U.S. FOOD & DRUG ADMINISTRATION

Accelerating Rare disease Cures (ARC) Program

2025 - 2030:

Strategic Plan

Driving Innovation through Scientific and Regulatory Advancement and Engagement

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A Message from the ARC Program Lead

On behalf of the Accelerating Rare disease Cures (ARC) Program, I am pleased to present the Program's 2025-2030 strategic plan. This strategic plan outlines the direction that we will take to support and achieve our mission and vision over the next five years.

Established in 2022, the ARC Program was launched to create strategic coordination for Center for Drug Evaluation and Research's (CDER) rare disease activities. ARC strives to increase the number of rare disease treatments by synergizing the efforts and experience of Centers' and Offices' rare disease initiatives, promoting and leveraging innovative scientific design, developing educational efforts, providing a deeper understanding of regulatory policies, and engaging with the rare disease community.

The ARC Program's strategic plan is based on the mission and vision set forth by CDER's Rare Diseases Team (RDT) and reflects the contributions of many Offices. It aligns with the Department of Health & Human Services (HHS), the Food and Drug Administration (FDA), and CDER priorities. The plan utilizes mission-driven, cross-agency collaboration and engagement, and sets forth the Program's three strategic goals:

1. **Scientific and Regulatory Innovations:** Provide strategic direction and drive the development and use of regulatory and scientific tools to support rare disease drug development.
2. **Engagement and Education:** Engage with the rare disease community to leverage expertise and innovation, strengthen dialogue and understanding of the regulatory framework, and promote effective collaboration.
3. **Operations:** Ensure CDER Staff have the scientific expertise and capacity to foster a culture of innovation to advise, support, and advance successful rare disease drug development.

This strategic plan works from the basis of the goals and outlines objectives, activities, and metrics designed to (1) effectively speed up and increase the development of rare disease treatment options and (2) drive innovation and engagement across the rare disease community to accelerate the availability of treatments to address the unmet needs of patients with rare diseases.

The overall objectives of this strategic plan serve as our north star, but as the ARC Program grows, evolves and sustains, this strategic plan will act as a living document allowing the RDT to work with our governance board to chart a unified path forward for existing ARC programs and initiatives while affording flexibility to modify our direction and approach to respond to rapid advances in rare disease science, agency priorities, and rare disease community needs.

Together, the strategic goals, objectives, and metrics position the ARC Program to contribute to improving public health in the years to come. The ARC Program has clear direction and is looking forward to rising to the challenge of building on our successes to serve the American public.

Sincerely,

Elizabeth Hart, MD
Associate Director for Rare Diseases (Acting)
Rare Diseases Team
Office of New Drugs | Center for Drug Evaluation and Research | FDA

ARC Strategic Plan Overview

The ARC Program's 2025 strategic plan outlines the vision, mission, and programmatic goals for the next five years. This plan details the specific outcomes, expected results, and related strategies on which ARC's leadership and the rare disease community will strive to work collaboratively to advance rare disease drug development and increase access to safe and effective therapies that improve the lives of patients.

The ARC Program's strategic plan is informed by its foundational priorities of alignment and engagement, and leverages collaboration to drive meaningful progress for the rare disease community. The plan presents ARC's core programs and initiatives and ties them to the goals and objectives they support.

This strategic plan provides a comprehensive overview of the ARC Program's current and planned portfolio of initiatives and includes activities intended to accelerate the development of safe and effective treatment options for patients with rare diseases. For each of the ARC goals, this plan maps strategic objectives and key performance indicators (KPIs) to program and initiative efforts and designates success indicators.

As the ARC Program expands and new programs and initiatives are launched, additional KPIs may be added to ensure alignment to ARC's goals and track progress toward defined metrics of success. In anticipation of future activities across all three strategic goals, this strategic plan operates as a living document.

How to Use This Plan

This strategic plan is a tool used to target, unify, and direct ARC's work based on set strategic goals and implementation plans. Within this plan, KPIs measure program progress toward fulfilling ARC's mission. The strategic plan is organized into four sections:

- **CDER's Rare Disease Landscape** provides insight into CDER's work to date addressing key issues in rare disease drug development.
- **ARC's Role within CDER** gives a high-level overview of ARC's program management staff and the key divisions, Offices, Centers, and rare disease community groups that inform ARC's various initiatives, programs, and collaborations.
- **ARC's Strategic Components** shares ARC's vision, mission, and overarching goals and objectives.
- **ARC's Goals and Objectives** defines key strategic objectives for each goal and identifies the KPIs that address opportunities and challenges in the rare disease landscape over the next five years..

As ARC grows and science evolves, opportunities for greater operational efficiencies and deeper engagement with the rare disease community will emerge. In response, ARC will continually evaluate programmatic progress and adjust KPIs and metrics to meet evolving needs and promote regulatory innovation. This living document aims to optimize programmatic impact and advance ARC's strategic goals over five years.

ARC's Maturity Model Growth Strategy

The ARC Program utilizes a maturity model growth strategy (Figure 1). The model provides a four-phase structured framework to assess capabilities, identify areas for improvement, set growth goals, and track progress over time. This maturity model enables ARC leadership to make informed decisions to direct ARC's overarching program strategy, promote sustainable development across its three strategic goals, and manage ARC's suite of programs and activities in their respective locations within the maturity timeline.

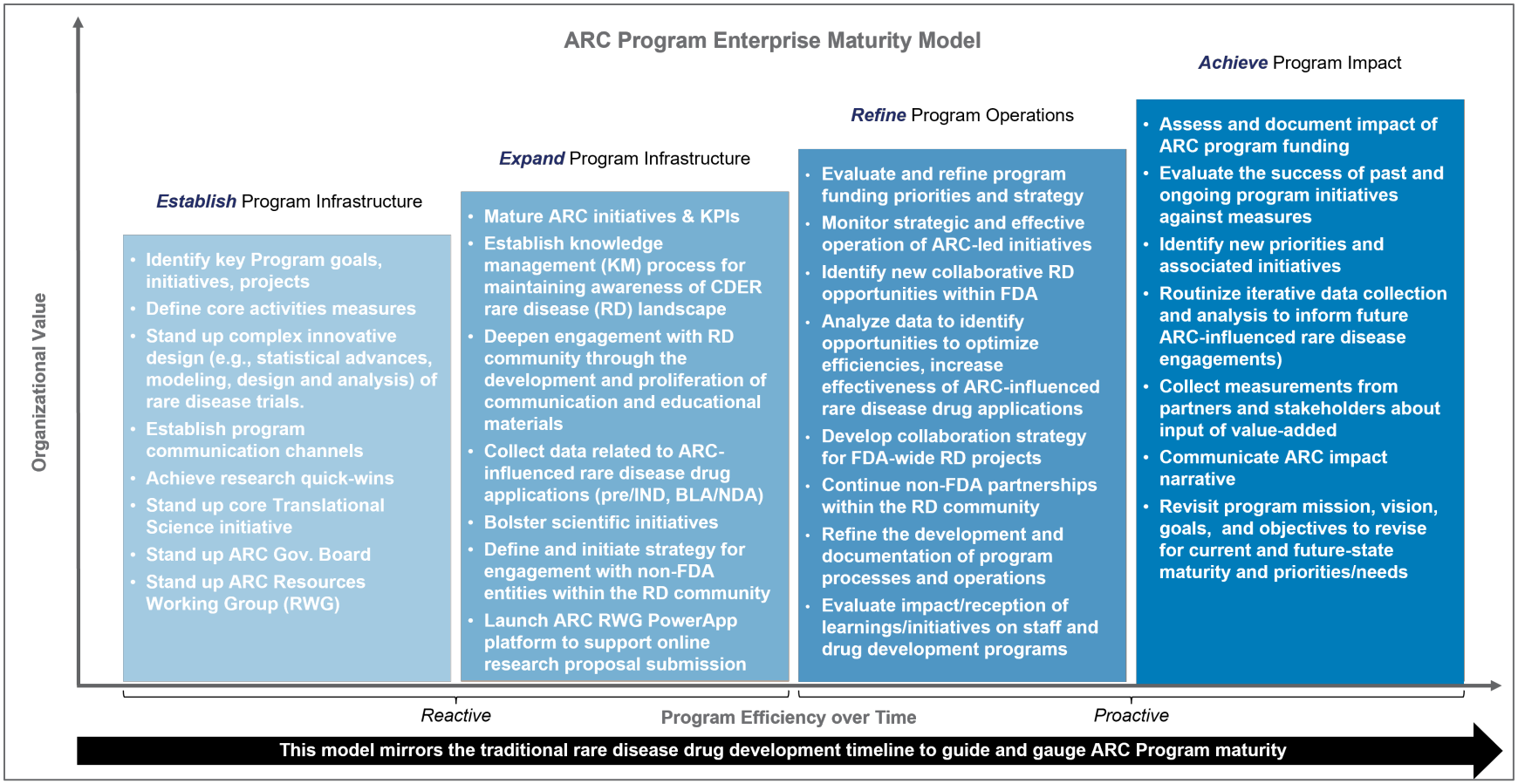


***Figure 1**: ARC's four-phase maturity model provides a structured framework to assess current capabilities, identify areas for improvement, set clear growth targets, and measure progress over time. This growth strategy supports ARC's rapid growth and program expansion while enabling continuous improvement and aligned programmatic efforts across ARC Program. ARC is currently in the Refine stage and is well positioned to enter the Achieve Program Impact stage by 2031, mirroring the 7 – 9-year rare disease drug development timeline.*

At an enterprise level, the ARC Program is assessed against a maturity model. Within ARC, individual initiatives have their own respective maturity models that they are tracked against. This nested system allows ARC leadership to not only track the macro maturity of the Program but also measure micro maturity of initiatives that function as a driver of programmatic growth and success.

ARC's overall growth is visualized via each initiative, tracking each through four distinct maturity stages: *Establish Program Infrastructure, Expand Program Infrastructure, Refine Program Operations,* and *Achieve Program Impact*. Each stage is designed to sustainably and systemically mature ARC's Programmatic functions and operations. This four-stage growth strategy enables ARC leaders to identify best practices, hone processes, and build organizational value over time. The maturity model serves two purposes: primarily, it provides holistic insight into ARC's programmatic operations; secondarily, it guides program launch, management, and growth to serve ARC's mission.

ARC, as an enterprise program[1], entered the *Establish* stage with its founding in 2022. Since then, ARC has met maturity milestone targets for the *Establish* and *Expand* stages. Today, ARC is in the *Refine* stage. The Program is well positioned to enter the *Achieve* stage in 2031, targeting a 2030 assessment of ARC's impact on rare disease drug application and review processes.

The ARC Program is designed to be highly adaptable; initiatives and programs are established on a rolling basis as needs and opportunities arise. ARC's flexible maturity model framework enables a robust and dynamic growth strategy to ease program stand-up and program management efforts across progressive development stages. Over time, ARC's scaling approach sustainably grows ARC's capacity to impact rare disease drug development. By prioritizing resource investment and allocation, developing efficiencies, and meeting maturity milestone targets, the ARC Program is positioned to respond to rare disease drug development issues, capitalize on scientific and regulatory advancements, engage with the rare disease community, and ensure efficient and effective operations to meet our vision.

Figure 2 below depicts the expectations for individual initiatives to be able to move from one stage to the next. The graphic captures the fiscal year 2025, quarter 4 maturity stage for all major ARC initiatives and includes initiatives in which ARC SMEs regularly participate but which are not managed by RDT (ex. Patient Listening Sessions (PLS) and Patient-Focused Drug Development (PFDD) meetings). Though the initiatives are listed under their respective stages, programs are at different points within each stage. Stage lengths are not directly comparable across initiatives, as each varies based on the factors affecting that initiative's development. This variability is by design, allowing each program to progress on an independent timeline that balances sustainable growth with available resources.

All fixed programs with rolling objectives (e.g., legislative and Prescription Drug User Fee Act [PDUFA] reporting tasks) are omitted as they run on established and efficient routines. Oak Ridge Institute for Science and Education (ORISE) and other ARC funded research projects are similarly omitted; as rolling programs with short-term objectives, they are expected to cycle through the entire maturity model within short periods of performance.

[1] Within this strategic plan, ARC is referred to as an "enterprise program" because its scope extends beyond a single initiative or department, enabling common approaches, shared visibility, and aligned planning, reporting, and decision-making across programs, teams, and leadership structures throughout the organization.

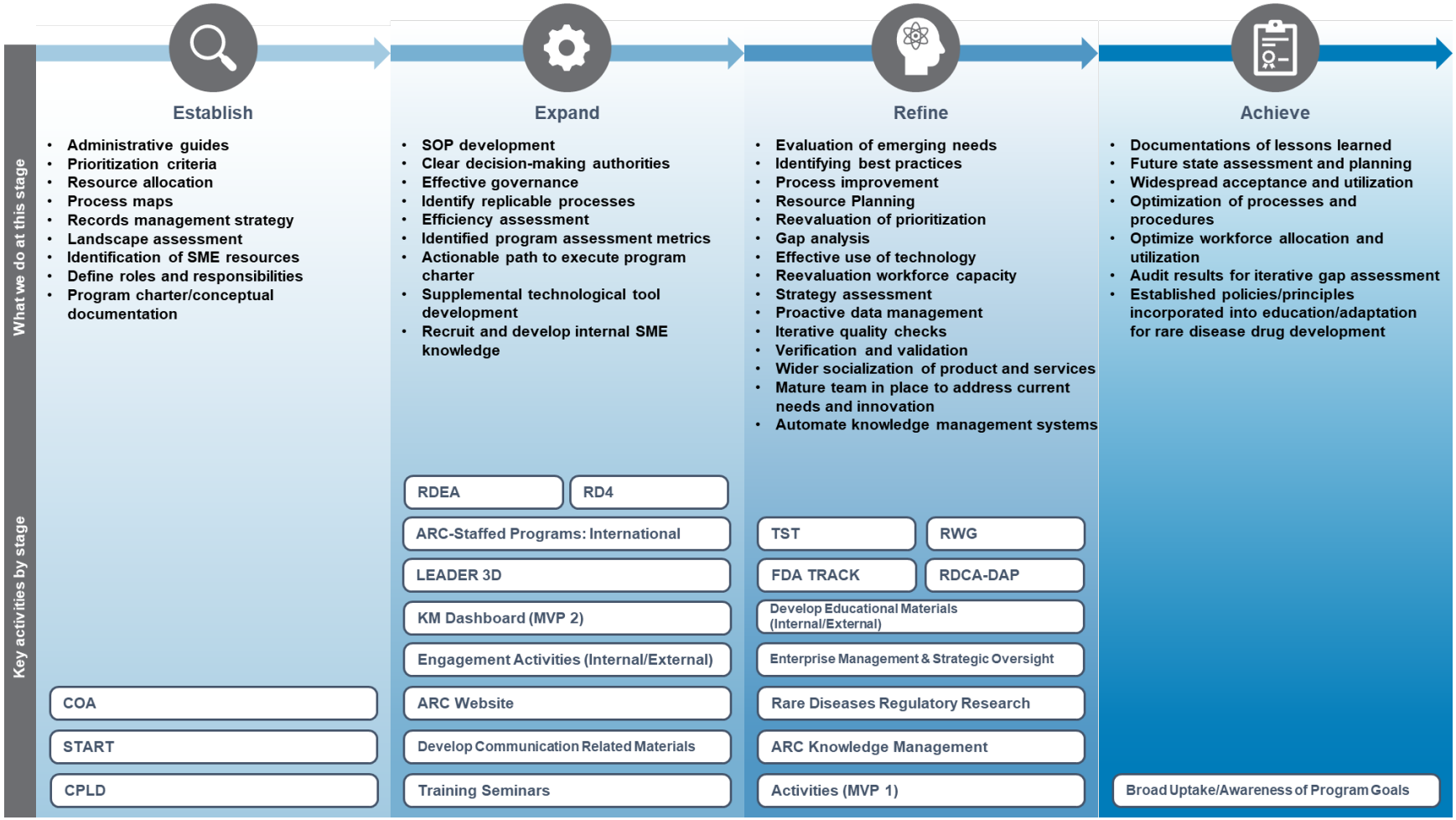


***Figure 2**: ARC's four-phase maturity model as it pertains to the growth of each individual program and initiative.*

CDER's Rare Disease Landscape

Since 2015, CDER has seen an upward trajectory in the percentage of drugs approved to treat rare disease conditions or diseases. In the past five years, CDER has approved 123 orphan new molecular entities, and in 2025 exactly half (23 of 46, or 50%) of CDER's novel drug approvals were for patients with rare diseases. However, despite an increase in novel rare disease approvals, there is still a tremendous unmet need for FDA-approved treatments for rare diseases and conditions.

There are approximately 25 to 30 million Americans living with a rare disease. Drug development for the approximately 10,000 rare diseases and conditions can be complex for many reasons, such as:

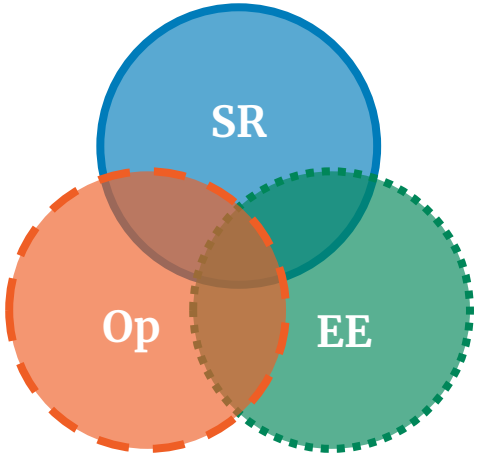
- Small patient populations can make it difficult to design, perform, and interpret rare disease clinical trials.
- Genotypic/phenotypic heterogeneity within a disease.
- Heterogeneous disease characteristics (clinical manifestations or disease progression).
- Limited understanding of the disease's natural history to inform trial design including trial duration, patient population, or endpoint selection.
- Lack of drug development tools including outcome measures and biomarkers.
- Lack of precedent, including clinically meaningful endpoints, for drug development especially with the limited understanding of a rare disease's natural history.

These gaps are sometimes exacerbated by limited resources, geographically dispersed patient populations, data quality issues, inexperience with regulatory requirements or clinical trial development, and the siloed nature of individual drug development programs.

As a result, there is significant unmet need for the many patients with rare diseases that have no available treatments. ARC strives to increase the number of safe and effective rare disease drugs by promoting innovative scientific design, providing a deeper understanding of regulatory policies, and engaging in dialogue with the rare disease community.

ARC's suite of programs and initiatives supports FDA's rare disease drug development work, leading the development of cross-cutting programs, guidances, and engagement on rare disease policies and practices across CDER.

The graphic on the following page (Figure 3) aligns ARC's programs and initiatives by primary purpose to one of ARC's three strategic goals. Because many programs inform and build upon each other, several have cross-cutting purposes that span multiple goals; Figure 3 uses icons to denote this overlap. These programs are highlighted in this strategic plan, with metrics linked to multiple strategic goals.



Scientific & Regulatory Innovations

- Translational Science Team (TST)
- Rare Disease Drug Development Design (RD4)
- Rare Diseases (RD) Clinical Outcomes Assessment (COA)
- **Rare Disease Regulatory Research**
  - ORISE Research Fellowship
  - Extramural Research – Broad Agency Announcement (BAA)
- **ARC-Led Initiatives**
  - Rare Disease Endpoint Advancement (RDEA)[1]
  - Support for clinical Trials Advancing Rare disease Therapeutics (START) Pilot Program
- **ARC-Staffed Programs: International**
  - Rare Disease International Cluster

Operations

- Enterprise Management and Strategic Oversight (Governance Board RDT leadership, and Resource Working Group (RWG))
- Information Technology and Knowledge Management
- Program Management

Engagement & Education

- **External EE Activities**
  - Learning and Education to ADvance and Empower Rare Disease Drug Developers (LEADER 3D)
  - Critical Path Institute (C-Path)
    - Rare Disease Cures Accelerator-Data Analytics Platform (RDCA-DAP)
    - Critical Path for Lysosomal Diseases (CPLD)
  - Conferences and Workshops
  - Drug and Biologics Transparency Results Accountability Credibility Knowledge Sharing (FDA TRACK)
  - ARC Website
  - Social Media
  - News Publications & Annual Report
  - Guidance Documents by topic
  - PFDD Meetings
  - PLS
- **Internal EE Activities**
  - LEADER 3D Case Studies
  - Rare Disease Trainings, Workshops, and Seminars
  - Zebragram Newsletter

ARC's Role within CDER

The ARC Program brings together CDER's collective expertise and activities to provide strategic overview and enhance collaboration between CDER's rare disease activities across its Offices (Figure 4). ARC is governed by leadership from across three of CDER's Super-Offices: Office of the Center Director (OCD), Office of New Drugs (OND), and the Office of Translational Sciences (OTS).

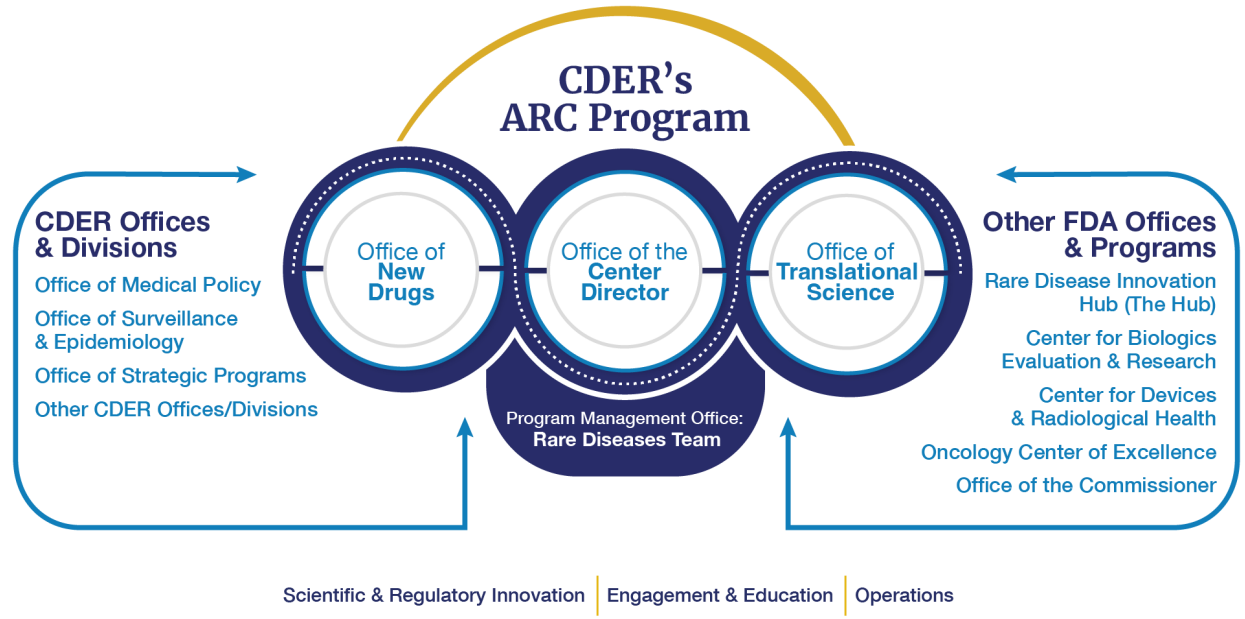


Figure 4: *CDER established the ARC Program in May 2022 to provide strategic overview and coordination of the Center's rare disease activities.*

The ARC Program is managed by CDER's RDT. The RDT is a multi-disciplinary team that connects CDER's rare disease initiatives across the OCD, OND, and OTS. The RDT, which carries out the operations and program management for ARC, resides within in the Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine (ORPURM). Within ORPURM, RDT falls under the Division of Rare Diseases and Medical Genetics (DRDMG). RDT's core activities include:

- Leading cross-cutting OND rare disease programs, guidances, policies, strategic research, retreats, and workshops.
- Providing advice based on guidance and knowledge management (KM) to review divisions on their rare disease programs.
- Developing and providing rare disease training and education.
- Engaging with internal and external members of the rare disease community.

Since the Program's launch in May 2022, the ARC Program has built upon and expanded CDER's existing capabilities to enhance collaboration across the Center to drive innovation in domains such as endpoints or biomarkers; quantitative methods; drug and disease modeling; clinical trial design; clinical outcome assessments; real world data platforms such as the RDCA-DAP; regulatory frameworks; and public engagement.

For cross-cutting rare disease considerations, the ARC Program connects other FDA Centers, Offices, and programs, such as the Center for Biologics Evaluation and Research (CBER), the Center for Devices and Radiological Health (CDRH), the Oncology Center of Excellence (OCE), and the Office of Orphan Products Development (OOPD) within the Office of the Commissioner (OC), to promote strong collaborations to advance treatment options for rare diseases and conditions.

The ARC Program closely partners with FDA's Rare Disease Innovation Hub (the Hub) to enhance collaboration across the Agency and address common scientific, clinical, and policy issues related to rare disease product development. ARC's strategic goals align with the Hub's mission of improving outcomes for rare disease patients. Through its work with the Hub, ARC supports CDER's and CBER's joint commitment to enhance and strengthen cross-center alignment to promote consistency in our approaches to advancing rare disease therapeutic development.

ARC's relationship with the Hub is both cooperative and mutually beneficial: the ARC Program's commitment to enhance to speed and increase effective treatment options for patients with rare diseases informs and advances the Hub's central mission and overarching goals. In turn, the Hub's work to advance the regulatory science of rare disease therapies, enhance coordination and alignment between medical product centers, and create a primary point of contact for rare disease community engagement informs and advances ARC's overarching goals and objectives across its strategic components.

The ARC Program identifies three areas of close alignment between its strategic goals and the Hub's strategic agenda (Rare Disease Innovation Hub Strategic Agenda 2025). Specifically, many of ARC's strategic goals and objectives within the Scientific and Regulatory Innovations component complement the Hub's goals to further advance regulatory science of rare disease therapies and enhance and strengthen coordination and alignment between medical product centers, (Hub Goals 1 and 2). ARC's Engagement and Education goals and objectives seek to strengthen and deepen connections between and across the rare disease community and can be leveraged to support the Hub's role as a centralized point of contact for external partners (Hub Goal 3).

Looking ahead, ARC will continue to connect CDER's key leadership and support CDER review staff with programs and tools to support their work on rare disease therapeutic development as they bridge the gap between the complexities of rare disease drug development and the pressing needs of patients. ARC welcomes the strengthening of current and future opportunities to partner with the Hub and other Agency Centers, offices, and the rare disease community to foster communication and leverage an array of expertise to accelerate the development of available treatment options for patients. The current challenges within rare disease drug development are multifaceted and engagement across the rare disease community is critical to achieve success.

ARC's Strategic Components

Vision

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

Mission

CDER's ARC Program drives scientific and regulatory innovation and engagement to accelerate the availability of treatments for patients with rare diseases.

Strategic Goals

The ARC Program coordinates, facilitates, and supports both new innovations and the enhancement of current initiatives within CDER. Through each of ARC's goals, the Program directs, coordinates, and manages a variety of activities and initiatives that directly correlate to the Program's objectives by contributing to the acceleration of rare disease drug development. ARC's goals are organized into three operational governances aimed at their core functions.

Scientific and Regulatory Innovations

Provide strategic direction and drive the development and use of regulatory and scientific tools to support rare disease drug development.

Engagement and Education

Engage with the rare disease community to leverage expertise and innovation, strengthen dialogue and understanding of the regulatory framework, and promote effective collaboration.

Operations

Ensure CDER staff have the scientific expertise and capacity to foster a culture of innovation to advise, support, and advance successful rare disease drug development.

Scientific and Regulatory Innovations Goal and Objectives

Goal

Provide strategic direction and drive the development and use of regulatory and scientific tools to support rare disease drug development.

ARC coordinates, facilitates, and supports both new innovations and the enhancement of current initiatives within CDER by providing strategic direction and driving the development and use of tools to support rare disease drug development. This work is addressed through *scientific and regulatory innovations* (S.R.) strategic objectives.

Strategic Objectives[2]

1. Provide advice for earlier application and expanded efforts in translational medicine approaches for individual rare disease programs to improve development of confirmatory evidence (CE) and/or surrogate endpoints in rare diseases.
2. Expand the development and use of innovative designs and Bayesian statistical approaches to small population, slowly progressive rare disease programs.
3. Support platforms to facilitate Natural History studies that can inform rare disease drug development (such as through informing trial design, endpoint selection, and external controls).
4. Develop, test, and validate methodologies to construct novel endpoints that can capture meaningful clinical change for patients with rare disease (including novel biomarkers, digital health technologies, multi component endpoints, and Clinical Outcome Assessments (COAs)).
5. Apply consistent, agile, and appropriately flexible approaches in the application of the regulatory framework to rare disease drug development programs.
6. Provide strategic direction and drive the development and use of regulatory and scientific tools to support rare disease drug development.
7. Expand the utilization of drug /disease modeling and design-by-simulation approaches.[3]
8. Explore and establish efficient approaches to dose-selection for drugs for small population diseases, such as the use of advanced pharmacometrics and other quantitative approaches.[4]

In pursuit of these objectives, the ARC Program manages various programs and initiatives to foster connections between FDA's scientific and regulatory innovation initiatives, connect internal and external subject matter experts (SMEs), and leverage rare disease research. Examples of programmatic activities may include:

- Supporting platforms (e.g. RDCA-DAP) and other mechanisms (e.g. research) that facilitate natural history studies for rare diseases.
- Developing, testing, and validating methodologies to construct novel endpoints.
- Expanding the utilization of drug/disease modeling.
- Establishing efficient approaches to dose-selection for drugs for small population diseases.
- Broadening efforts in translational medicine approaches for individual rare disease programs.
- Providing subject matter expertise via consulting support for review teams.

[2] Strategic objectives are dynamic and can be reassessed and revised by the governance board according to Center priorities and evolving needs of the rare disease community.
[3] Though the ARC Program does not have activities supporting this initiative, ARC identifies this issue as a future area of expansion.
[4] Though the ARC Program does not have activities supporting this initiative, ARC identifies this issue as a future area of expansion.

Engagement and Education Goal and Objectives

Goal

Engage with the rare disease community to leverage expertise and innovation, strengthen dialogue and understanding of the regulatory framework, and promote effective collaboration.

ARC's *engagement and education* initiatives seek to engage with the rare disease community to leverage expertise and innovation, strengthen dialogue, deepen patient and patient advocates' understanding of the regulatory framework, and promote effective collaboration. This collaboration is a top priority for ARC as input from patients living with rare diseases is crucial throughout the drug development process. This work is addressed through *engagement and education* (E.E.) strategic objectives.

Strategic Objectives[5]

1. Collaborate with external academic partners to promote deeper understanding among the relevant rare disease community of the regulatory requirements for rare disease drug approval.
2. Strengthen the dialogue and engagement with patient groups and organizations and actively solicit their needs and perspectives.
3. Ensure CDER and ARC have the expertise and/or capacity to foster a culture of innovation to advise, support, and advance successful rare disease drug development.
4. Coordinate with domestic and international partners and regulatory bodies on common issues and scientific approaches to rare disease drug development.
5. Collaborate within and across Centers, when appropriate, to provide timely and relevant reviewer training and education on scientific issues and approaches to rare disease drug development.

In pursuit of these objectives, ARC leverages strategic partnerships with the rare disease community, including non-profit centers, industry leaders, research institutions, Federal partners, and international health agencies. Through these collaborations and partnerships, the ARC Program can produce effective and timely conferences, workshops, and other events responsive to the needs of the rare disease community to foster rare disease drug development best positioned to meet regulatory considerations. Communication mechanisms also enable timely and evergreen access to information about CDER rare disease news, initiatives, and educational materials.

[5] Strategic objectives are dynamic and can be reassessed and revised by the governance board according to Center priorities and evolving needs of the rare disease community.

Operations Goal and Objectives

Goal

Ensure CDER Staff have the scientific expertise and capacity to foster a culture of innovation to advise, support, and advance successful rare disease drug development.

To accomplish mission-critical initiatives, the ARC program must have an aligned strategy and organizational accountability. To strive for strategic excellence and support ARC's suite of programs and initiatives, ARC engages CDER leadership for programmatic oversight, leverages technological tools to increase efficiency and centralize information, and directs RDT to develop and provide program support. This work is addressed through *operations* (O.P.) strategic objectives.

Strategic Objectives[6]

1. Manage resources to advance ARC's mission and address rare disease community needs.
2. Develop and expand the use of technological solutions to facilitate collaboration, streamline operations, track process, manage data, and communicate information to support rare disease drug development.
3. Capture end to end program management efforts (knowledge management systems, process maps, review structures, governance documents, etc.) to support program goals and strategic objectives.

In pursuit of these objectives and in accordance with RDT's strategic role for CDER, ARC leverages RDT resources to sustain resource management and make data-driven decisions to ensure responsible stewardship and Programmatic accountability. ARC's *operations* initiatives proactively communicate ARC's strategic priorities and initiative-specific activities to the rare disease community while prioritizing resource efficiency and management and modernizing knowledge management tools to meet the Programs' strategic priorities.

[6] Strategic objectives are dynamic and can be reassessed and revised by the governance board according to Center priorities and evolving needs of the rare disease community.

Appendix I: Acronyms

Abbreviation	Definition
ARC	Accelerating Rare disease Cures
AWC	Adequate and Well-Controlled
BA	Budget Authority
BAA	Broad Agency Announcement
BLA	Biologics License Application
C3TI	CDER Center for Clinical Trial Innovation
CBER	Center for Biologics Evaluation and Research
CDER	Center for Drug Evaluation and Research
CDRH	Center for Devices and Radiological Health
CE	Confirmatory Evidence
CERSI	Centers of Excellence in Regulatory Science and Innovation
CID	Complex Innovative trial Design
COA	Clinical Outcome Assessment
C-Path	Critical Path Institute
CPLD	Critical Path for Lysosomal Diseases
CR	Complete Response
DIA	Drug Information Association
DRDMG	Division of Rare Diseases and Medical Genetics
E.E.	Engagement and Education
EMA	European Medicines Agency
FDA	Food and Drug Administration
FDORA	Food and Drug Omnibus Reform Act
IND	Investigational New Drug
IO	Immediate Office
KM	Knowledge Management
KPI	Key Performance Indicator
LEADER 3D	Learning and Education to Address and Empower Rare Disease Drug Developers
MIDD	Model-Informed Drug Design
MVP	Minimal Viable Product
NCATS	National Center for Advancing Translational Science
NDA	New Drug Application
NIH	National Institutes of Health
NORD	National Organization for Rare Disease
O.P.	Operations
OB	Office of Biostatistics
OBI	Office of Business Informatics
OC	Office of the Commissioner
OCD	Office of the Center Director
OCE	Oncology Center of Excellence
OCP	Office of Clinical Pharmacology
OND	Office of New Drugs
OOPD	Office of Orphan Products Development
OPO	Office of Program Operations
ORISE	Oak Ridge Institute for Science and Education
ORO	Office of Regulatory Operations

Abbreviation	Definition
ORPURM	Office of Rare Diseases, Pediatrics, Urologic, and Reproductive Medicine
OTS	Office of Translational Sciences
PDUFA	Prescription Drug User Fee Act
PFDD	Patient-Focused Drug Development
PLS	Patient Listening Sessions
QM CoE	Quantitative Medicine Center of Excellence
RD4	Rare Disease Drug Development Design
RDA	Rare Disease Analytics
RDCA – DAP	Rare Disease Cures Accelerator – Data Analytics Platform
RDEA	Rare Disease Endpoint Advancement
RDT	Rare Diseases Team
RWE	Real World Evidence
RWG	Resources Working Group
S.R.	Scientific and Regulatory Innovations
SMEs	Subject Matter Experts
START	Support for clinical Trials Advancing Rare disease Therapeutics
TRACK	Transparency-Results-Accountability-Credibility-Knowledge Sharing
TST	Translational Science Team