Accelerating Rare disease Cures (ARC) Program

UPDATE ON CDER ENGAGEMENT INITIATIVES FOR RARE DISEASES

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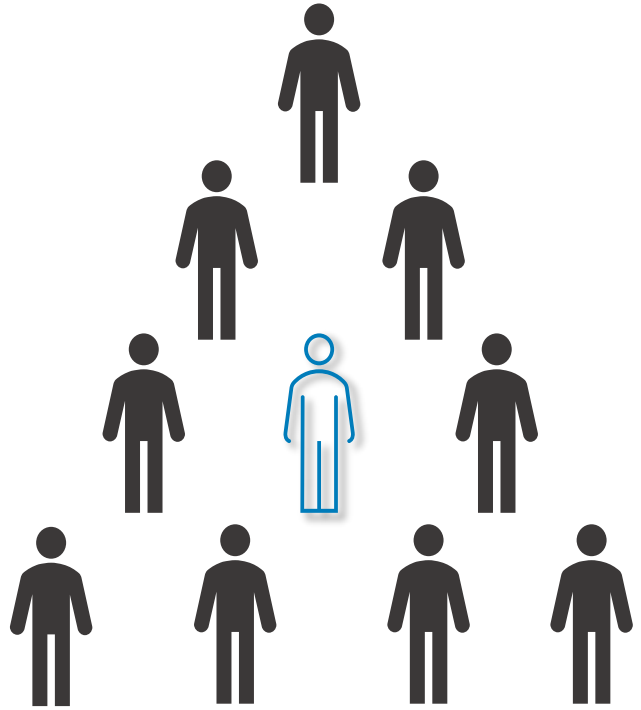
DRDMG | ORPURM | CDER | US FDA

FDA U.S. FOOD & DRUG ADMINISTRATION

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

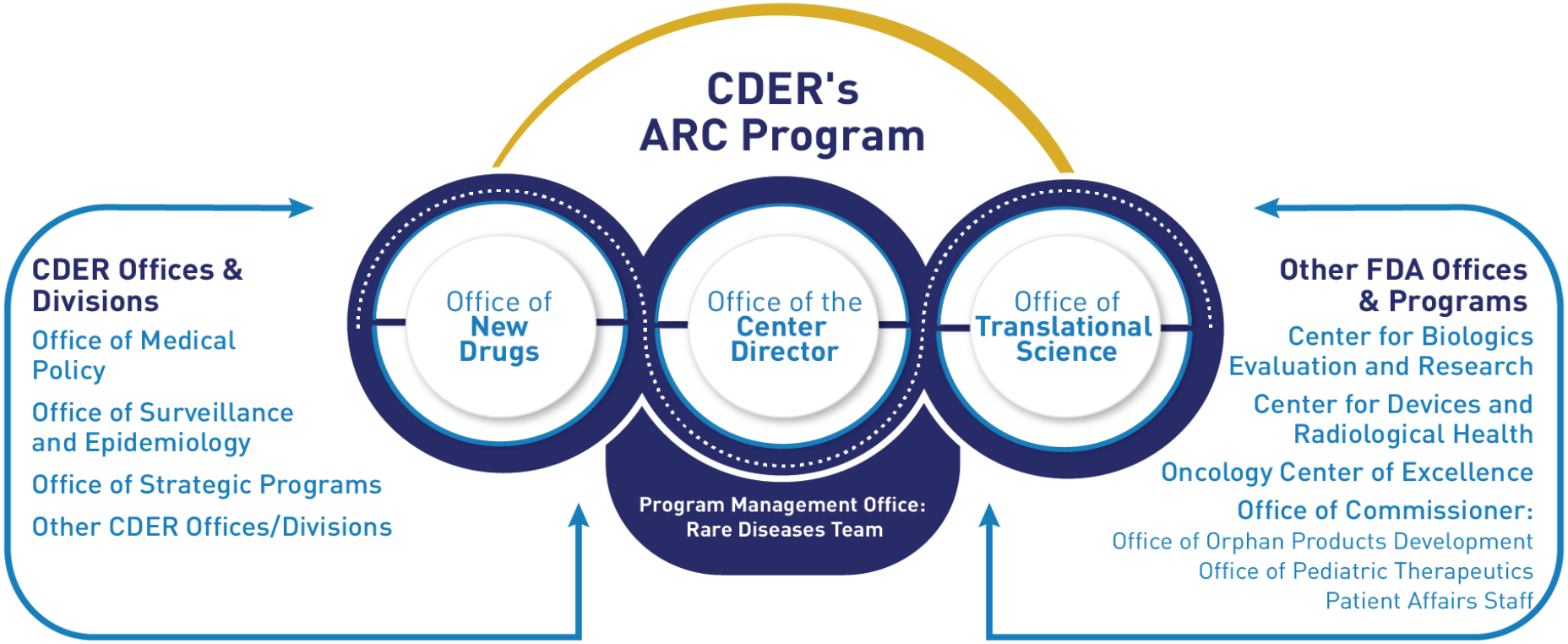
- **Natural history** is often poorly understood
- Diseases are progressive, **serious**, **life-limiting** *and* often lack adequate **approved therapies – urgent needs,** many have **pediatric onset**
- **Small populations** often restrict study design options
- **Phenotypic and genotypic** diversity 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

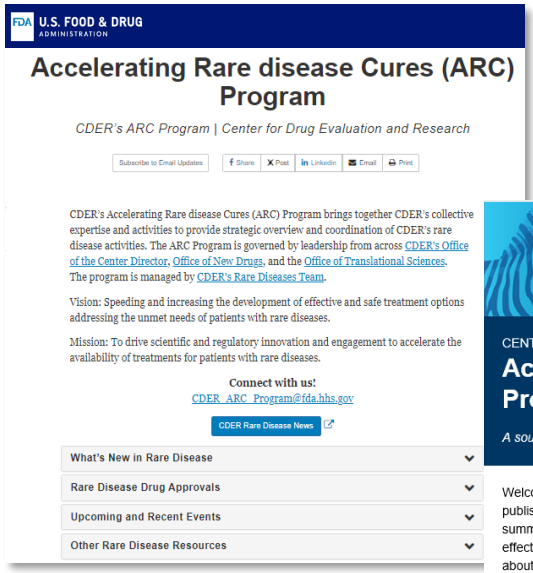
https://www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/cders-arc-program

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



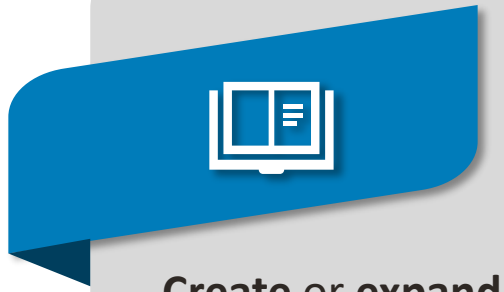
ARC Website

ARC Quarterly Newsletter

What is **LEADER 3D**?

CDER is seeking input from stakeholders who **design or conduct** rare disease drug development programs

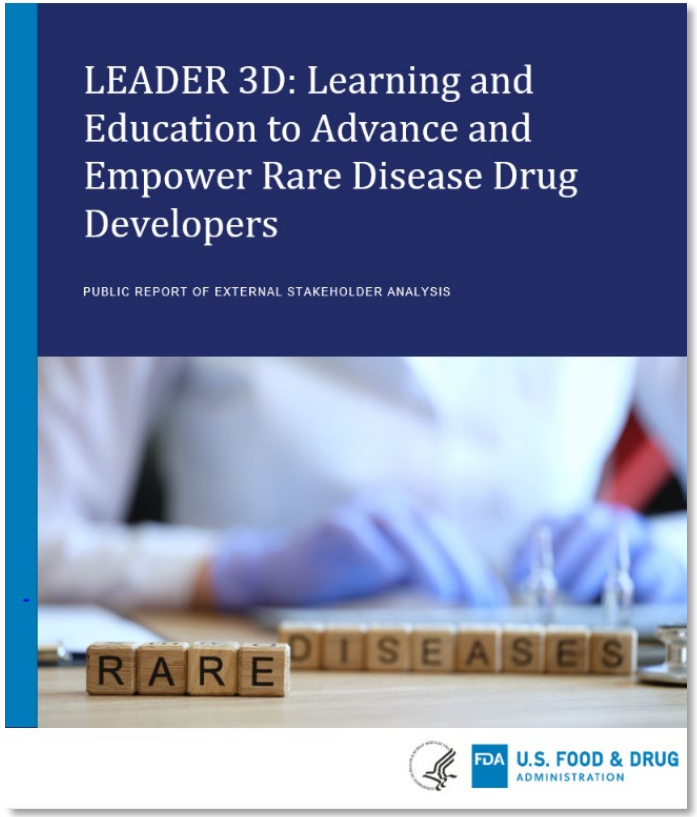
Identify **knowledge gaps** for stakeholders about the regulatory process of rare disease drug development



Create or **expand** educational resources for stakeholders

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

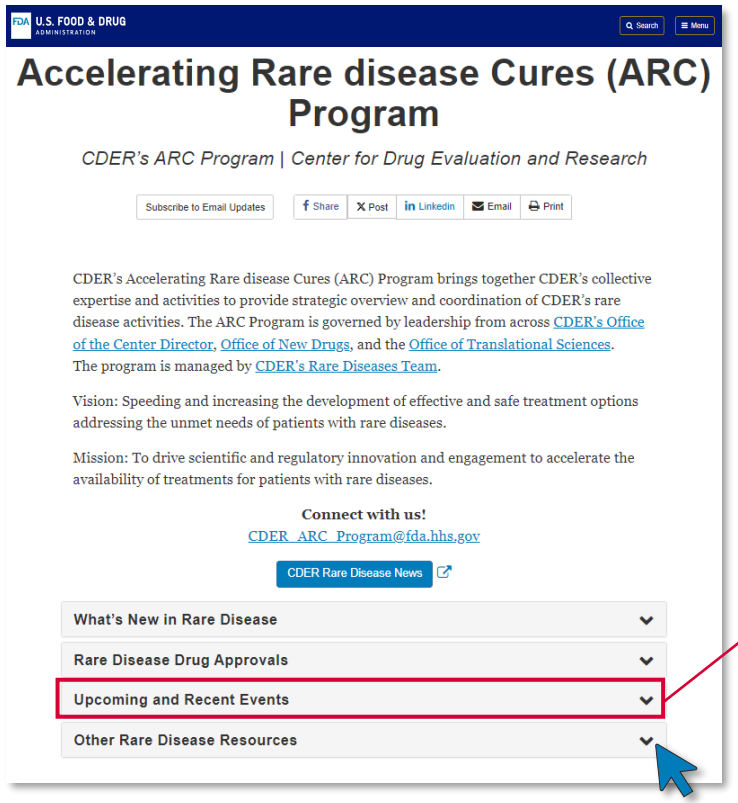
Selected guidances relevant to rare disease drug development organized by topic

Funding Opportunities Tab

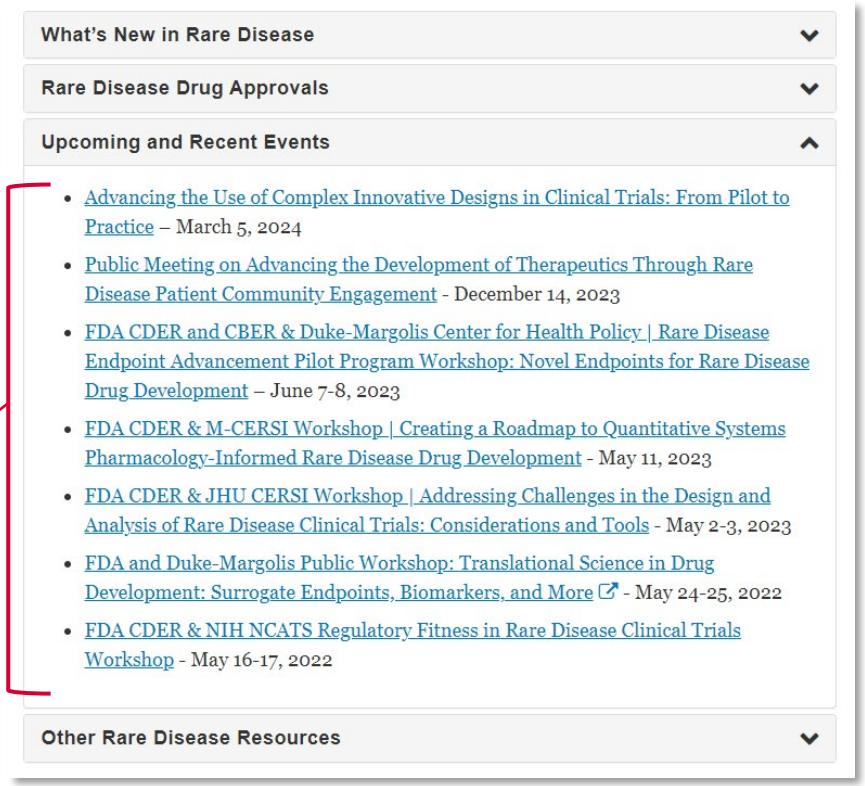
Available funding and fellowship opportunities for rare disease product development research

ARC Website: Conferences and Workshops Tab

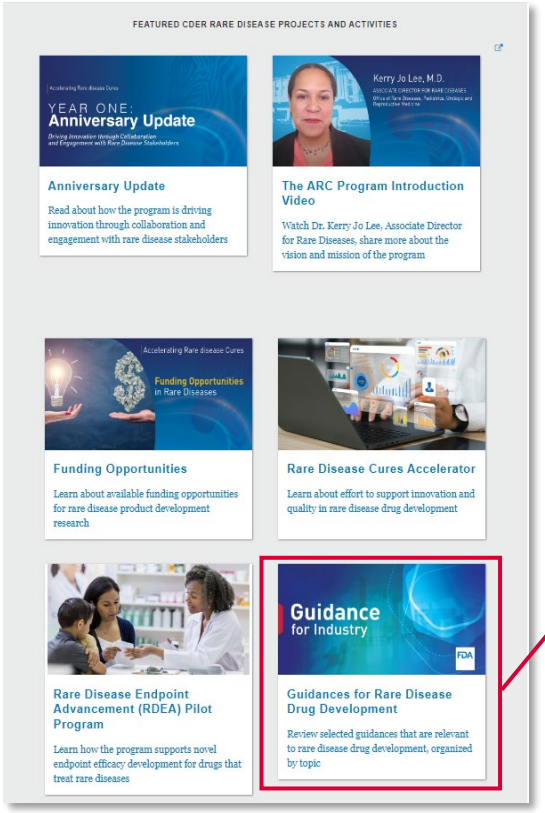
ARC Website



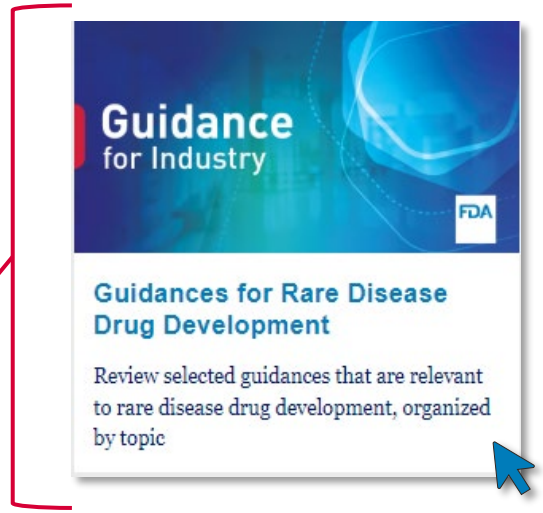
Drop Down Tab



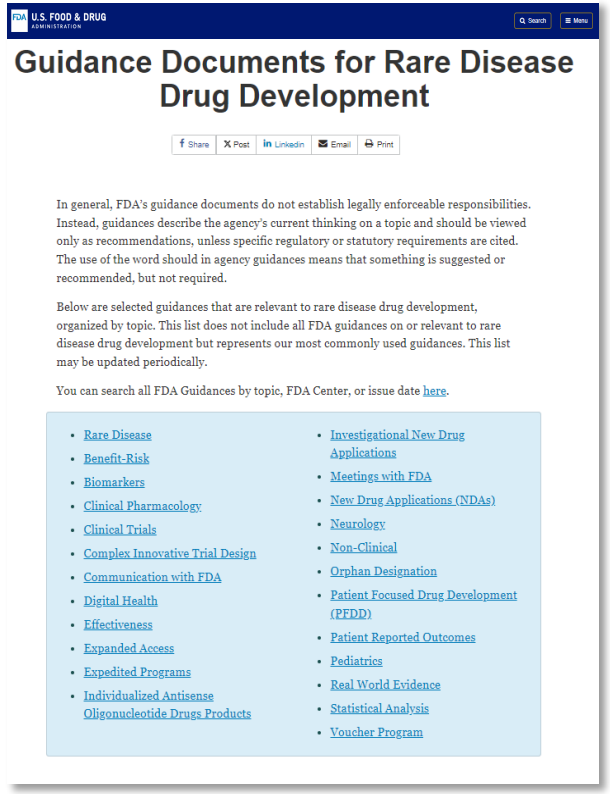
ARC Website: Rare Diseases Guidance Placard



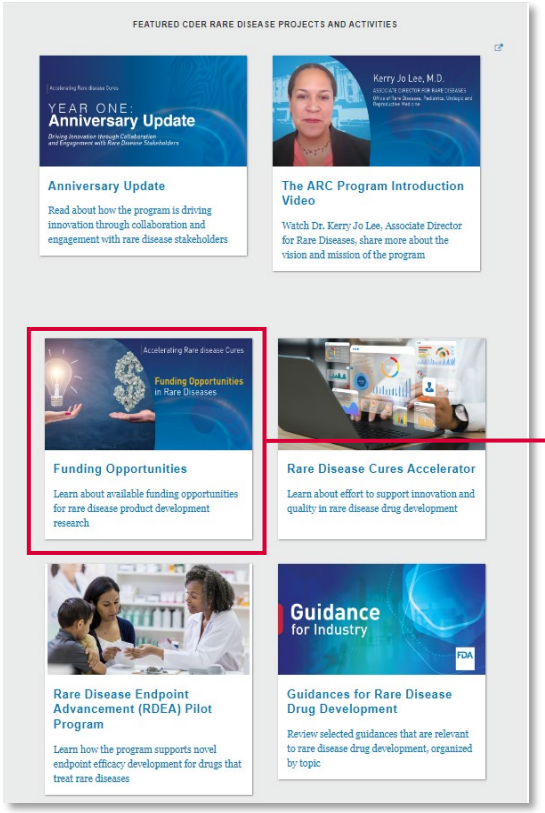
ARC Guidance Placard



Placard Topic Selections

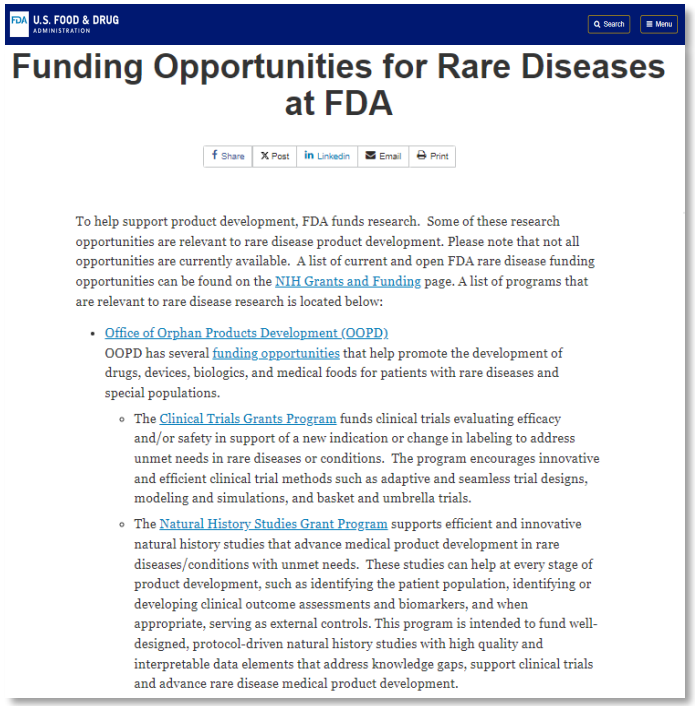


ARC Website: Funding Opportunities Placard



Funding Opportunities Placard

Learn About Funding Opportunities



ARC's Quarterly Newsletter

- To subscribe: U.S. Food and Drug Administration (govdelivery.com)

New Guidance

GUIDANCE DOCUMENT

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

DECEMBER 2023

The Purpose