



Accelerating Rare disease Cures (ARC) Program

UPDATE ON CDER ENGAGEMENT INITIATIVES FOR RARE DISEASES

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Rare Diseases Team

DRDMG | ORPURN | CDER | US FDA

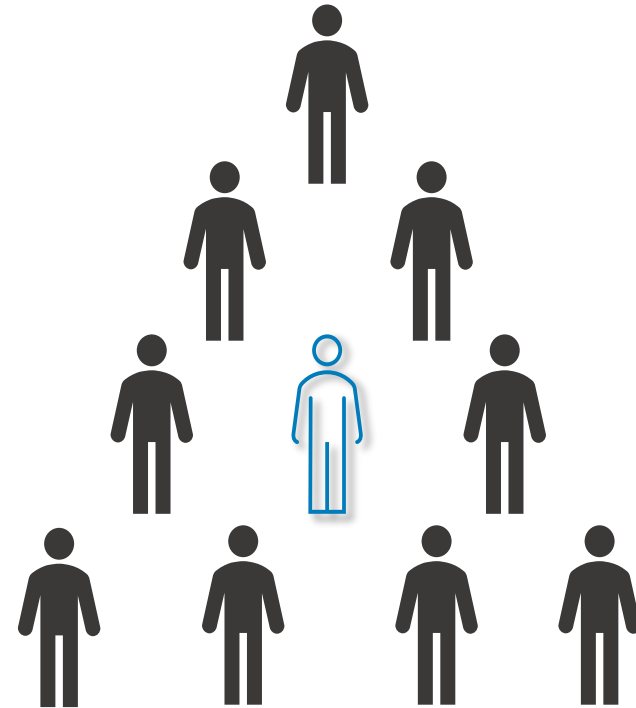


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Rare Diseases and Available Treatments

- About **1 in 10** Americans have a Rare Disease
 - ~30 million people in the US
- The majority of rare diseases do not have FDA approved treatment



We Face Common Challenges in Rare Disease Drug Development

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

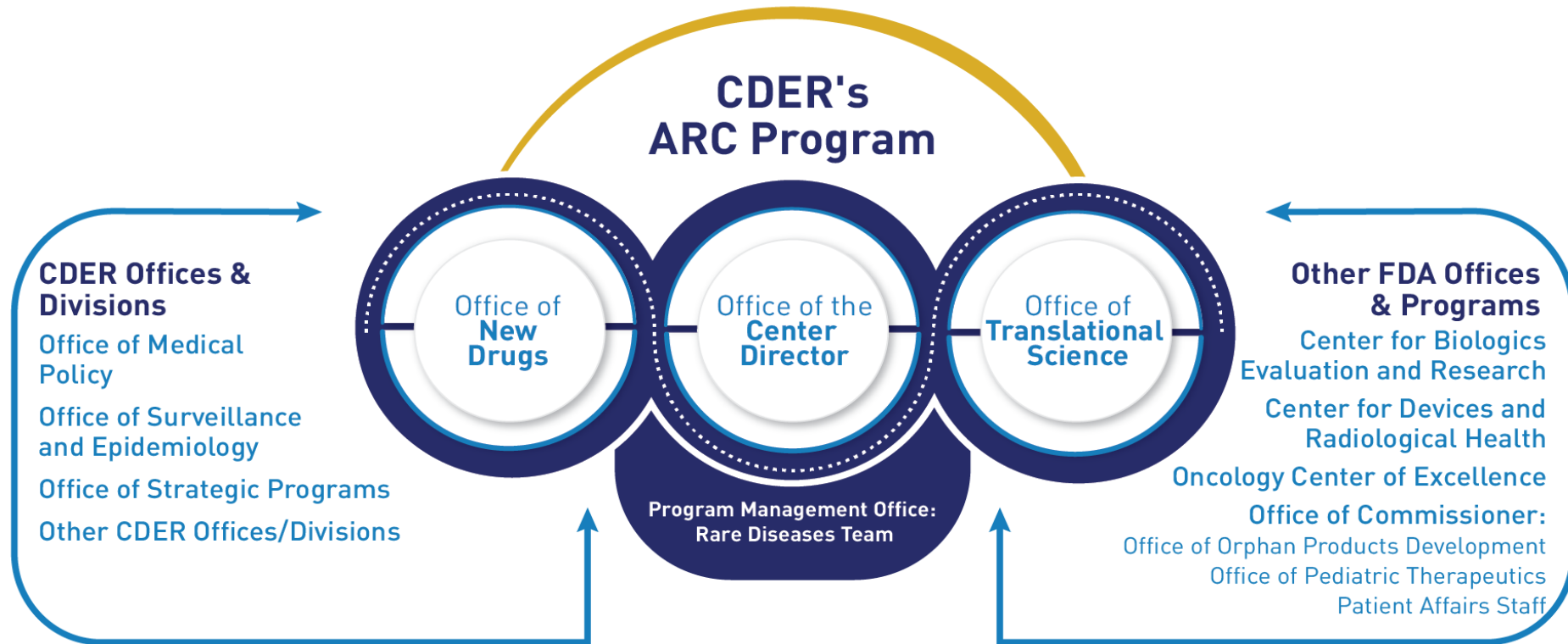
And, Common Considerations in the “Environment” for Rare Disease Drug Development

- 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, and gain their alignment and support

CDER's Accelerating Rare disease Cures Program



CDER_ARC_Program@fda.hhs.gov

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

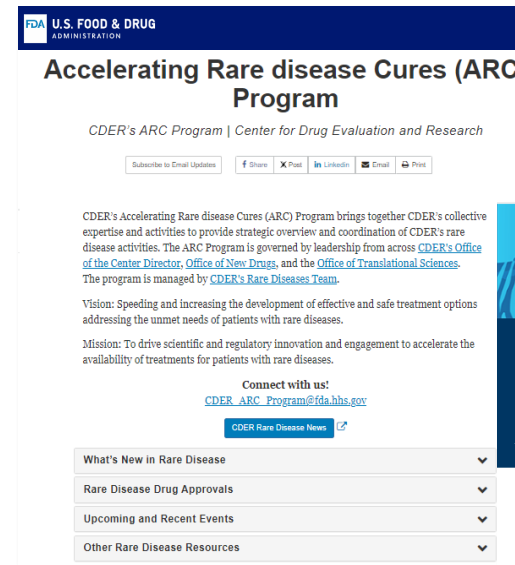
ARC Website

- <https://www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/accelerating-rare-disease-cures-arc-program>

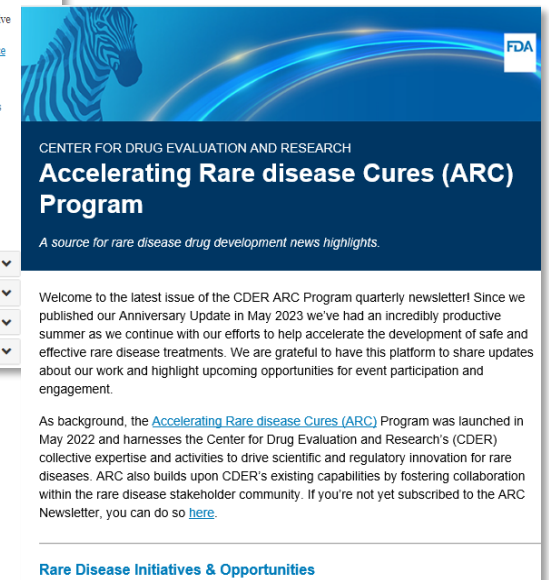


Selected Engagement Efforts

- Learning and Education to ADvance and Empower Rare Disease Drug Developers (**LEADER 3D**)
- ARC Website Resource Tabs
 - Educational Conferences and Workshops Tab
 - Guidance Tab
 - Funding Opportunities Tab
- ARC Quarterly Newsletter

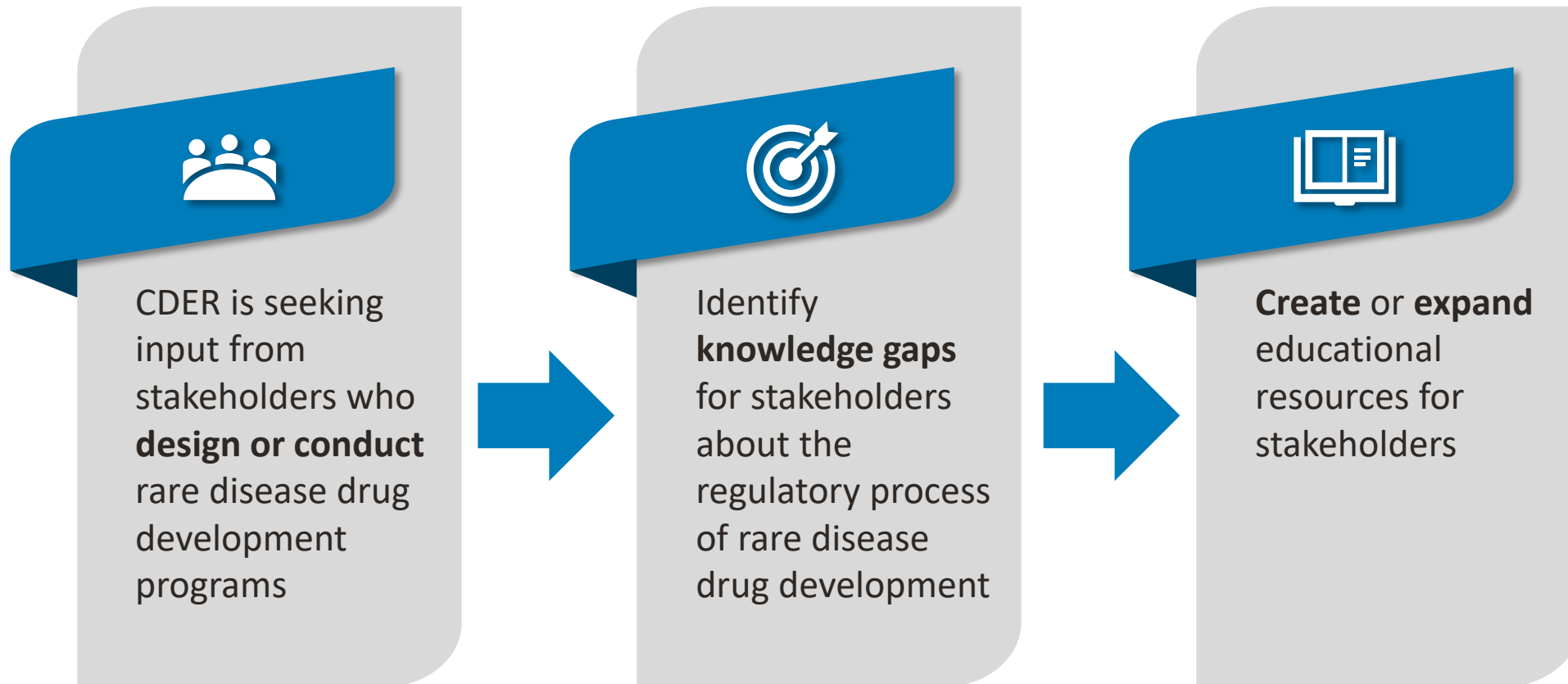


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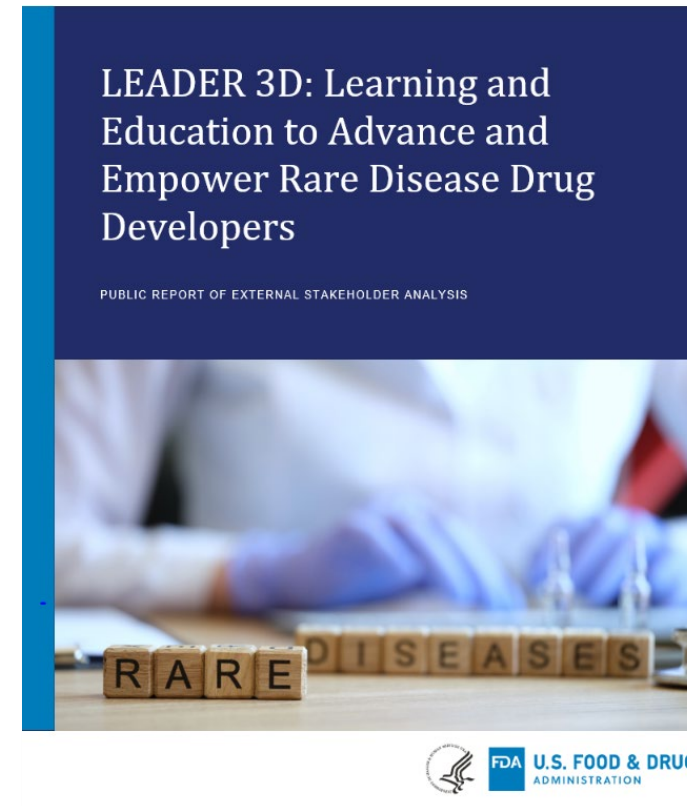
ARC Quarterly Newsletter

What is LEADER 3D?



LEADER 3D: Public Report

- Topics include feedback from members from the external rare disease community who design or conduct rare disease drug development programs on:
 - Nonclinical studies
 - Dose-Finding
 - Natural History Studies and Registries
 - Novel Endpoint and Biomarker Development
 - Clinical Trial Design and Analysis
 - Rare Disease Drug Development Regulatory Considerations



LEADER 3D Based Developments: ARC Website Resources



Educational Conferences and Workshops Tab

Serves as a hub for educational conferences relevant to rare disease topics



Guidances for Rare Disease Drug Development Tab

Selected guidances relevant to rare disease drug development organized by topic



Funding Opportunities Tab

Available funding and fellowship opportunities for rare disease product development research

ARC Website: Conferences and Workshops Tab

ARC Website

Drop Down Tab

ARC Website: Rare Diseases Guidance Placard

FEATURED CDER RARE DISEASE PROJECTS AND ACTIVITIES

YEAR ONE: Anniversary Update

Anniversary Update

Read about how the program is driving innovation through collaboration and engagement with rare disease stakeholders

Kerry Jo Lee, M.D.

The ARC Program Introduction Video

Watch Dr. Kerry Jo Lee, Associate Director for Rare Diseases, share more about the vision and mission of the program

Funding Opportunities in Rare Diseases

Funding Opportunities

Learn about available funding opportunities for rare disease product development research

Rare Disease Cures Accelerator

Learn about effort to support innovation and quality in rare disease drug development

Rare Disease Endpoint Advancement (RDEA) Pilot Program

Learn how the program supports novel endpoint efficacy development for drugs that treat rare diseases

Guidance for Industry

Guidances for Rare Disease Drug Development

Review selected guidances that are relevant to rare disease drug development, organized by topic

ARC Guidance Placard

Guidance for Industry

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Placard Topic Selections

U.S. FOOD & DRUG ADMINISTRATION

Guidance Documents for Rare Disease Drug Development

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In general, FDA's guidance documents do not establish legally enforceable responsibilities. Instead, 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.

Below are selected guidances that are relevant to rare disease drug development, organized by topic. This list does not include all FDA guidances on or relevant to rare disease drug development but represents our most commonly used guidances. This list may be updated periodically.

You can search all FDA Guidances by topic, FDA Center, or issue date [here](#).

- Rare Disease
- Benefit-Risk
- Biomarkers
- Clinical Pharmacology
- Clinical Trials
- Complex Innovative Trial Design
- Communication with FDA
- Digital Health
- Effectiveness
- Expanded Access
- Expedited Programs
- Individualized Antisense Oligonucleotide Drugs Products
- Investigational New Drug Applications
- Meetings with FDA
- New Drug Applications (NDAs)
- Neurology
- Non-Clinical
- Orphan Designation
- Patient Focused Drug Development (PFDD)
- Patient Reported Outcomes
- Pediatrics
- Real World Evidence
- Statistical Analysis
- Voucher Program

ARC Website: Funding Opportunities Placard

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Funding Opportunities Placard

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Learn About Funding Opportunities

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Funding Opportunities for Rare Diseases at FDA

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To help support product development, FDA funds research. Some of these research opportunities are relevant to rare disease product development. Please note that not all opportunities are currently available. A list of current and open FDA rare disease funding opportunities can be found on the [NIH Grants and Funding](#) page. A list of programs that are relevant to rare disease research is located below:

- [Office of Orphan Products Development \(OOPD\)](#)
 OOPD has several [funding opportunities](#) that help promote the development of drugs, devices, biologics, and medical foods for patients with rare diseases and special populations.
 - The [Clinical Trials Grants Program](#) funds clinical trials evaluating efficacy and/or safety in support of a new indication or change in labeling to address unmet needs in rare diseases or conditions. The program encourages innovative and efficient clinical trial methods such as adaptive and seamless trial designs, modeling and simulations, and basket and umbrella trials.
 - The [Natural History Studies Grant Program](#) supports efficient and innovative natural history studies that advance medical product development in rare diseases/conditions with unmet needs. These studies can help at every stage of product development, such as identifying the patient population, identifying or developing clinical outcome assessments and biomarkers, and when appropriate, serving as external controls. This program is intended to fund well-designed, protocol-driven natural history studies with high quality and interpretable data elements that address knowledge gaps, support clinical trials and advance rare disease medical product development.

ARC's Quarterly Newsletter

- To subscribe: [U.S. Food and Drug Administration \(govdelivery.com\)](https://www.fda.gov/delivery)



New Guidance

GUIDANCE DOCUMENT

Rare Diseases: Considerations for the Development of Drugs and Biological Products

DECEMBER 2023

The Purpose

