.

Goal 3: Create a Centralized Point of Contact for External Partners

- The Hub created a public-facing email address and website to promote contact with the rare disease community and to facilitate the sharing of information about Hub initiatives

- The Hub initiated a year-long landscape assessment and gap analysis of public-facing rare disease educational materials focused on biologics development and the role of patients and disease advocates in the development of rare disease therapies. The project will culminate in a report with recommendations for future development of FDA educational offerings.

- Hub leadership engaged in extensive public outreach throughout the year with rare disease advocacy organizations and industry groups, both socializing the Hub with the rare disease community and publicizing FDA's work in the rare disease space.

Rare Disease Innovation Hub Infrastructure

The Hub's primary function is to play a coordinating role between rare disease efforts within FDA's medical product Centers, which includes identifying and developing strategies to address cross-cutting issues that concern the development and review of therapies across product areas. The Hub works with the many FDA programs focused on rare diseases.[1] The Hub's task is neither to supplant nor to take over any major operations from existing programs, but instead to implement strategies to ensure coordination and alignment between FDA's rare disease teams.

For 2026, the Hub received limited dedicated funding via commitments of $250,000 from CBER and $750,000 from CDER. These commitments will fund a small team of Hub staff and facilitate activities supporting the Hub's 2026 goals. Additional support for the Hub's planned activities will continue to be supported by several CDER and CBER staff members who work on Hub activities

[1] GAO-25-106774, RARE DISEASE DRUGS: FDA Has Steps Underway to Strengthen Coordination of Activities Supporting Drug Development

as a portion of their duties. Additional resources to fully fund the Hub are still being pursued and would enable the Hub to expand its programming.

Rare Disease Innovation Hub Goals

Goal 1: Further Advance Regulatory Science of Rare Disease Therapies

Create and enhance opportunities for consideration of novel endpoints, biomarker development and assays, innovative trial design, real world evidence, and statistical methods. Initiate engagement with federal research partners and the broader rare disease research community with the purpose of sharing scientific innovations and enhancing collaboration in regulatory science.

Goal 2: Enhance and Strengthen Coordination and Alignment Between Medical Product Centers

Move the agency towards greater cross-Center consistency on rare disease standards and decisions, facilitate regular collaboration between leadership and review teams between FDA's medical product Centers and rare disease programs. Promote cross-Center information sharing and, where possible, alignment between Centers on review standards and decision-making. Where scientific reasons exist for differences in review approaches between medical product Centers, promote clarity about those reasons with the rare disease community. Encourage transparency and communication between FDA Centers and drug developers throughout the review process.

Goal 3: Create a Centralized Point of Contact for External Partners

Serve as a primary point of connection and engagement with the rare disease community, including patient and caregiver groups, trade organizations, and scientific/academic organizations, for matters that concern the development of therapies for rare diseases. Promote engagement with the rare disease community via enhancements to rare disease information publicly available throughout the Agency's webpage and facilitate the development of public information regarding the Agency's rare disease efforts.

Rare Disease Innovation Hub 2026 Actions

Goal 1: Further Advance Regulatory Science of Rare Disease Therapies

Rare disease Innovation, Science, and Exploration Workshop Series

Based on the success of the 2025 Rare disease Innovation, Science, and Exploration (RISE) Workshops in bringing together innovators in drug development, rare disease research, patient advocacy, and regulatory science to discuss critical issues and new methods in the development of rare disease therapies, the Hub will continue to sponsor the RISE Workshop series in 2026 and beyond. In 2026, the Hub will sponsor up to three RISE Workshops, facilitated by the Duke

Margolis Institute for Health Policy, on challenges that are common to multiple rare diseases or a class of diseases and for which evolving science offers innovative solutions. The workshops will be open to the public and designed for interaction, sharing of perspectives, and discourse between the various rare disease community members, including drug developers, patients and patient/disease organizations, academics, FDA regulators and reviewers, and relevant staff from other federal agencies. The internal agency planning group for each of these workshops will at minimum include staff from both CBER and CDER, with CDRH and other agency offices represented as appropriate for the topic. The workshops will also include a discussion of the role of patients and patient/disease organizations in the design and implementation of innovative solutions. The workshops will primarily focus on cross-cutting or common issues and will not be focused on any specific product under review by the Agency.

Topics for RISE Workshops are based on proposals submitted by the rare disease community via a public docket. The Hub selected a topic for the March 30, 2026, RISE Workshop from a docket that was open through December 31, 2025, and the Hub will maintain a 2026 docket to receive further proposals. In reviewing the proposals, preference will be given to those submitted as collaborative efforts between multiple other organizations, individuals, or companies.

Patient and Patient Organization Engagement in the Development and Review of Drugs for Rare Diseases

FDA currently has many opportunities for patients, patient organizations, and disease organizations to engage with the Agency and to provide input on patient experience and preferences. FDA's most utilized programs are FDA-led Patient-Focused Drug Development (PFDD) Public Meetings, Externally-led Patient-Focused Drug Development Meetings, and Patient Listening Sessions. These patient engagement opportunities provide the Agency and stakeholders in the rare disease community with valuable information about patient experience, risk tolerance, and the relevance of clinical endpoints to the lived experience of the patients who are using medical products.

FDA has also issued multiple guidance documents on patient-focused drug development, offering insight on multiple topics including collecting input from patients and patient representatives, identifying what is important to patients, encouraging fit-for-purpose clinical outcome assessments, and incorporating clinical outcome assessments into endpoints for regulatory decision-making. These guidance documents are intended to facilitate patient organizations' engagement in the drug development process, and to encourage drug developers to voluntarily involve patients and patient organizations throughout the drug development process.

While this active involvement with patients and patient organizations has had a significant and positive impact on FDA's incorporation of the patient experience on drug development, there remains a strong interest among patient and disease organizations and many drug developers for deeper and more substantive involvement of patients and the patient voice in drug development processes. To address this sustained interest, the Hub is planning a 2026 program on the role of the patient voice in informing drug development. This event will provide industry partners, patient advocacy groups, and disease organizations a forum to suggest enhanced opportunities to involve patients and patient advocates in the drug development process.

Goal 2: Enhance and Strengthen Coordination and Alignment Between Medical Product Centers

Promote Knowledge-Sharing Between Medical Product Centers Regarding Rare Disease Treatment Review

CBER and CDER share a vision of a drug review process that takes full advantage of the deep knowledge and experience of all of FDA and its clinical and scientific staff. One aspect of that vision is ensuring that our comprehensive drug reviews are conducted within the context of other applications, approval decisions, and related information within the Agency, particularly when it comes to innovative approaches in the areas of novel endpoints, biomarker development, and trial designs for rare diseases. It is imperative that internal expertise be shared within and across Centers so that knowledge gained in a single IND, NDA, or BLA review, or during Patient-Focused Drug Development Meetings and Patient Listening Sessions can be retained and utilized across the Agency as institutional knowledge.

The Hub will work with FDA's medical product Centers to put in place best practices that promote drug review information sharing opportunities that will have broader cross-Center implications throughout the rare disease drug review process, from pre-IND through NDA or BLA. The Hub previously launched the Rare Disease Policy and Portfolio Council (RDPPC), which is a senior level forum to promote cross-Center dialogue on challenging and complex rare disease drug development program-related issues. The RDPPC meetings bring together technical experts from across FDA's medical product Centers to discuss scientific, regulatory science, and policy issues related to rare disease product development that are common to the Centers. Building upon the continued success of the RDPPC meetings, the Hub will work with the leadership of medical product Centers to establish additional practices and policies to regularize cross-Center communication regarding the same or related diseases or issues, particularly regarding innovative approaches in rare disease, to ensure that drug review within the Centers have common approaches when evaluating similar populations or issues. While the drug product reviews within each Center remain independent, the Hub will work with the Centers to create systems that proactively share useful and relevant information and facilitate access to those seeking information regarding specific disease states or populations.

Rare Disease Review Staff Training Feedback Pilot

The development of rare disease therapies is an evolving scientific landscape where researchers and drug developers leverage novel endpoints and biomarkers, harness cutting-edge technologies, and apply new methods in the pursuit of urgently needed treatments. Part of FDA's regulatory mission is to ensure that reviewers are prepared to encounter these innovations and new methods in ascertaining the safety and efficacy of new therapies for rare disease. CDER and CBER rare disease program staff work together each year to develop a unique curriculum with didactic and case study presentations given by experienced staff from both Centers for the Annual Rare Disease Training for Reviewers, a PDUFA commitment since PDUFA V (2013-2017). In line with the objective for this annual training as stated in the PDUFA VII (2023-2027) commitment letter, it is designed to familiarize review staff with challenges frequently associated with rare disease applications and strategies to address them, promote best practices for review and regulation of rare disease applications, e.g., consistency, and encourage flexibility and scientific judgment among reviewers in the review and regulation of rare disease therapies.

Because the scientific innovations being leveraged in the development of rare disease therapies are progressing so quickly, the Hub recognizes the need for increased engagement with the rare disease community to ensure that the latest techniques and methods are represented in FDA's annual rare disease reviewer training. In 2026, the Hub will pilot a program to solicit topics for the coming year's CBER and CDER rare disease reviewer training program. Resources permitting, the pilot will include a meeting with industry and other rare disease community stakeholders to propose innovations for inclusion in upcoming rare disease reviewer training. The Hub will coordinate this event with the rare disease reviewer training planning committee.

Goal 3: Create a Centralized Point of Contact for External Partners

Public, Agency-Wide Rare Disease Newsletter

FDA currently has numerous rare disease-specific programs across the Agency.[2] While many are housed in CDER and CBER, some are within other FDA Centers and offices. The Hub aims to ensure that the urgent and important work of each of these programs is publicized and accessible for the rare disease community through the dissemination of a public-facing newsletter that will highlight rare disease initiatives from across the entire Agency.

The newsletter, which will be published quarterly and disseminated via GovDelivery, will collect news and programmatic activities from rare disease programs across the FDA. The newsletter will share information about noteworthy rare disease product approvals, publicize upcoming events that are open to the rare disease community, and alert the community to new guidance and rare disease initiatives at the Agency.

Website-Based Rare Disease Roadmap

The public feedback regarding the launch of the Hub emphasized the need for a clear entry point to FDA's rare disease programs that would provide greater clarity regarding where external community members should go for their issues or questions, and guidance on the distinctions between many of these programs. For example, one commenter suggested that the Hub be a "…centralized "First Stop" webpage for comprehensive rare disease resource." Another focused on the numerous FDA programs from the drug developer's perspective, suggesting that "we would recommend FDA to prepare a road map to help drug developers navigate the various regulatory science initiatives and opportunities and delineate the linkage across centers."

In 2026, the Hub will undertake a website consolidation project intended to make information about rare disease programs at the Agency more accessible to the rare disease community, including patient advocates, drug developers, and researchers. This project may include the design of a single website that encompasses all FDA rare disease programs and is searchable and valuable to all rare disease community members. Due to potential resource constraints, the Hub is also considering less comprehensive alternatives that place all programs in one website location comprised primarily of brief explanations and links to existing programs.

[2] GAO-25-106774, RARE DISEASE DRUGS: FDA Has Steps Underway to Strengthen Coordination of Activities Supporting Drug Development

Hosting FDA Rare Disease Day 2026

Every year, Rare Disease Day is observed around the world as a means of raising awareness and generating change for the millions of people living with a rare disease. On February 23, 2026, the Hub, together with our partners in the medical product Centers, will host this year's conference, which is specifically directed to the rare disease community—patients, caregivers, families, and patient advocate groups. Our purpose is to create a bridge to FDA—to help our stakeholders understand our processes for promoting rare disease drug development within the Agency.

Encourage Hub Interaction with the Community at Multiple Meetings and Conferences

A key purpose of the Rare Disease Innovation Hub is to engage directly with the rare disease community, including drug developers, patients, patient organizations, and researchers to ensure that FDA is informed of the concerns and needs of the rare disease community and that the community is given all appropriate opportunities to engage with FDA. This engagement is fundamental to the Hub's mission of facilitating the development, evaluation, and availability of safe and effective therapies for rare diseases. In 2026, the Hub will seek opportunities to engage directly with the rare disease community. This engagement will include conferences, meetings, webinars, and similar gatherings, and will provide opportunities for the Hub's Director to educate about Hub activities and hear feedback from the community on this Strategic Agenda, other FDA rare disease programs, and areas where the Agency needs to enhance its access and communication.

Conclusion

This year's Rare Disease Innovation Hub Strategic Agenda is intended as a guidebook for the year and as a way of publicly sharing the Hub's goals. It is the expectation of FDA leadership and the Hub that this Agenda will evolve as the Hub evolves. It is also anticipated that the rare disease community will remain heavily engaged in this evolution. Throughout the year, there will be multiple opportunities for the community to engage with and offer suggestions to the Hub, both regarding the implementation of its 2026 goals and future work. We encourage all rare disease community members to partner with the Hub in our continued growth and work.