U.S. FOOD & DRUG ADMINISTRATION

Welcome from the FDA

EL-PFDD Meeting on Ehlers-Danlos syndrome (EDS) and hypermobility spectrum disorders (HSD)

October 31, 2023

CDER's Rare Diseases Team

- **Mission**: To facilitate, support, and accelerate the development of drugs and therapeutic biologics for rare diseases
- A multi-disciplinary team located in the Division of Rare Diseases and Medical Genetics in ORPURM

- **Select activities**:
  - Providing advice to review divisions on their rare disease programs
  - Promoting rare disease considerations across CDER's Office of New Drugs (OND)
  - Leading cross-cutting OND rare disease guidance documents, policies, strategic research, and workshops
  - Developing rare disease training and education
  - Engaging with internal and external stakeholders

CDER'S Accelerating Rare disease Cures Program

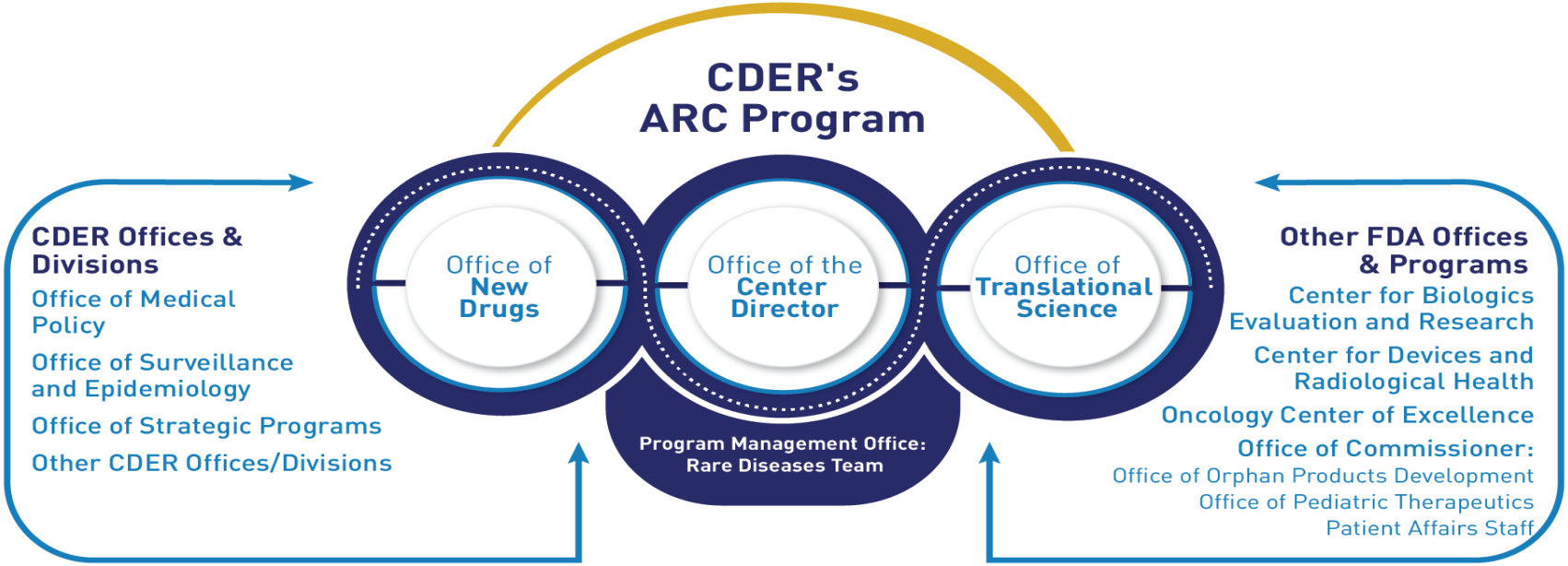
- Vision

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

- Mission

CDER's Accelerating Rare disease Cures (ARC) Program drives scientific and regulatory innovation and engagement to accelerate the availability of treatments for patients with rare diseases.

CDER's Accelerating Rare disease Cures Program



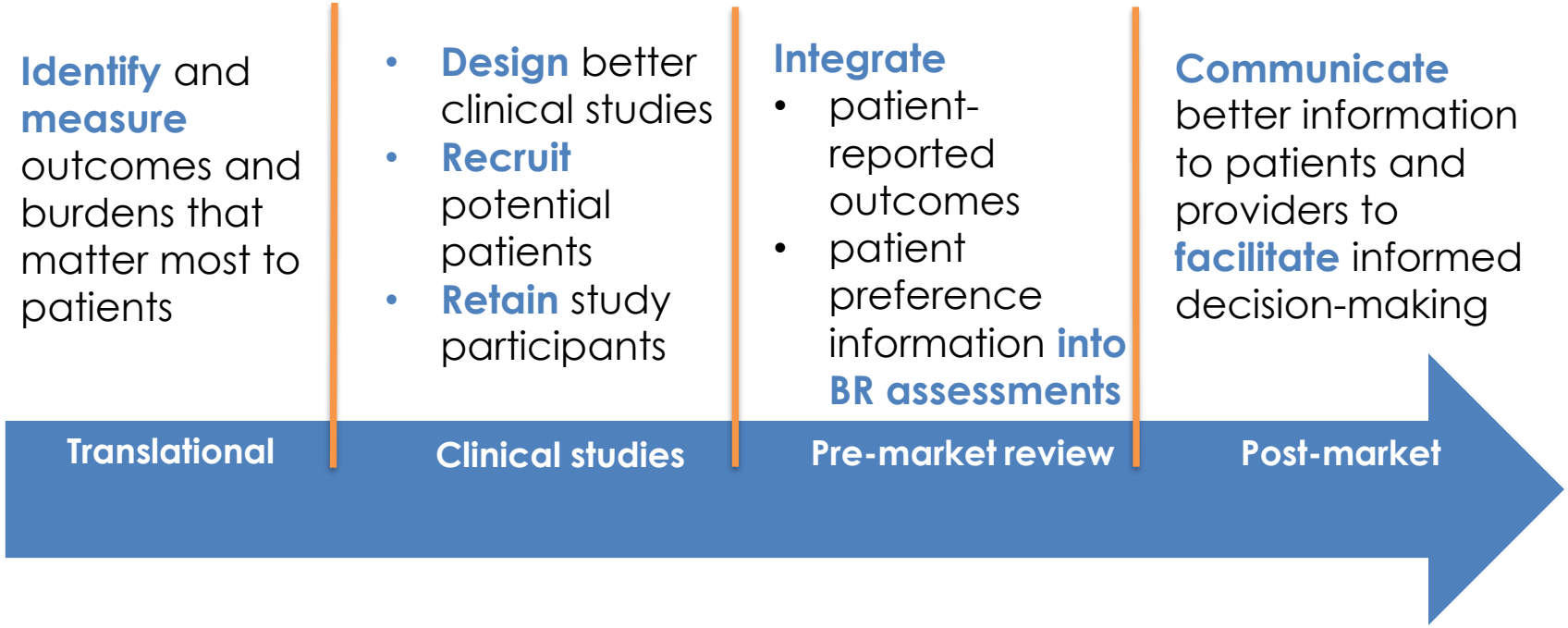
CDER_ARC_Program@fda.hhs.gov

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

Patient-focused drug development (PFDD) is a systematic approach to help ensure that **patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated** into drug development and evaluation.

Source: FDA's Patient-focused Drug Development Glossary (https://www.fda.gov/Drugs/DevelopmentApprovalProcess/ucm579400.htm)

Further integrating patient input into drug development and decision making – life cycle approach



Need to build in the patient's perspective starting in the translational phase

Meetings **Strengthen Understanding** of Disease and Treatment Burden

Patient input from meetings can support FDA staff:

- In conducting benefit-risk assessments for products under review, by informing the therapeutic context
- Advising drug sponsors on their development programs

It might also support drug development more broadly:

- Identify areas of unmet need in the patient population
- Identify or develop tools that assess benefit of potential therapies
- Raise awareness and channel engagement within the patient community

Meeting summary reports capturing patient experience data may be shared on FDA's website:

- FDA's *External Resources or Information Related to Patients' Experience* webpage provides links to certain publicly available external reports and resources

Thank You!

Contact Us

Visit CDER's Patient-Focused Drug Development Homepage

Contact CDER Patient-Focused Drug Development Program Staff at:

patientfocused@fda.hhs.gov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