U.S. FOOD & DRUG ADMINISTRATION

Oncology Center of Excellence

FDA Drug Topics:

An Overview of the Oncology Expanded Access Program

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Health Scientist

Disclosure Information

- Nothing to disclose

Learning Objectives

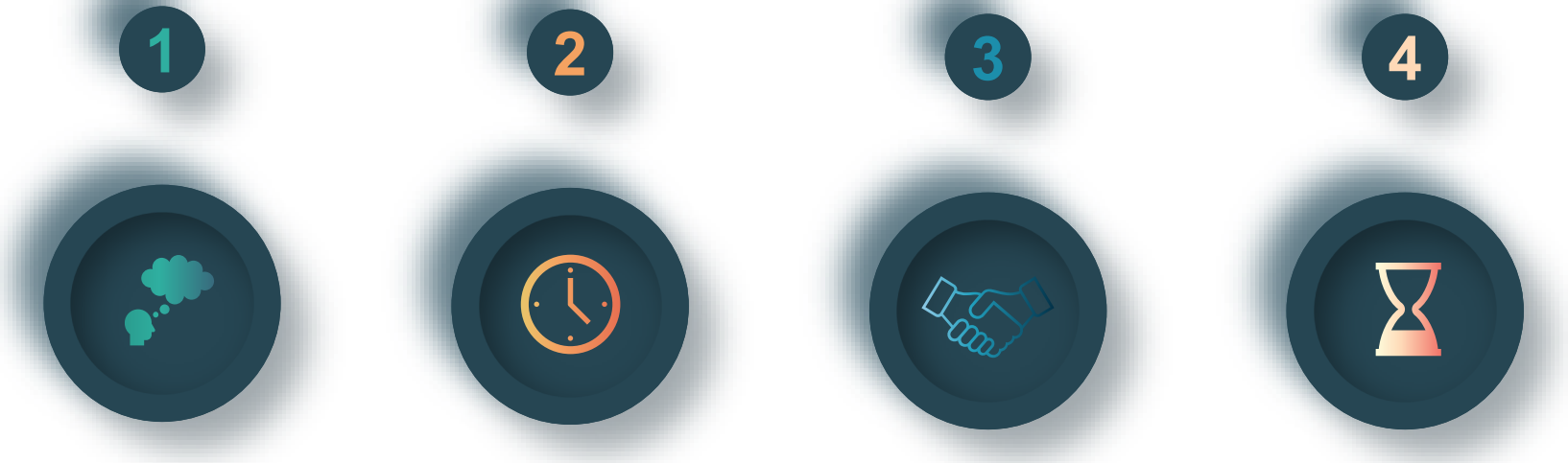
1. Define expanded access and the key requirements for an expanded access request for an individual patient.

2. Summarize the key responsibilities of the oncology healthcare professional when considering expanded access for a patient.

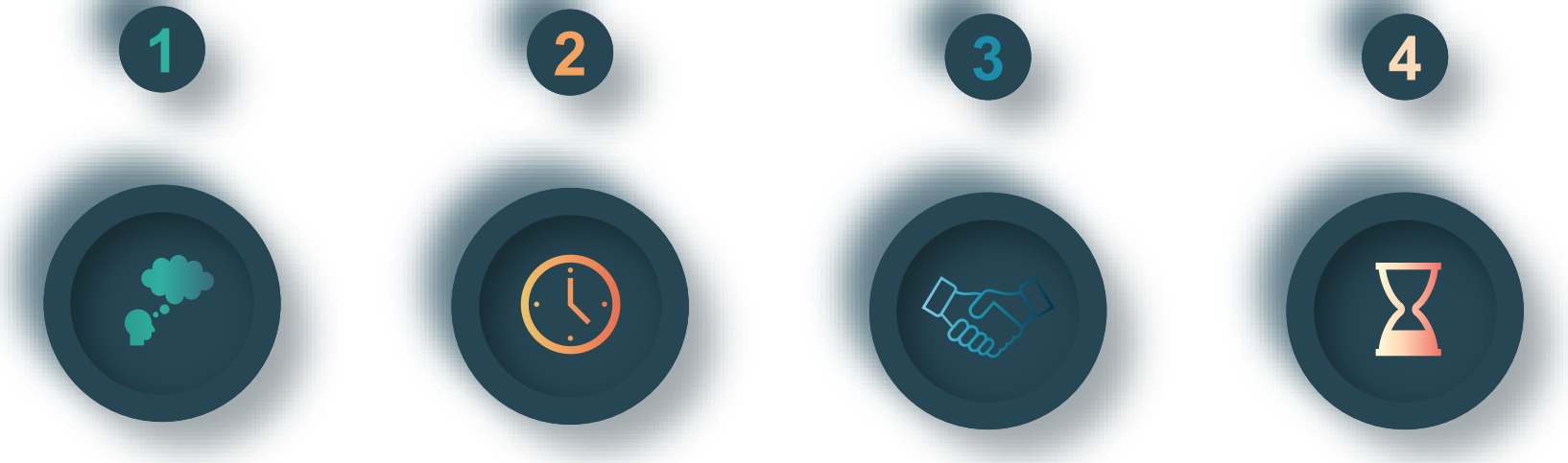
3. Identify resources available to healthcare professionals considering submission of an oncology expanded access request for an individual patient.

4. Describe how Project Facilitate is a resource to navigate the oncology Expanded Access pathway

Misconceptions



Misconceptions



Misconceptions

Myth: I'm not an investigator so FDA will never approve our request for expanded access.

Fact: *An oncologist does not have to be a researcher or belong to a research institution to utilize expanded access.*

Misconceptions

2

Myth: Expanded Access (EA) is inaccessible because the oncologist I work with is too busy to do the paperwork and we don't have a regulatory office

Fact: *Project Facilitate (PF) works with any regulatory or healthcare professional through the process, helps you with filling out forms, and walks you through the EA process*

Misconceptions

3

Myth: Most drug companies don’t participate in EA

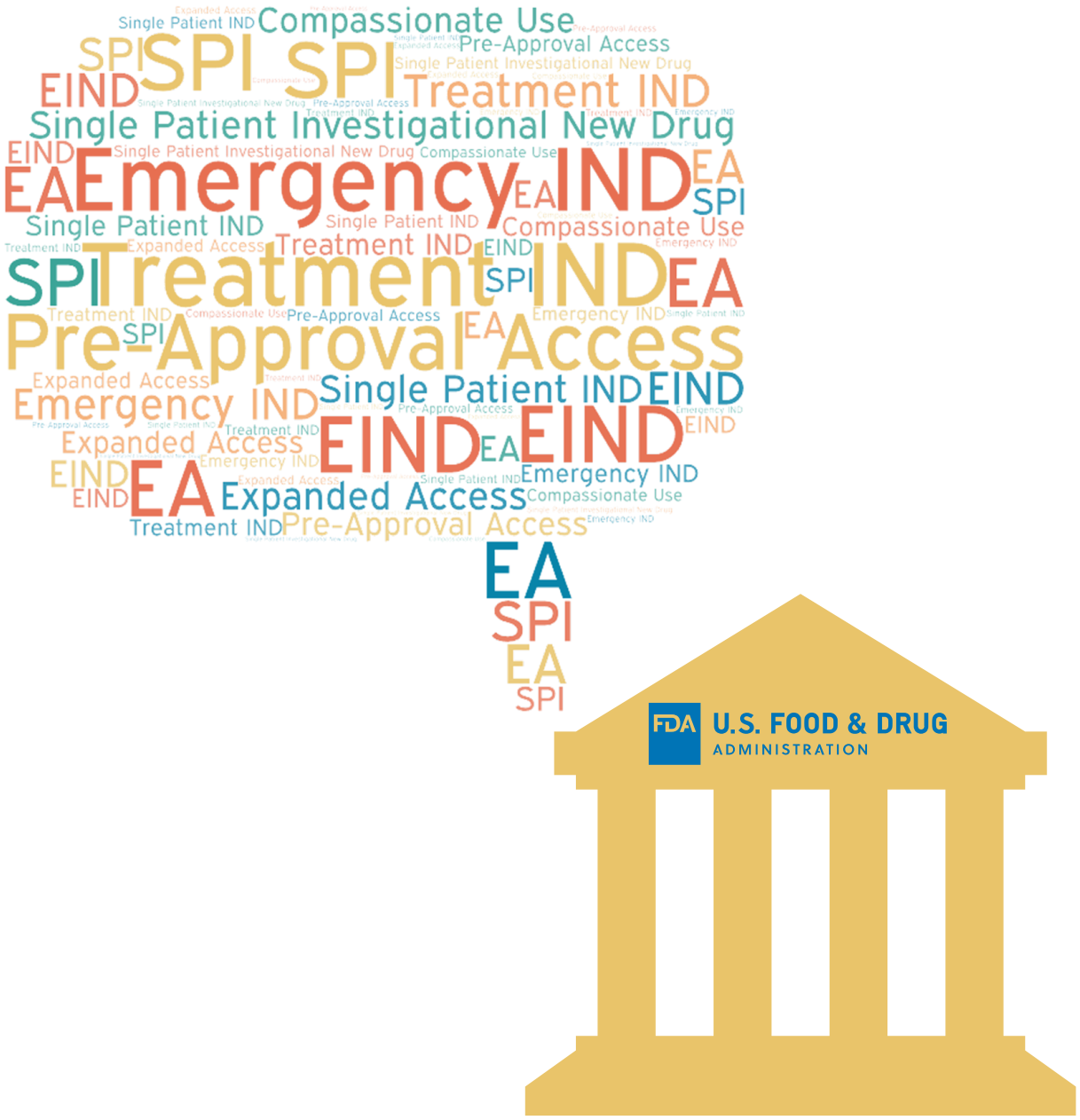
Fact: *Many drug companies participate in EA and are required to make a policy regarding expanded access readily available.*

Misconceptions

4

Myth: It takes too long to get the FDA to grant my request

Fact: *Review times are very fast for CDER oncology products that Project Facilitate reviews. A "granted" letter is usually sent within one day for both emergency and non-emergency requests*


Single Patient IND
Compassionate Use
SPI
SPI
SPI
Pre-Approval Access
Single Patient Investigational New Drug
EIND
Treatment IND
Single Patient Investigational New Drug
EIND
Single Patient Investigational New Drug
Compassionate Use
EA
Emergency
EA
IND
EA
SPI
Single Patient IND
Single Patient IND
Compassionate Use
Treatment IND
Expanded Access
Treatment IND
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Expanded Access
Single Patient IND
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Emergency IND
Pre-Approval Access
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Treatment IND
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EA
Expanded Access
Single Patient IND
Emergency IND
Compassionate Use
Treatment IND
Pre-Approval Access
EA
SPI
EA
SPI
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What is Expanded Access?

- Use of an investigational medical product to **treat a patient** with a serious disease or condition who does not have comparable or satisfactory alternative therapies to treat the disease or condition

- Contrast with investigational medical product in a clinical trial where the **primary intent is research**
  - systematic collection of data with the intent to analyze it to learn about the investigational medical product

Access to Treatments

Approved Drugs

- ❑ Studied and characterized
- ❑ Labeled
- ❑ Broadest availability
- ❑ Reimbursement is by 3rd party

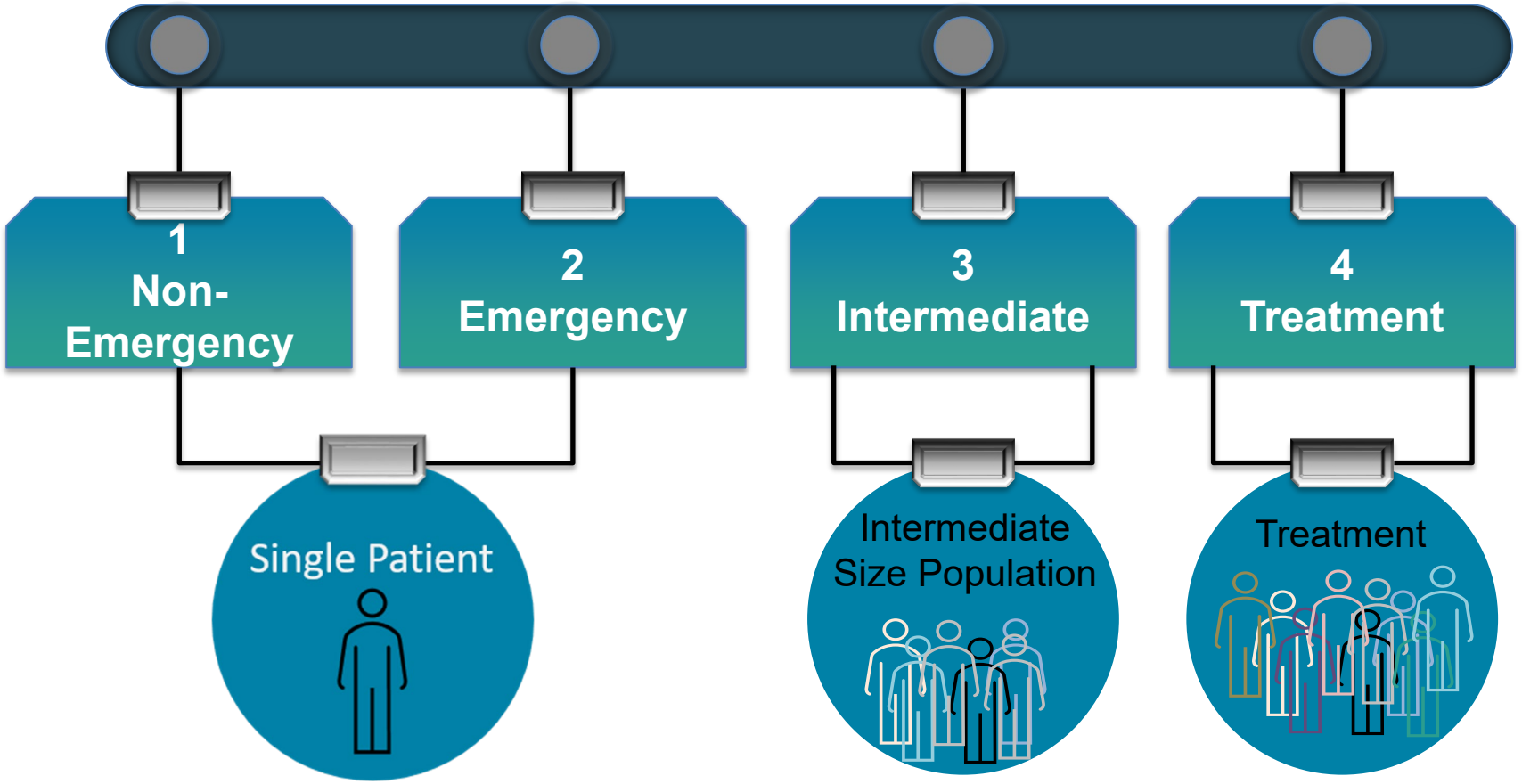
Clinical Trials

- ❑ Goal is research
- ❑ Provide necessary data to determine safety and effectiveness
- ❑ Most efficient path to market and broad availability
- ❑ https://clinicaltrial.gov

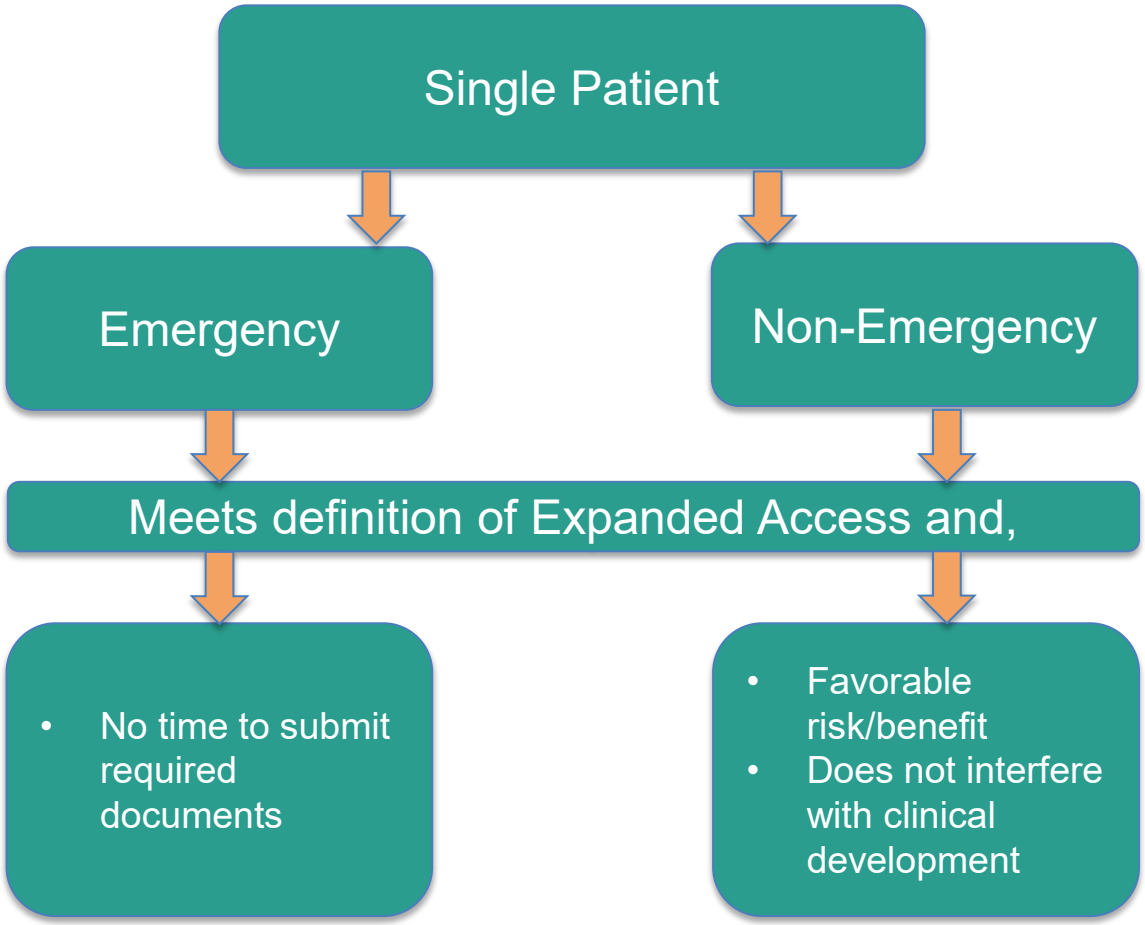
Expanded Access

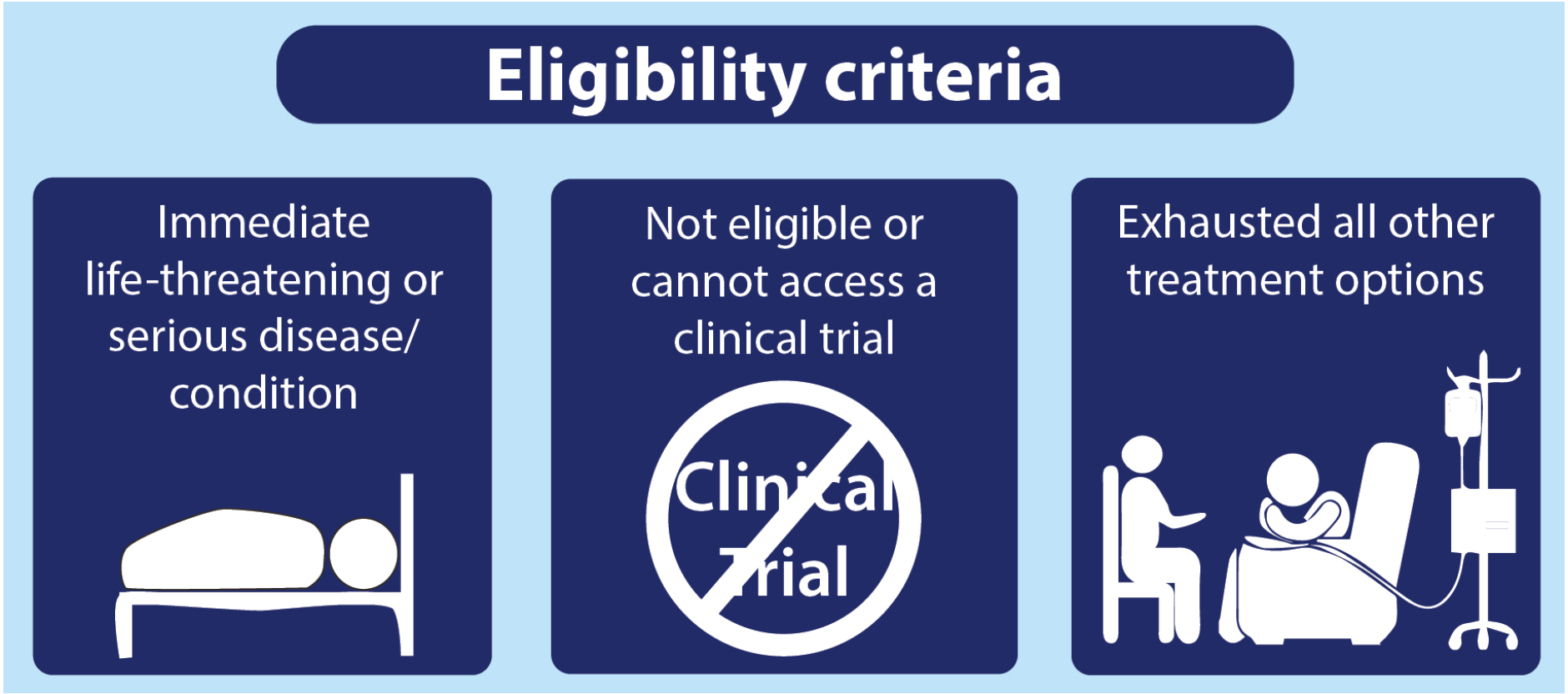
- ❑ Goal is access to treatment
- ❑ Represents opportunity when other options are exhausted
- ❑ https://www.fda.gov/news-events/public-health-focus/expanded-access

Expanded Access Program



Types of Applications





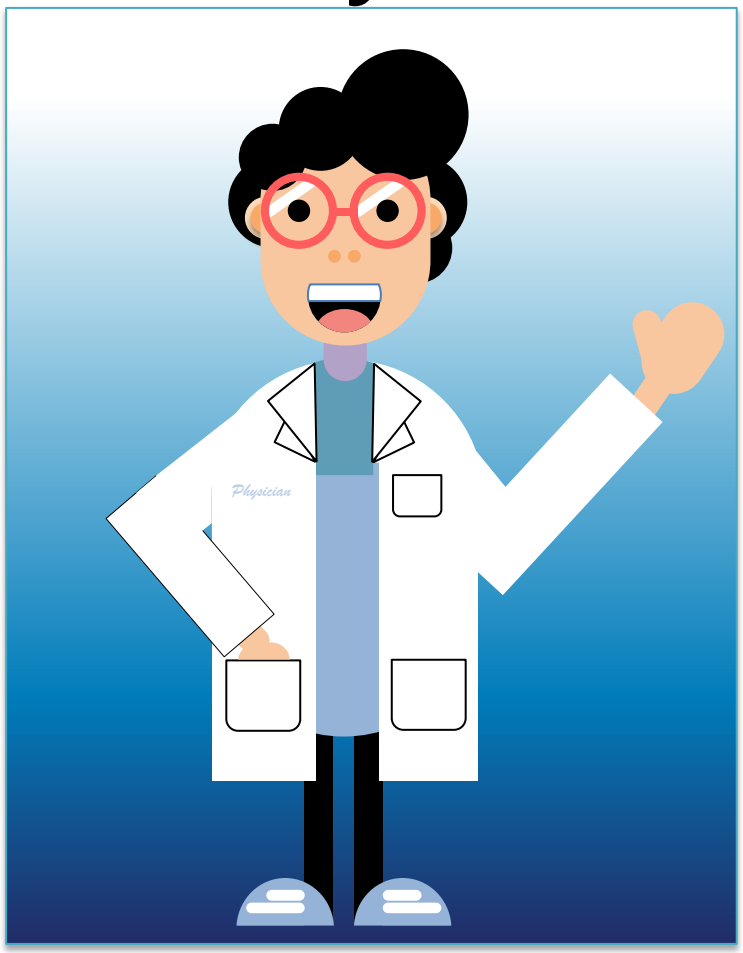
21 CFR 312.305

Human Subject Protection

- Apply to all expanded access requests

- Products under expanded access are investigational drugs, and are subject the following requirements:
  - Protection of human subjects (informed consent)
  - Institutional Review Boards (IRB)
    - With single patient IND requests, a chairperson or designated IRB member can authorize (no need for a full IRB convening)
  - Clinical holds based on safety
  - Reporting requirements (adverse event reports, annual reports)

Physician Responsibilities



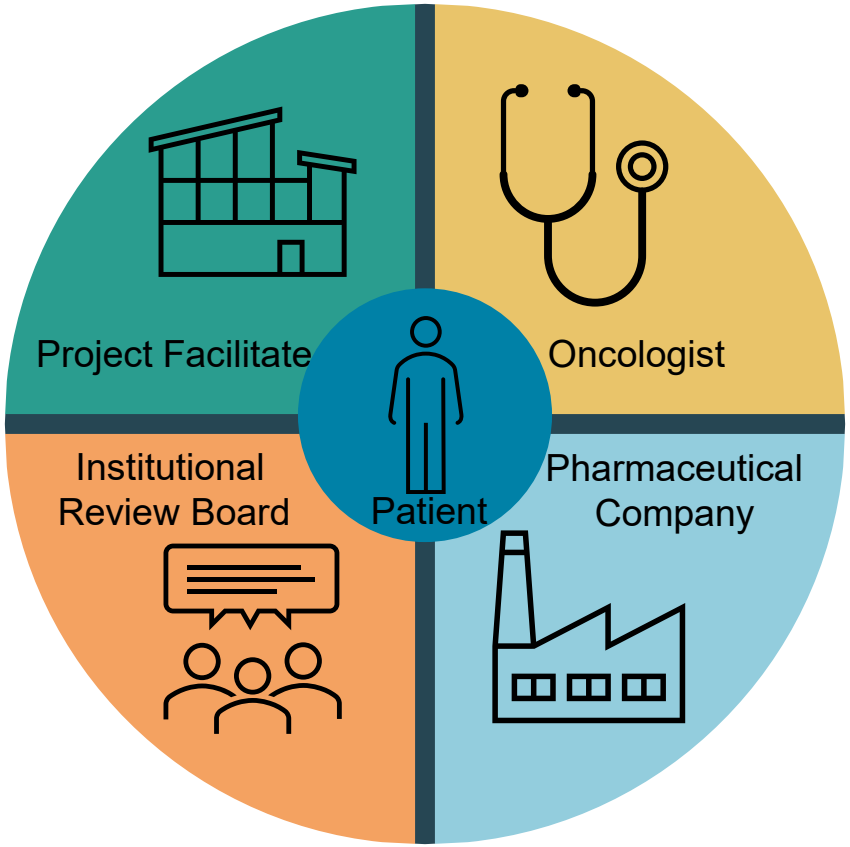
- Agrees to oversee the patient's treatment
- Works with industry (e.g., medical product developer)
- Files paperwork with FDA and IRB
- Responsible for patient care and reporting

Considerations

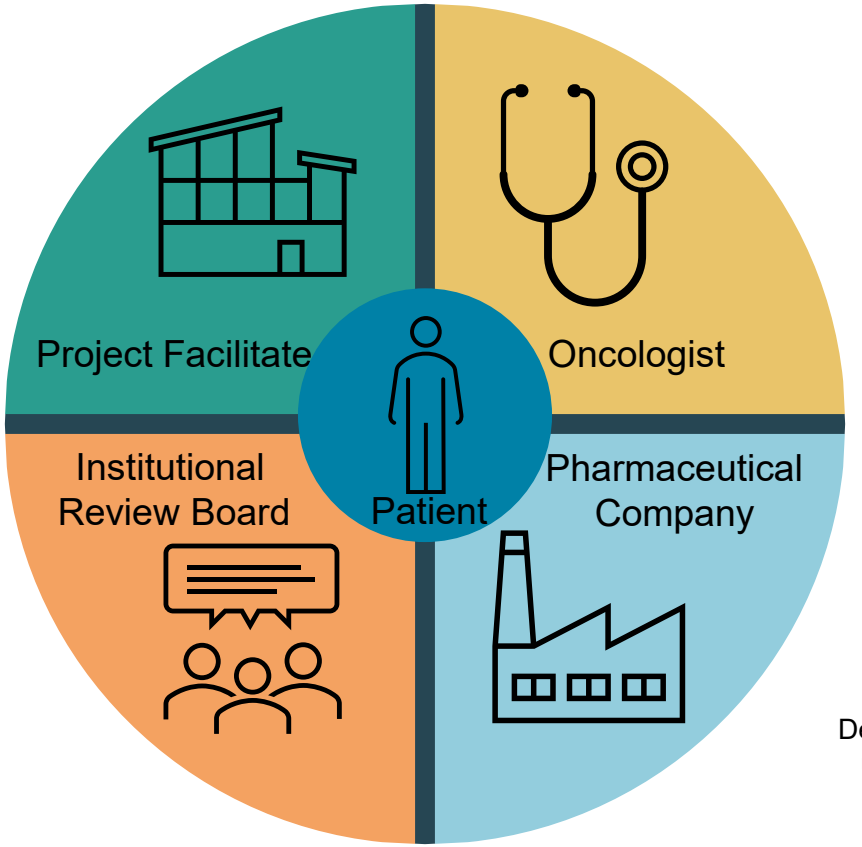
- Unknown risks associated with access to investigational products for which there is limited information about safety and effectiveness
  - Some patients may benefit
  - Some patients may experience no effect
  - Some patients may have serious adverse events
- FDA considers:
  - Potential harm to patient
  - Need to exhaust all existing approved therapies
  - Scientific likelihood of an efficacious response

Potential Benefits

- **Access** for oncology patients with serious or life-threatening diseases who have no other alternatives, and are willing to accept greater risk
- **Patient autonomy** over their health care decision
- **Bridge the gap** between the latter stages of product development and approval by making a drug widely available during that period
- **May provide data** to support development

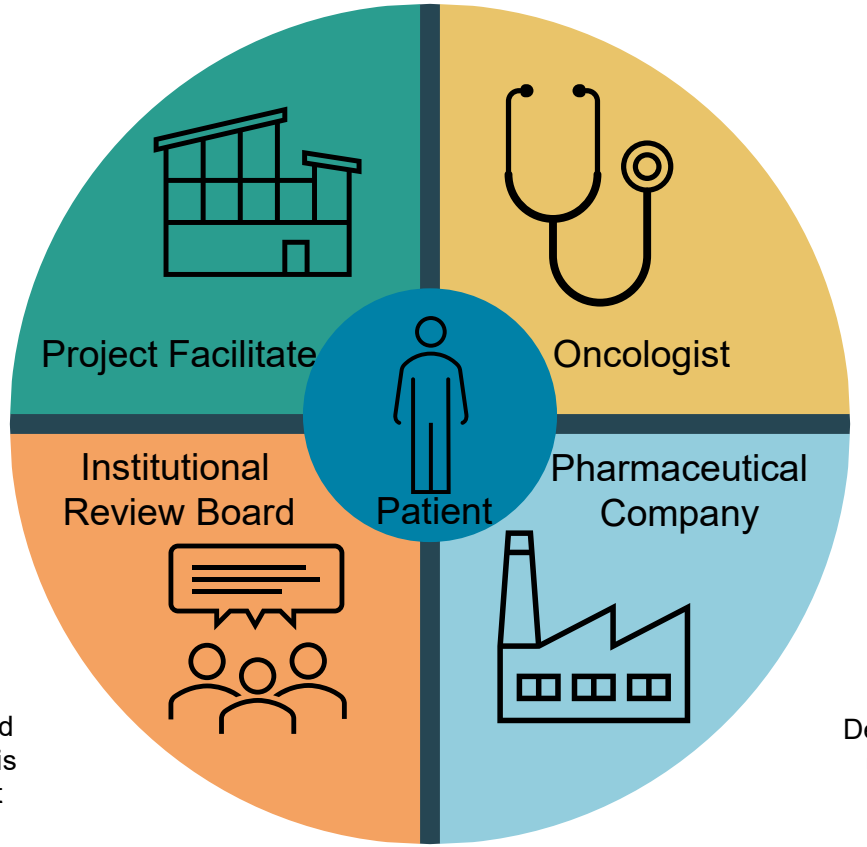


Initiates request, oversees the patient's treatment, and provides annual updates to the FDA



Initiates request, oversees the patient's treatment, and provides annual updates to the FDA

Determines if the product will be provided under Expanded Access and issues a Letter of Authorization to the treating physician



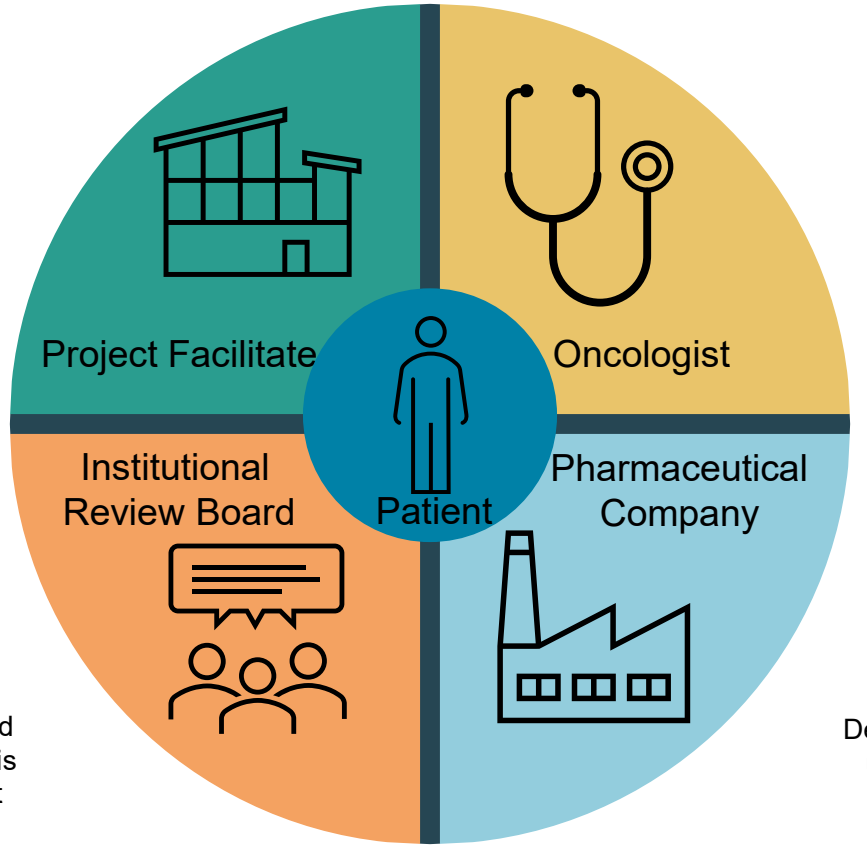
Initiates request, oversees the patient's treatment, and provides annual updates to the FDA

Reviews Expanded Access protocol and informed consent to ensure the patient is informed about the nature of treatment

Determines if the product will be provided under Expanded Access and issues a Letter of Authorization to the treating physician

Assists in completing the Expanded Access request, reviews the application, and determines if the treatment may proceed

Initiates request, oversees the patient's treatment, and provides annual updates to the FDA



Reviews Expanded Access protocol and informed consent to ensure the patient is informed about the nature of treatment

Determines if the product will be provided under Expanded Access and issues a Letter of Authorization to the treating physician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Project Facilitate Mission

…to promote equitable access to investigational products for patients with cancer by providing comprehensive support to oncology healthcare professionals in completing expanded access requests.

Addressing Barriers

- Collecting metrics on application Expanded
- Recording reasons why applications are denied
- Long-term surveillance
- Patient outcomes: benefits, adverse events

- Public outreach
- Personalized presentations and walkthroughs
- Reducing regulatory burden

Two-Pronged Approach for Oncology

Patient awareness and information on specific programs

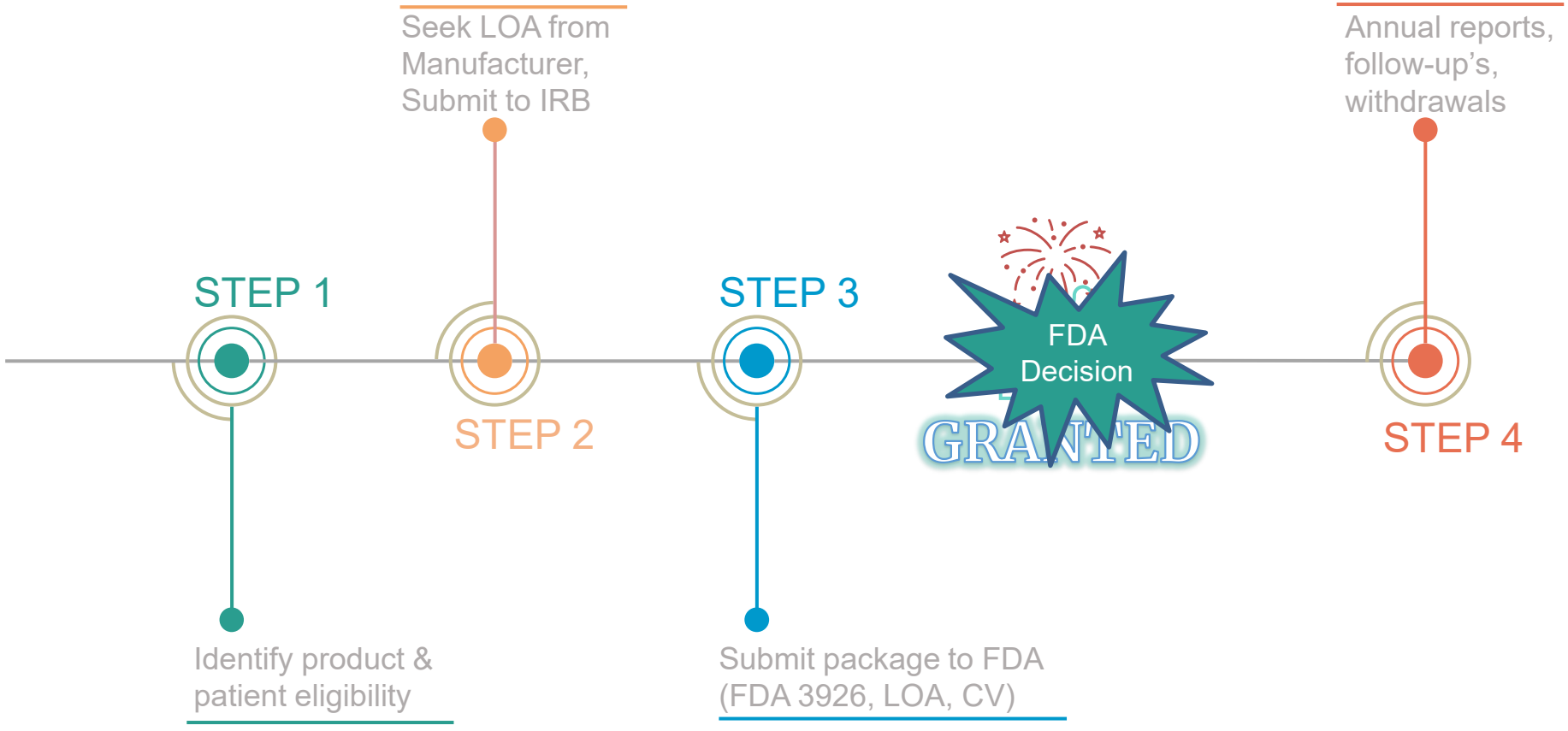
- *Reagan-Udall Expanded Access Navigator* website provides information on sponsors' policies and listings on ClinicalTrials.gov

Oncology healthcare provider access

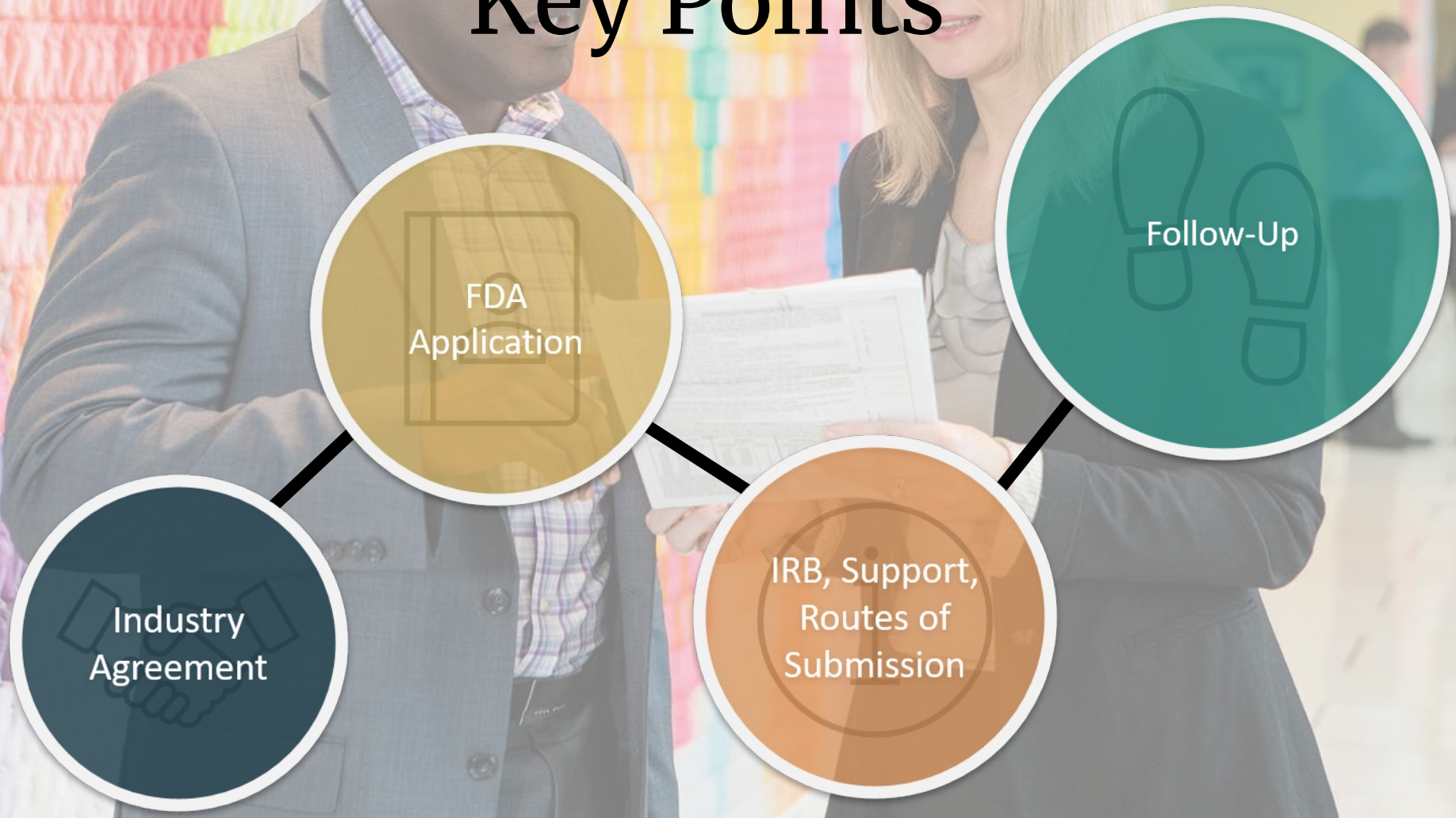
- *Oncology Center of Excellence Project Facilitate* program to provide continuous support to healthcare professionals and their teams throughout the EA process

Benefits of Project Facilitate

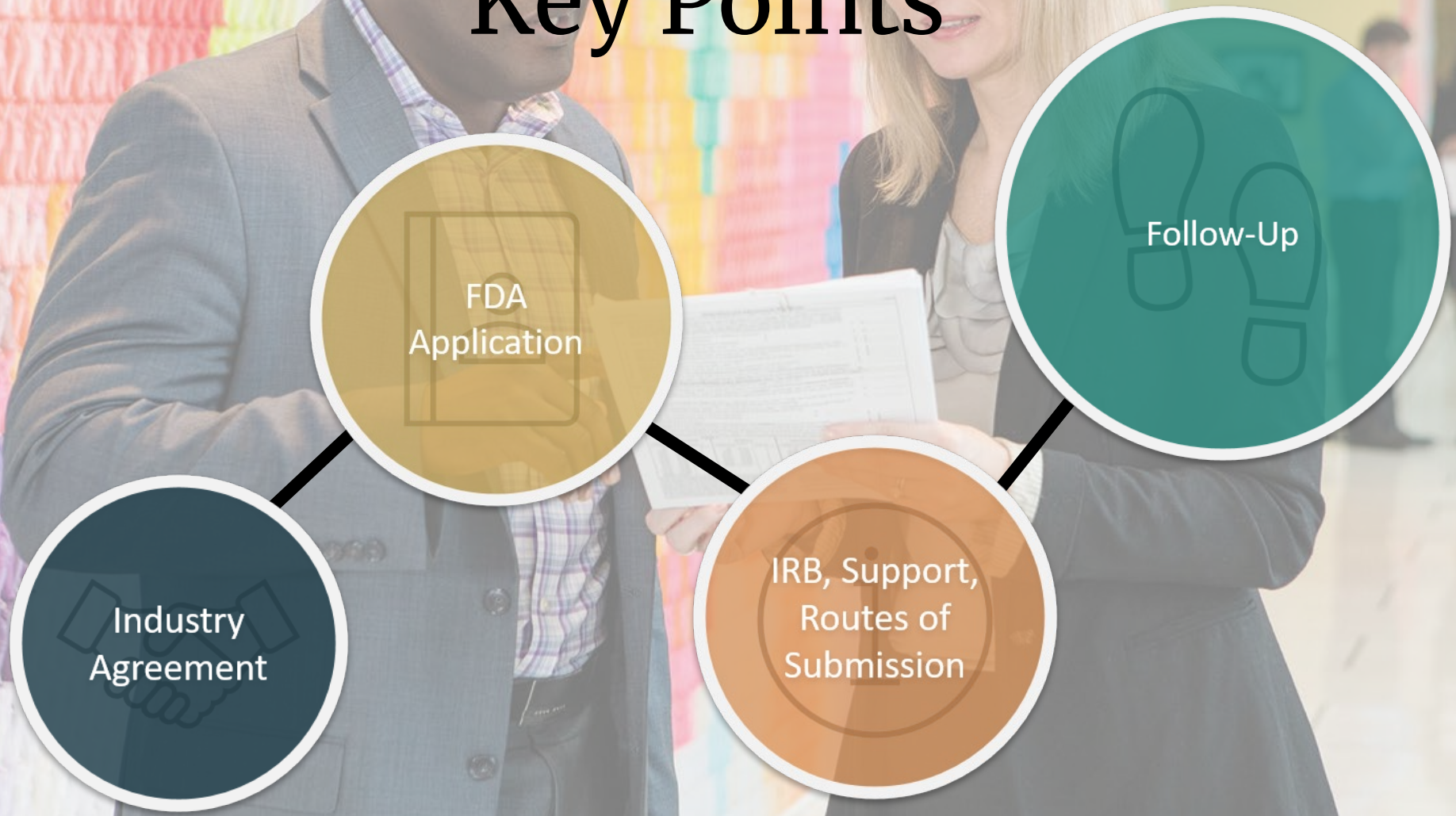
- One point of contact for all oncology single patient and emergency requests
- Dedicated clinical staff available during business hours to support requestors via phone or email
- Additional support options to provide:
  - IRB options
  - EA contact for drug/biotech company
  - Assistance filling out Form FDA 3926, if needed
- Efficiency in processing
- Collection of metrics
- Annual report reminders/Follow up

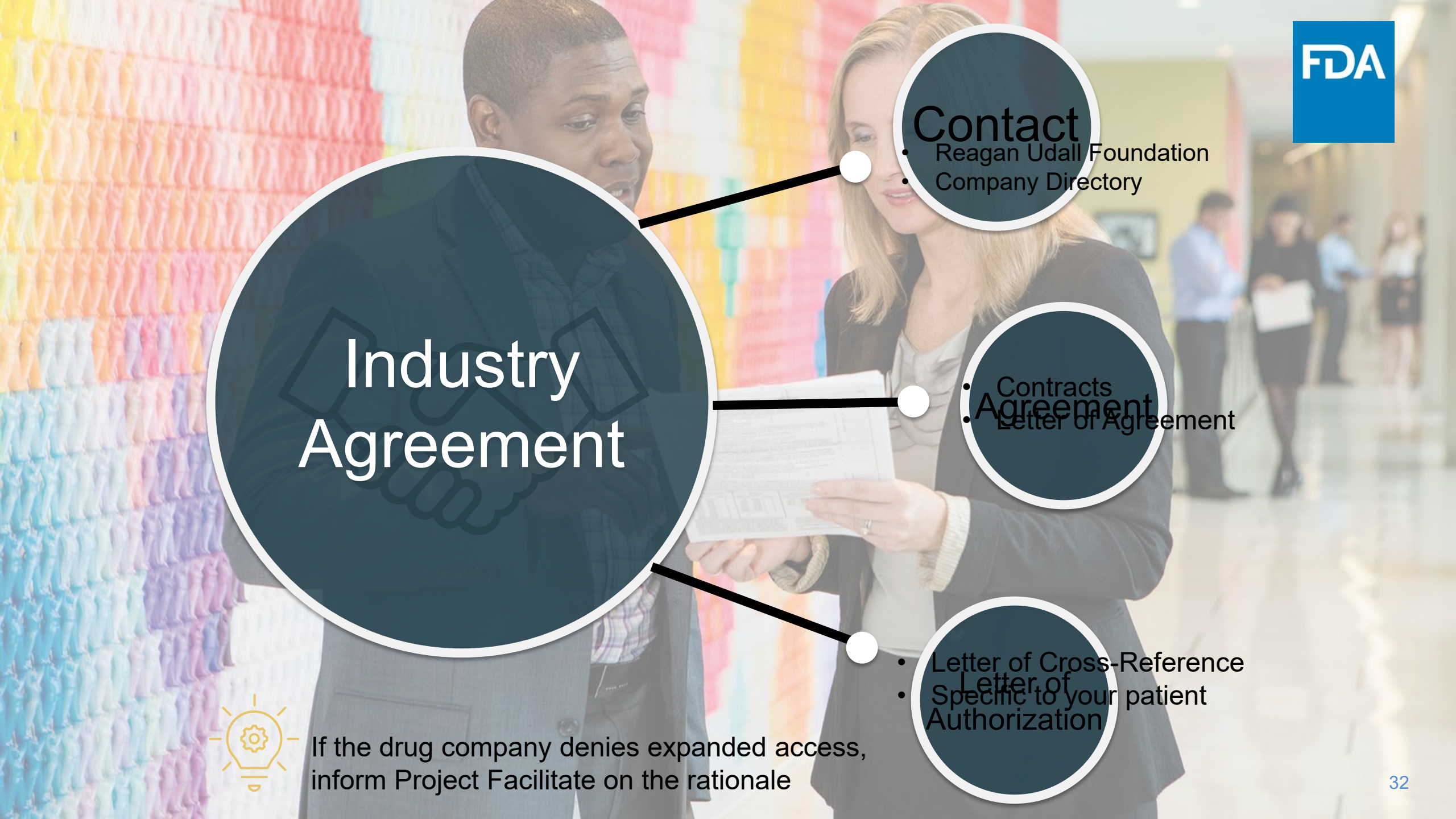

Seek LOA from Manufacturer, Submit to IRB
Annual reports, follow-up's, withdrawals
STEP 1
STEP 3
FDA Decision
GRANTED
STEP 2
STEP 4
Identify product & patient eligibility
Submit package to FDA (FDA 3926, LOA, CV)

Key Points


FDA Application
Follow-Up
Industry Agreement
IRB, Support, Routes of Submission

Key Points


FDA Application
Follow-Up
Industry Agreement
IRB, Support, Routes of Submission



If the drug company denies expanded access, inform Project Facilitate on the rationale

FDA Application

- Patient's medical and therapy history
- Treatment, monitoring and dose-modification plan
- Signed by treating physician

FDA Form 3926

- Letter of Authorization

LOA

- CV or Resume of treating physician
- Can be abbreviated
- *can fill in Section 7 of the 3926

CV

7. Physician's Qualification Statement *(Including medical school attended, year of graduation, medical specialty, state medical license number, current employment, and job title. Alternatively, attach the first few pages of physician's curriculum vitae (CV), provided they contain this information. If attaching the CV electronically, use normal PDF functions for file attachments.)*

IRB, Support, Routes of Submission

- Resources: patient and HCP
- Company directory
- Publications and resources on EA

Reagan-Udall Foundation

- RUF
- Paid and unpaid options

Third Party IRBs

- Email to PF
- RUF eRequest Gateway
- CDER NextGen Portal
- Paper

Email, PF, Portals

Submit Package to:
ONCProjectFacilitate@fda.hhs.gov

Follow-Up

Safety Reports, Amendments

- Safety Initial and Follow-Up Reporting
  - 15-day report
  - 7-day report
- Follow-up IND Safety Report

Annual Reports

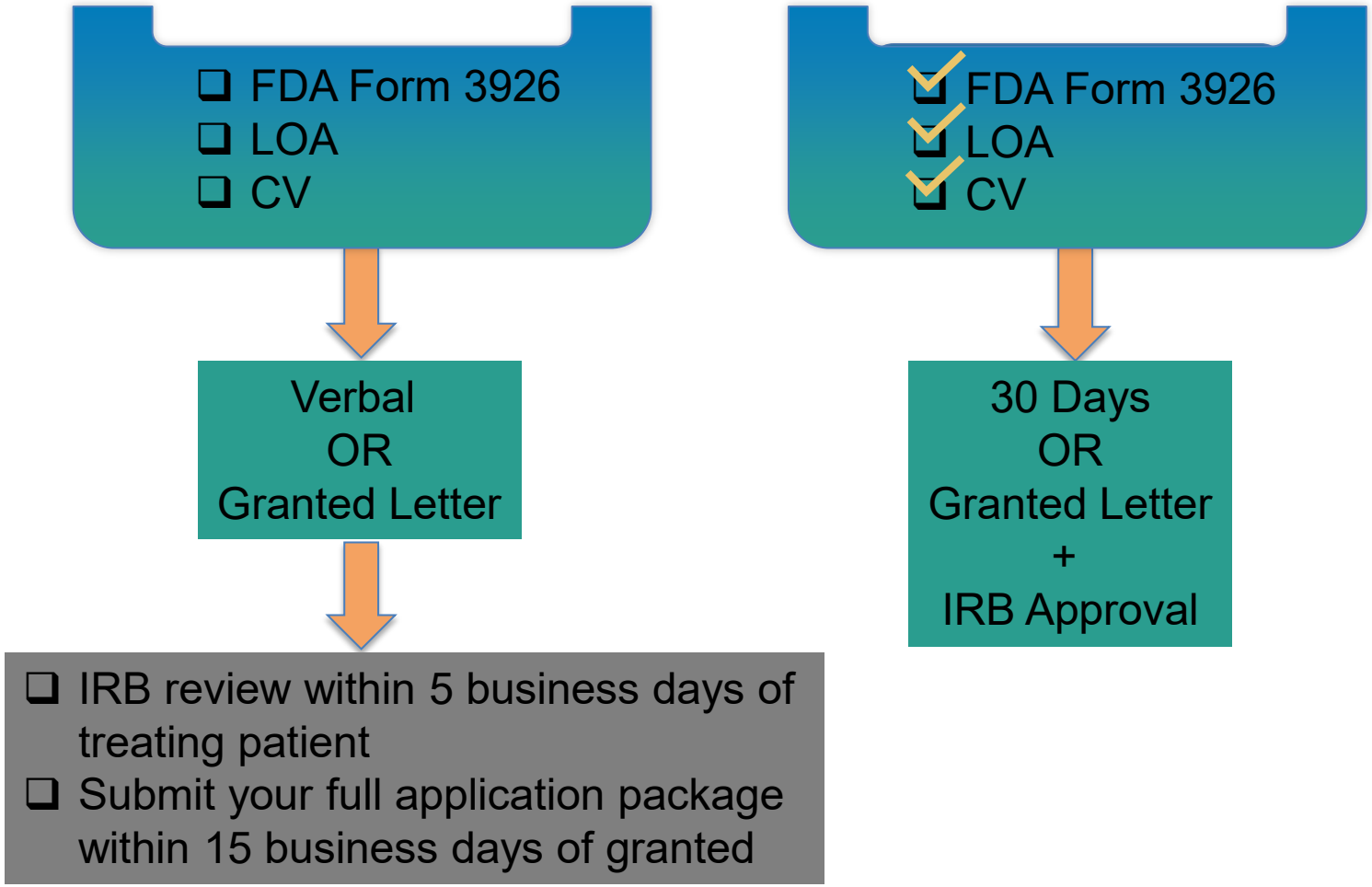
- Due every anniversary
- Summary information
- Suspected adverse events due to the inv. product

Withdrawals

- Patient no longer on investigational product

Submit follow-ups digitally or by paper. PF does not accept emailed follow-ups except for emailed safety reports

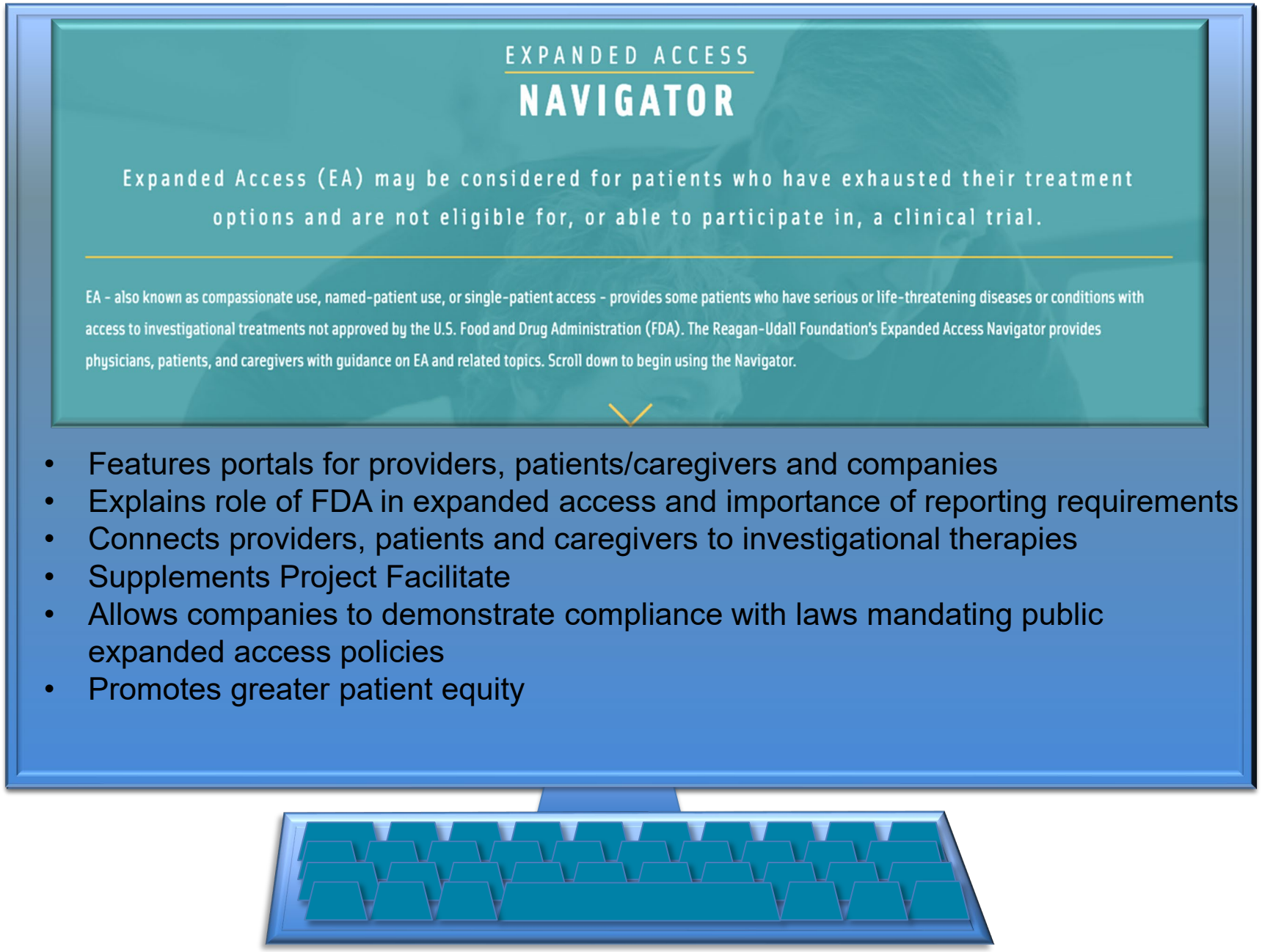
When Can Access Begin?



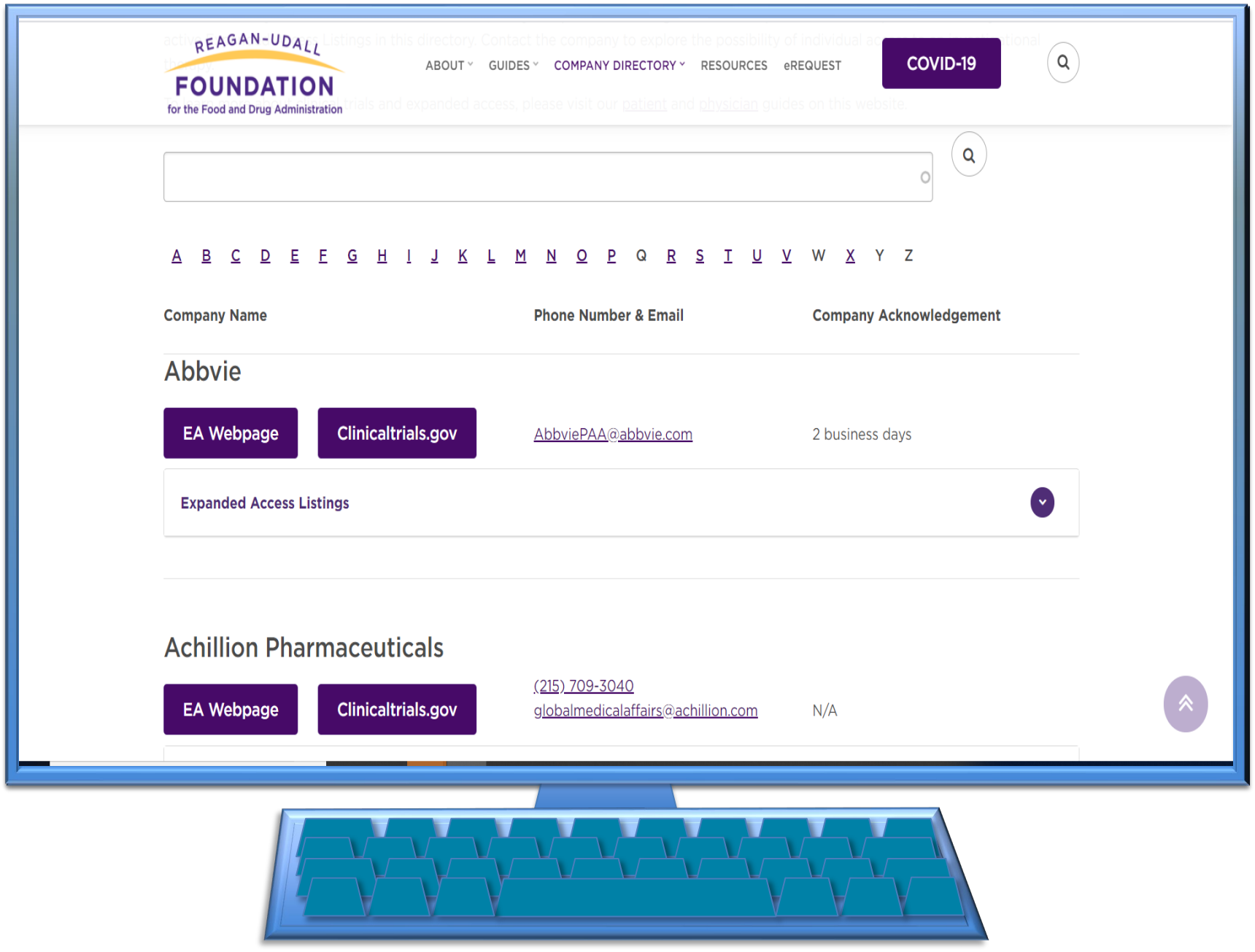
Reagan-Udall Foundation: Expanded Access Navigator

Created by the Reagan-Udall Foundation for the FDA, the EA Navigator:

- Provides clear and factual information in an online platform
- Takes users step-by-step through the process of expanded access requests
- Serves as a roadmap for single-patient expanded access requests that inform patients, physicians and companies exploring EA



- Features portals for providers, patients/caregivers and companies
- Explains role of FDA in expanded access and importance of reporting requirements
- Connects providers, patients and caregivers to investigational therapies
- Supplements Project Facilitate
- Allows companies to demonstrate compliance with laws mandating public expanded access policies
- Promotes greater patient equity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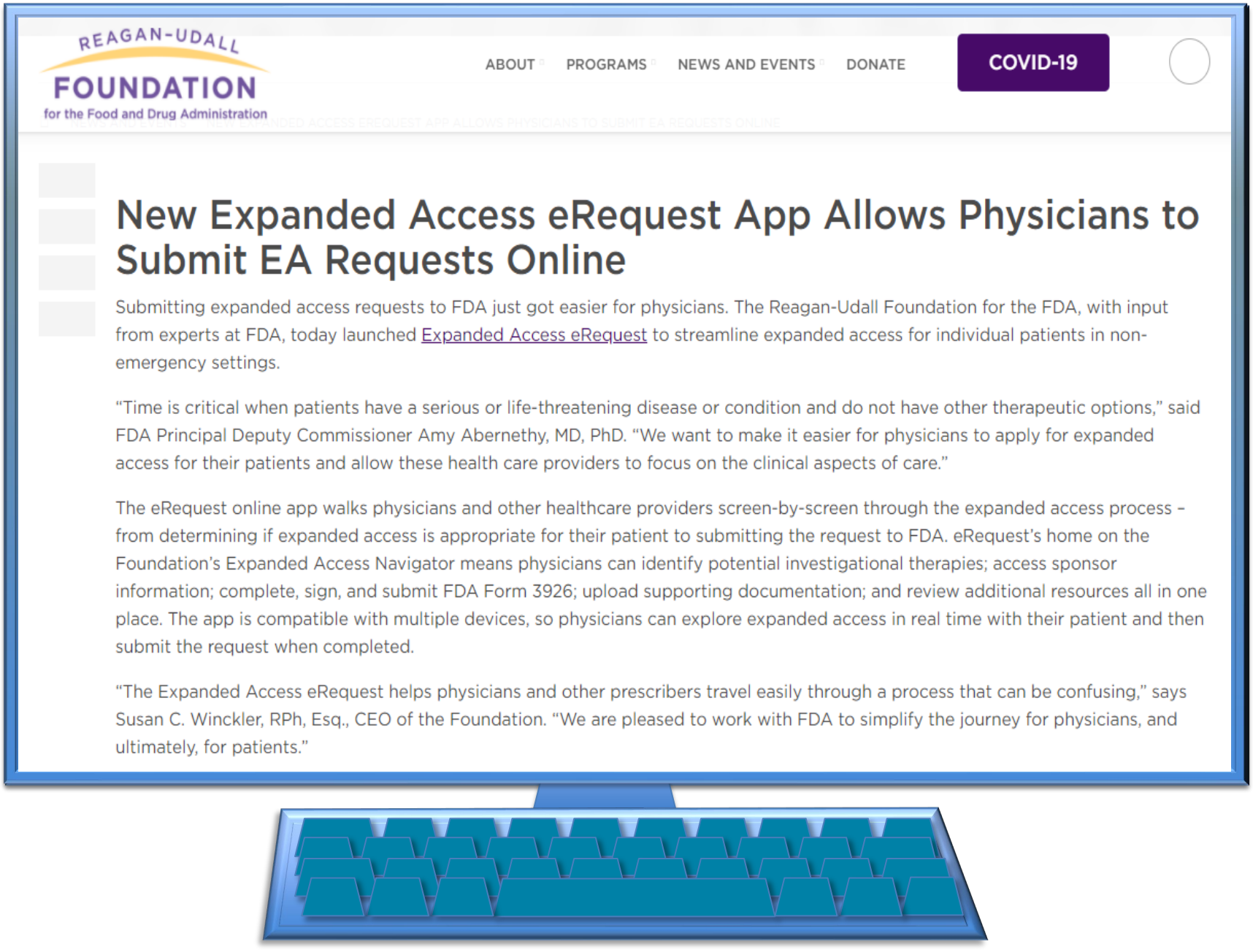
for the Food and Drug Administration
ABOUT
GUIDES
COMPANY DIRECTORY
RESOURCES
eREQUEST
COVID-19
A B C D E F G H I J K L M N O P Q R S T U V W X Y Z
Company Name
Phone Number & Email
Company Acknowledgement
Abbvie
EA Webpage
Clinicaltrials.gov
AbbviePAA@abbvie.com
2 business days
Expanded Access Listings
Achillion Pharmaceuticals
EA Webpage
Clinicaltrials.gov
(215) 709-3040
globalmedicalaffairs@achillion.com
N/A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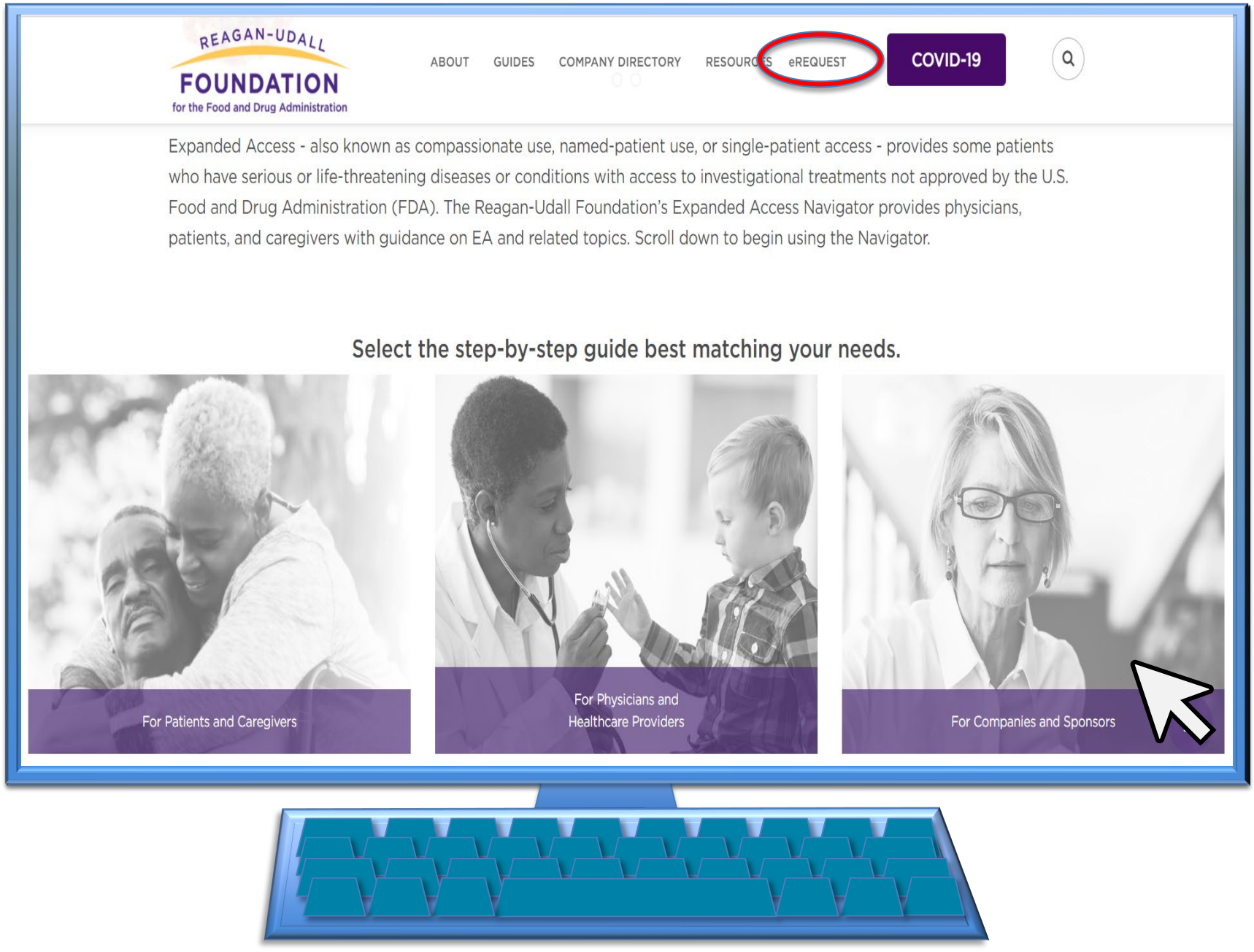
New Expanded Access eRequest App Allows Physicians to Submit EA Requests Online
Submitting expanded access requests to FDA just got easier for physicians. The Reagan-Udall Foundation for the FDA, with input from experts at FDA, today launched Expanded Access eRequest to streamline expanded access for individual patients in non-emergency settings.
"Time is critical when patients have a serious or life-threatening disease or condition and do not have other therapeutic options," said FDA Principal Deputy Commissioner Amy Abernethy, MD, PhD. "We want to make it easier for physicians to apply for expanded access for their patients and allow these health care providers to focus on the clinical aspects of care."
The eRequest online app walks physicians and other healthcare providers screen-by-screen through the expanded access process – from determining if expanded access is appropriate for their patient to submitting the request to FDA. eRequest's home on the Foundation's Expanded Access Navigator means physicians can identify potential investigational therapies; access sponsor information; complete, sign, and submit FDA Form 3926; upload supporting documentation; and review additional resources all in one place. The app is compatible with multiple devices, so physicians can explore expanded access in real time with their patient and then submit the request when completed.
"The Expanded Access eRequest helps physicians and other prescribers travel easily through a process that can be confusing," says Susan C. Winckler, RPh, Esq., CEO of the Foundation. "We are pleased to work with FDA to simplify the journey for physicians, and ultimately, for patients."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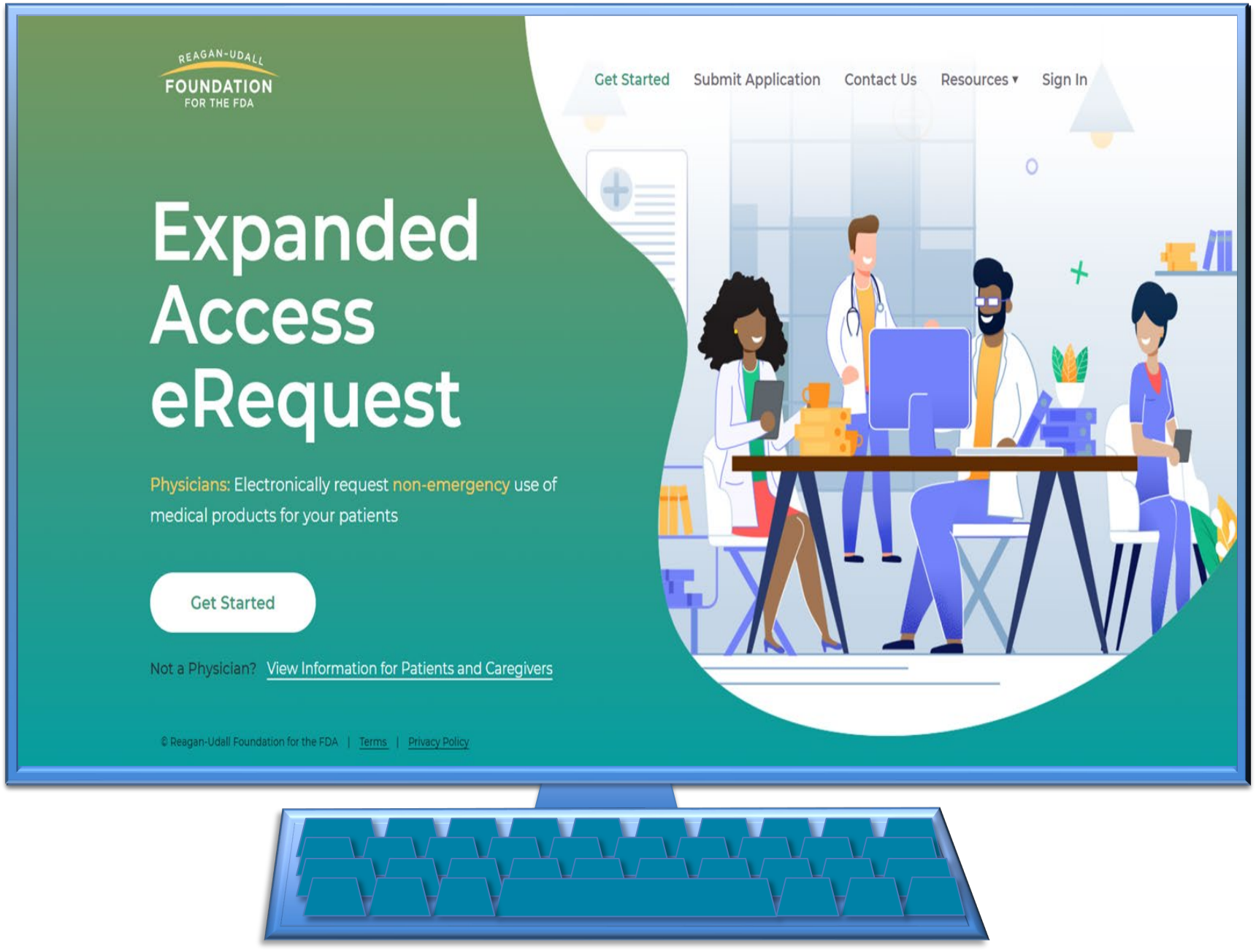
Expanded Access - also known as compassionate use, named-patient use, or single-patient access - provides some patients who have serious or life-threatening diseases or conditions with access to investigational treatments not approved by the U.S. Food and Drug Administration (FDA). The Reagan-Udall Foundation's Expanded Access Navigator provides physicians, patients, and caregivers with guidance on EA and related topics. Scroll down to begin using the Navigator.
Select the step-by-step guide best matching your needs.
For Patients and Caregivers
For Physicians and Healthcare Providers
For Companies and Sponsors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FOUNDATION
FOR THE FDA
Get Started
Submit Application
Contact Us
Resources ▾
Sign In
Expanded Access eRequest
Physicians: Electronically request non-emergency use of medical products for your patients
Get Started
Not a Physician? View Information for Patients and Caregivers
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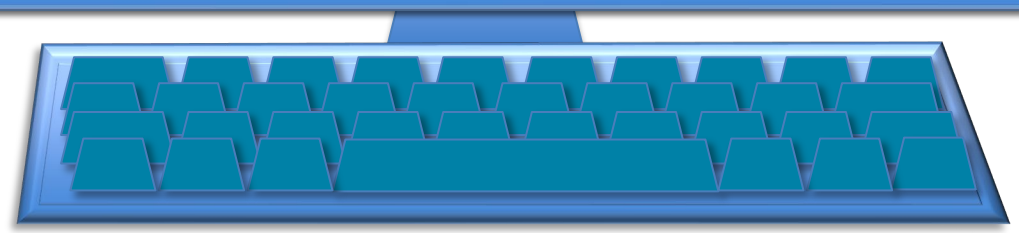
Expanded Access Resources

- Patients and Non-Oncology Healthcare Professionals
  - Reagan Udall Foundation
    - Website: https://reaganudall.org/
  - FDA Division of Drug Information (DDI)
    - Website: https://www.fda.gov/news-events/expanded-access/fdas-expanded-access-contact-information
    - (855) 543-DRUG or druginfo@fda.hhs.gov
- Oncology Healthcare Professionals
  - Project Facilitate
    - Website: https://www.fda.gov/about-fda/oncology-center-excellence/project-facilitate
    - (240) 402-0004 or ONCProjectFacilitate@fda.hhs.gov
  - Reagan Udall Foundation
    - EA Navigator: https://navigator.reaganudall.org/expanded-access-navigator



2023: A Year in Review

Date: January 1, 2023 – October 31, 2023

Comprehensive Program

- Call Center
- Review and manage applications
- Full-time clinical staff
- Outreach
- Publications
- Research
- Policy

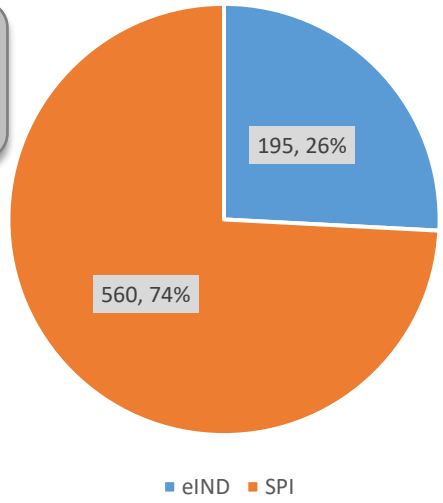
Total Calls: 338

95% answered on first call

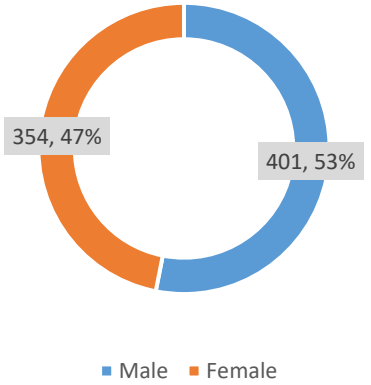
Total Emails: 9646

Applications by eIND/SPI (1/1/2023 - 10/31/2023)

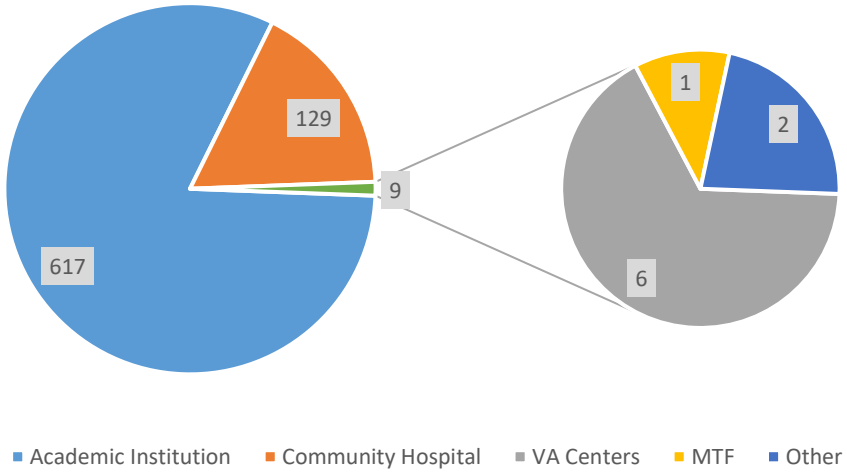
Total 755



Applications by Gender (1/1/2023 - 10/31/2023)

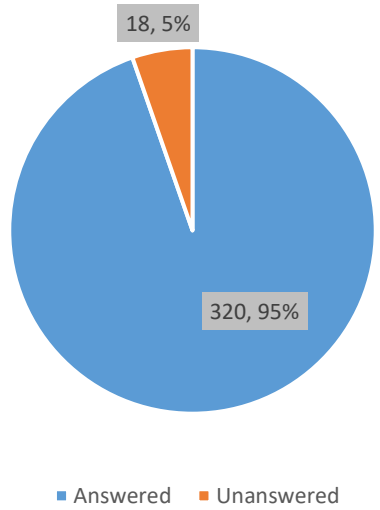


Applications by Institution/Sponsorship Type (1/1/2023 - 10/31/2023)



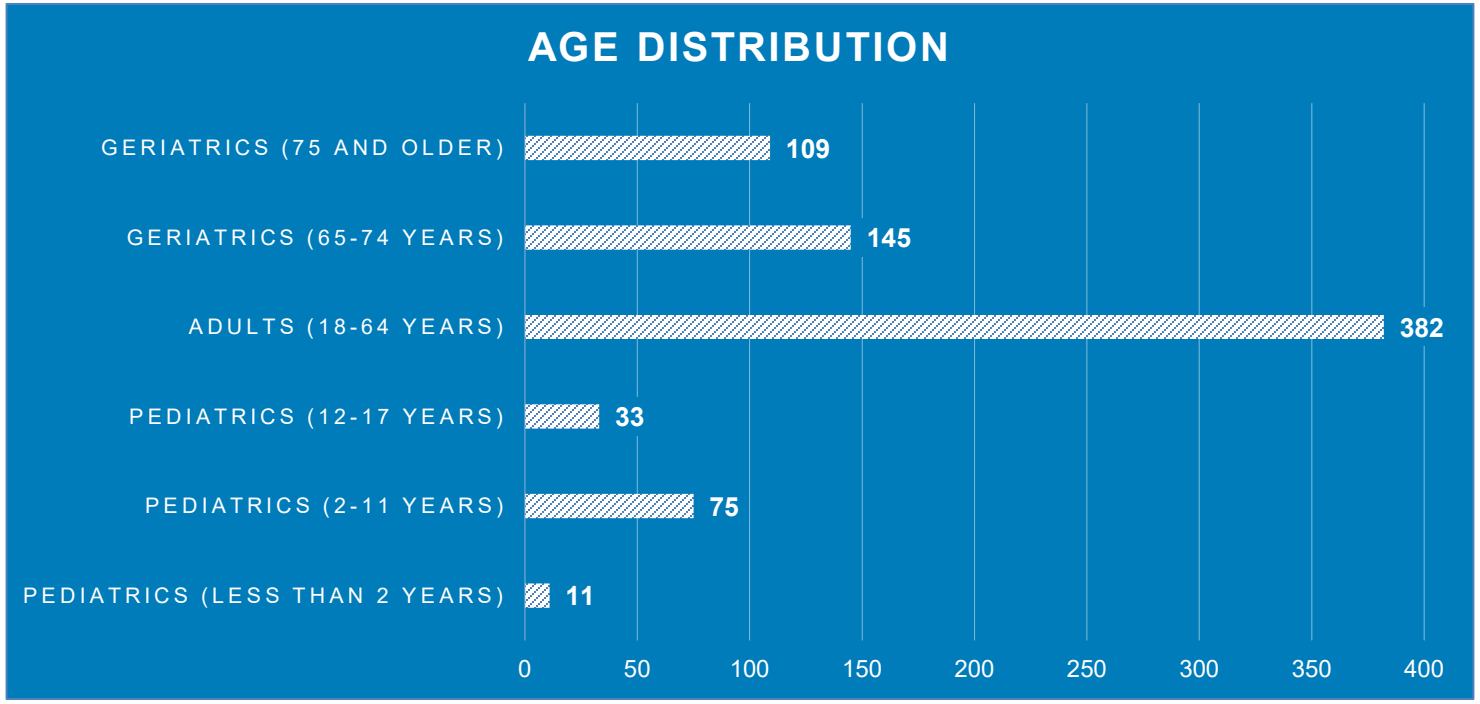
Number of Call Center Calls Received (1/1/2023 - 10/31/2023)

Total 338

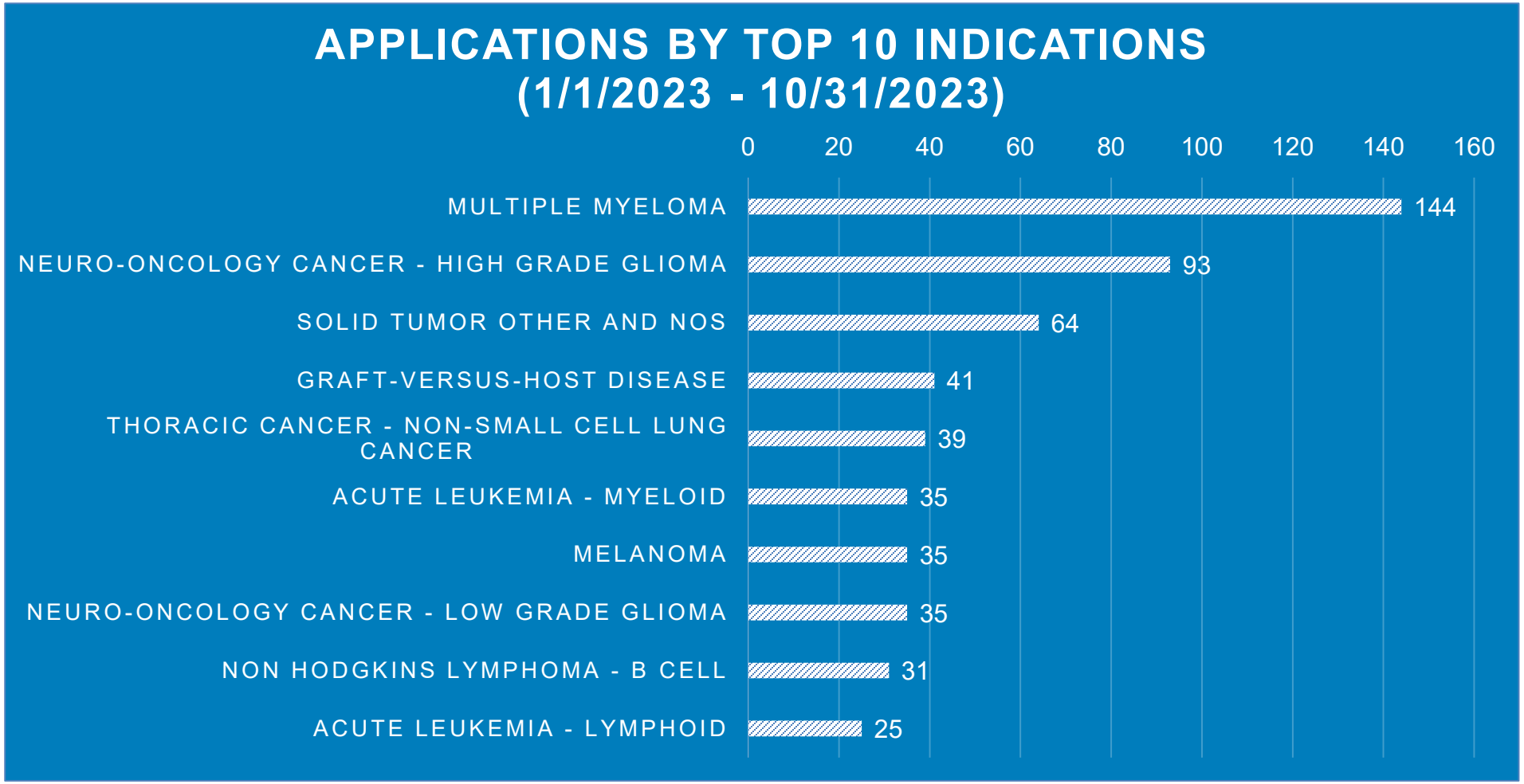


A Year in Review

- Total applications: 755
- Total pediatric applications: 119 (15.8%)



Top 10 Indications



Useful Resources

- FDA Project Facilitate Website: https://www.fda.gov/about-fda/oncology-center-excellence/project-facilitate
- FDA Expanded Access Site: https://www.fda.gov/news-events/public-health-focus/expanded-access
- Reagan-Udall Foundation EA Navigator: https://navigator.reaganudall.org/expanded-access-navigator
- eRequest: https://erequest.navigator.reaganudall.org
- Form 3926: https://www.fda.gov/media/98616/download
- Instructions for 3926: https://www.fda.gov/media/98627/download
- FDA Drug Info Rounds Expanded Access Video Series: https://www.fda.gov/drugs/information-healthcare-professionals-drugs/fda-drug-info-rounds-expanded-access-video-series

After Hours Emergency Requests: Contact FDA's Emergency Call Center at (866) 300-4374

Oncology Center of Excellence: What We are About

Oncology Center of Excellences' Programs and Initiatives

- Immuno-Oncology
- Oncology Cell and Gene Therapy
- Oncology Device and Diagnostics
- Oncology Regulatory Affairs and Policy
- Patient-Focused Drug Development
- Pediatric Oncology
- Precision Oncology
- Oncology Real World Evidence

- Project Facilitate
- Project Renewal
- Project Accelerate
- Project Point/Counterpoint
- Project Protect
- Project Patient Voice
- Project SignifiCanT
- Project Equity
- Project Silver
- Project Post Covidity
- Project Pragmatica
- Project Catalyst

Learn more at https://www.fda.gov/about-fda/oce-annual-reports/2022-oce-annual-report

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