Accelerating Rare disease Cures (ARC) Program

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CHALLENGES in the Design and Conduct of Rare Disease Clinical Trials

- **Natural history** is often poorly understood
- Diseases are progressive, **serious, life-limiting** and often **lack adequate approved therapies – urgent unmet needs**
- **Small populations** often restrict study design options
- **Phenotypic and genotypic diversity** within a disorder
- Development programs often **lack solid translational science background**
- **Drug development tools** - biomarkers and outcome measures – **often lacking**
- **Lack of precedent, including clinically meaningful endpoints**, for drug development in many rare diseases

CONSIDERATIONS in the "Environment" for Rare Disease Drug Development

- Many smaller companies with less regulatory experience
- Active patient stakeholder groups looking to navigate and participate in rare disease drug development
- A dedicated academic community that may have limited knowledge of regulatory requirements or aspects of clinical trial development

We must engage our stakeholders to enhance their understanding, improve efficiency, and gain alignment to accelerate rare disease drug development

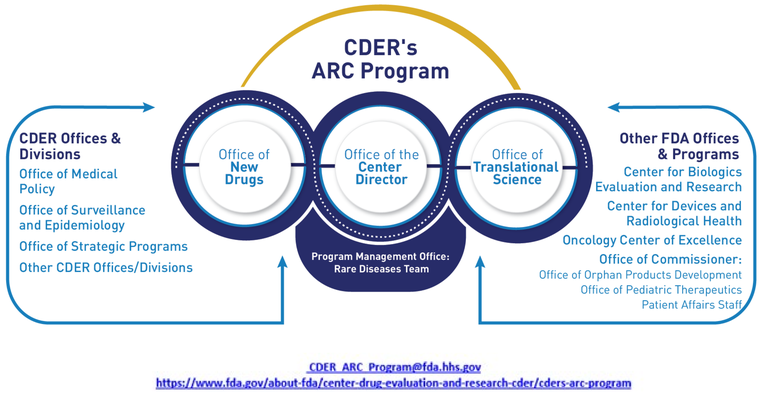
CDER'S ARC PROGRAM

With this engagement in mind, CDER developed the ARC Program

Last year, CDER launched the ARC Program to bridge the gap between the complexities of rare disease drug development and the pressing needs of patients.

Vision: Speeding and increasing the development of effective and safe treatment options addressing the unmet needs of patients with rare diseases.

Mission: To drive scientific and regulatory innovation and engagement to accelerate the availability of treatments for patients with rare diseases.



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https://www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/cders-arc-program

ARC utilizes mission-driven cross agency collaboration and engagement to achieve its vision

ARC'S FIRST YEAR

One year later, ARC has emerged as a conduit for empowering rare disease stakeholders (patients, patient advocates, drug developers, and academic researchers) to harness their collective experiences and expertise to drive progress.

Year One by the Numbers

- **22** rare disease new drug approvals
- **25+** public speaking engagements featuring the ARC Program
- **19** externally-led Patient-Focused Drug Development Meetings on rare diseases supported by CDER staff
- **23** patient listening sessions on rare diseases supported by CDER staff
- **4** ARC Quarterly Newsletters
- **10k+** CDER Rare Disease News subscribers

Other highlights from the Past Year

- Publication of the Program's **Anniversary Update** Report
- Launch in late 2022 of **Learning and Education to Advance and Empower Rare Disease Drug Developers (LEADER 3D**)
- Addition of "Original Rare Disease Application Approval" and "Novel Rare Disease Drugs Approval" filters to CDER's Drugs and Biologics Dashboard hosted on **FDA-TRACK**.

LEADER 3D

ENGAGEMENT, COLLABORATION, AND EDUCATION

CDER's Rare Diseases Team (RDT) is leading cross-center work on the LEADER 3D initiative to better understand the unique challenges in bringing rare disease products to market and, where necessary, **develop educational materials to help address challenges and confusion around regulatory processes.**

New Resource on the ARC Website

Consistent feedback from LEADER 3D outreach is that **rare disease stakeholders have a hard time locating or accessing clear, cross-cutting guidance.**

- We've compiled a collection of currently available guidance relevant to rare disease drug development.
- This list is not exhaustive, but **focused on what we heard from stakeholders they would find helpful**.
- The list is also **dynamic and will be updated periodically**.
- Guidance Documents for Rare Disease Drug Development

LOOKING TO THE FUTURE…

In year two ARC will build on outreach efforts to further empower the community by:

- **creating and disseminating educational materials** through the LEADER 3D initiative;
- partnering with the CDER Patient-Focused Drug Development program to **develop patient materials**;
- **sharing what has been learned from using novel endpoints for rare disease** therapies in the Rare Disease Endpoint Advancement pilot program; and
- **continuing participation in patient listening sessions** with rare disease advocates.

The program also aims to make strides in the scientific and regulatory aspects of rare disease drug development to include **expanding efforts in translational medicine approaches for individual rare diseases**.