REAGAN-UDALL FOUNDATION FOR THE FDA

VIRTUAL PUBLIC MEETING

Expanded Access: Insights & Resources

Understanding the process and the pathways to investigational treatment

August 18, 2026
2–3:30 pm (Eastern)

PhRMA provided funding for this webinar

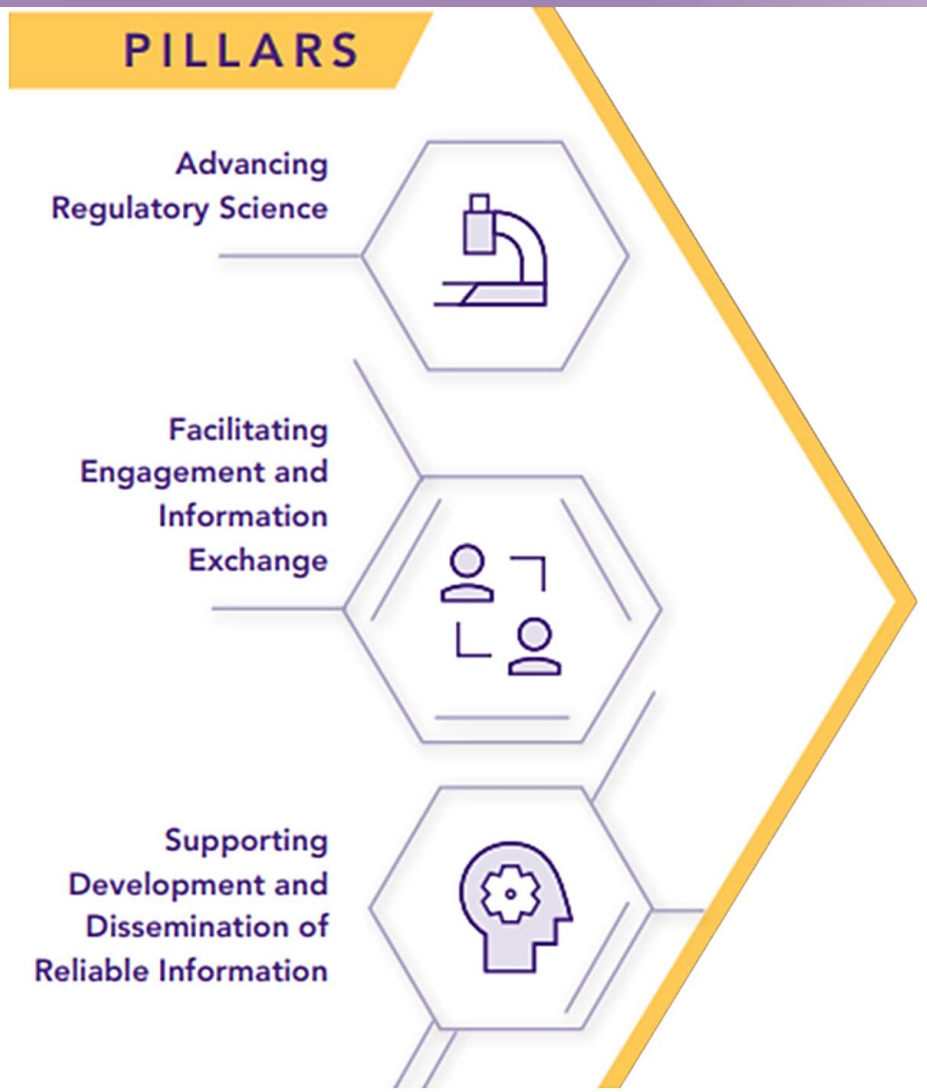
REAGAN-UDALL FOUNDATION FOR THE FDA

Welcome & Opening Remarks

Susan C. Winckler, RPh, Esq.

CEO, Reagan-Udall Foundation for the FDA

About the Foundation



- Independent nonprofit organization created by US Congress in 2007 to help FDA "do more" to protect and promote the public's health by advancing innovation
- Foundation receives financial support from the US FDA and many organizations within the FDA ecosystem
- As a working Foundation, we manage a suite of programs that stimulate change: we help the FDA engage with external stakeholders and facilitate evidence generation, improve public understanding of the Agency and the products it regulates, and deliver more accessible health information to the public

PhRMA provided funding for this webinar

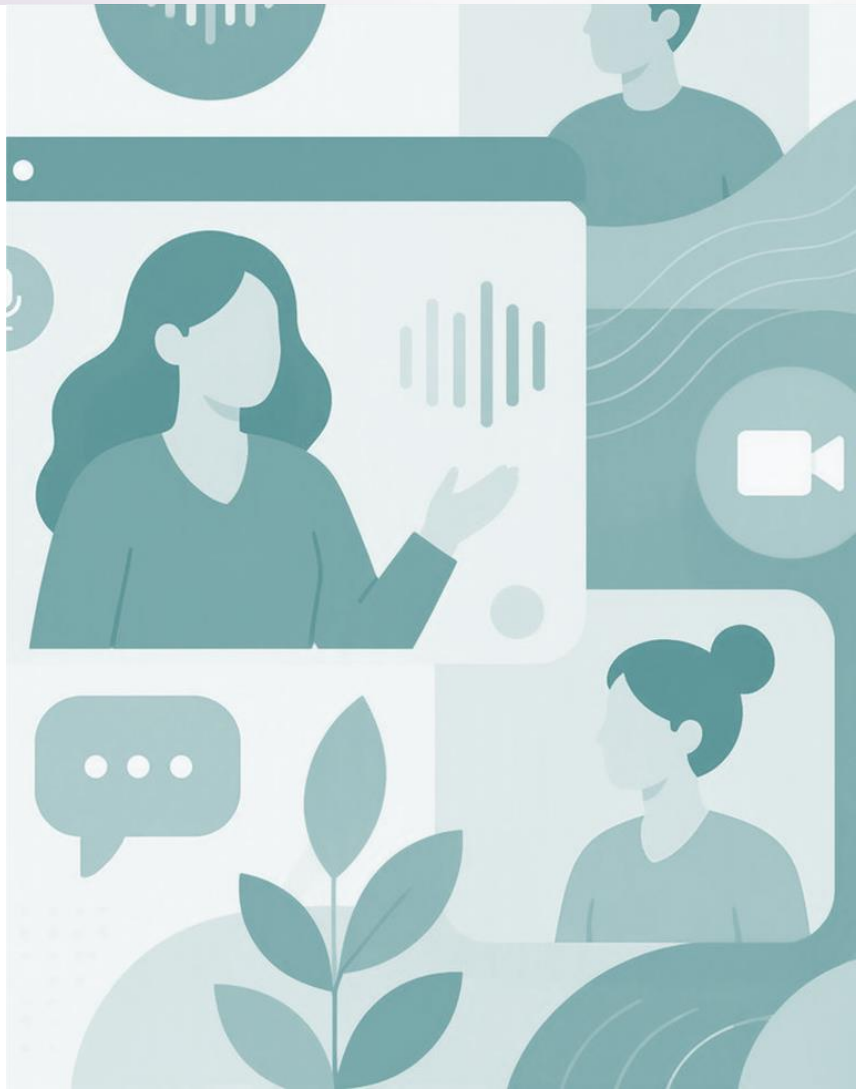
Housekeeping

REAGAN-UDALL FOUNDATION FOR THE FDA

All attendee microphones will remain muted for the duration of the webinar

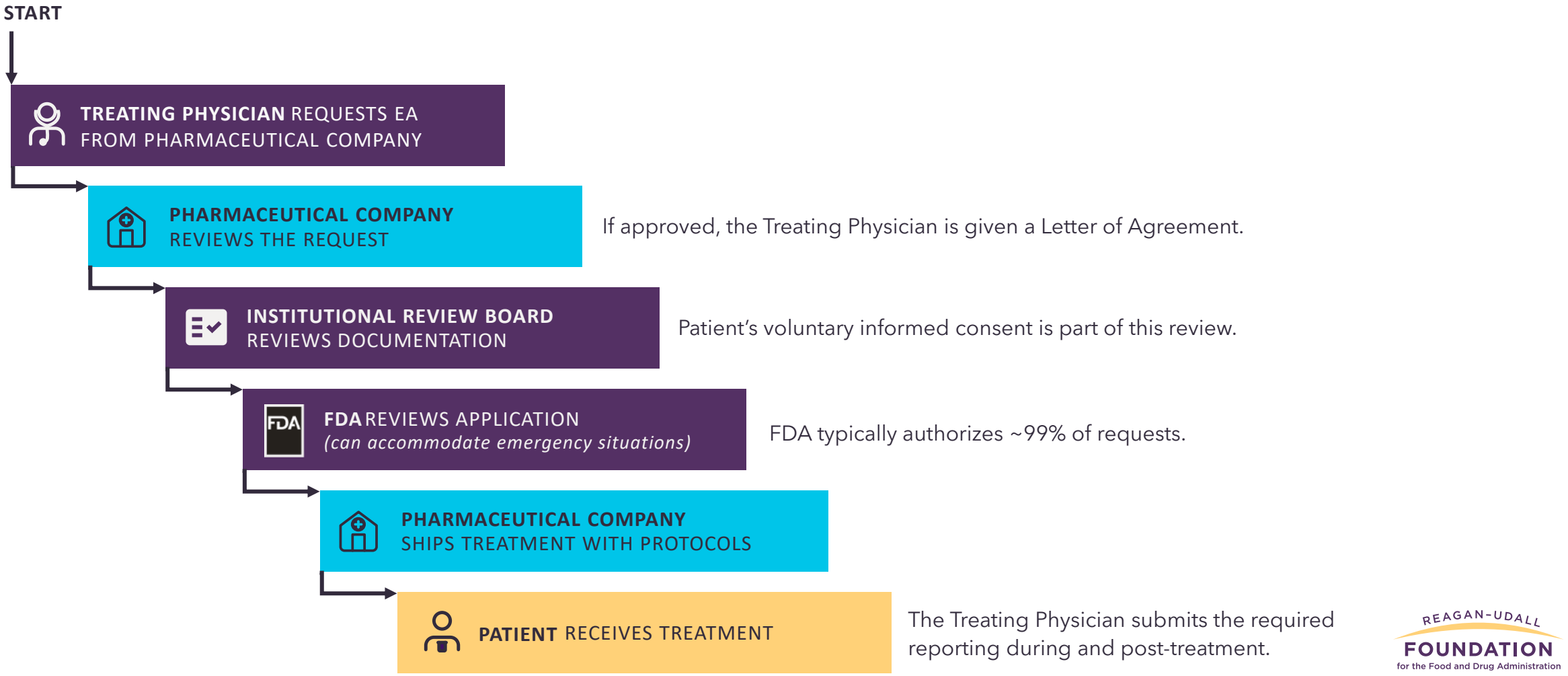
Please submit your questions at any time using the Zoom Q&A function

This webinar is being recorded. The recording and transcript will be posted to our website following the event at www.ReaganUdall.org



Single-Patient Expanded Access Pathway

Expanded access (EA) can provide a pathway for patients with serious or life-threatening diseases or conditions to access investigational (unapproved) medical products outside of a clinical trial when no comparable or satisfactory treatment options are available.



REAGAN-UDALL FOUNDATION for the Food and Drug Administration

Agenda

REAGAN-UDALL FOUNDATION FOR THE FDA

2pm	**Welcome & Opening Remarks**	**Susan C. Winckler, RPh, Esq.**, CEO, Reagan-Udall Foundation for the FDA
2:10pm	**FDA Remarks**	**Grace Graham**, **MPP,** Deputy Commissioner for Policy, Legislation, and International Affairs, FDA
2:15pm	**Expanded Access Overview**	**Rihana Miller, JD**, Regulatory Counsel, Office of Medical Policy, Center for Drug Evaluation and Research, FDA
2:30pm	**FDA & Foundation Resources**	**Jessica Palfreyman**, Senior Program Associate, Reagan-Udall Foundation for the FDA
2:40pm	**Stakeholder Dialogue**	**Alison Bateman-House, PhD, MPH**, Associate Professor, NYU Grossman School of Medicine **Mitchell Chan, PharmD, BCPS,** Team Lead, Clinical Analyst, Oncology Center of Excellence, FDA **Pat Furlong**, **BSN, MS**, Founding President & CEO, Parent Project Muscular Dystrophy **Allison Martin, MS**, Director, Regulatory Science & Policy, Sanofi **Fabian Sandoval, MD**, President & CEO, Emerson Clinical Research Institute
3:15pm	**Closing Remarks & Adjourn**	

REAGAN-UDALL FOUNDATION FOR THE FDA

FDA Remarks

Grace Graham, MPP

Deputy Commissioner for Policy, Legislation, and International Affairs
FDA

REAGAN-UDALL FOUNDATION FOR THE FDA

Expanded Access Overview

Rihana Miller, JD

Regulatory Counsel, Office of Medical Policy
Center for Drug Evaluation and Research
FDA

Overview of the Expanded Access (EA) Program

Rihana Miller, JD
Regulatory Counsel
Office of Medical Policy
Center for Drug Evaluation and Research
FDA

Objectives

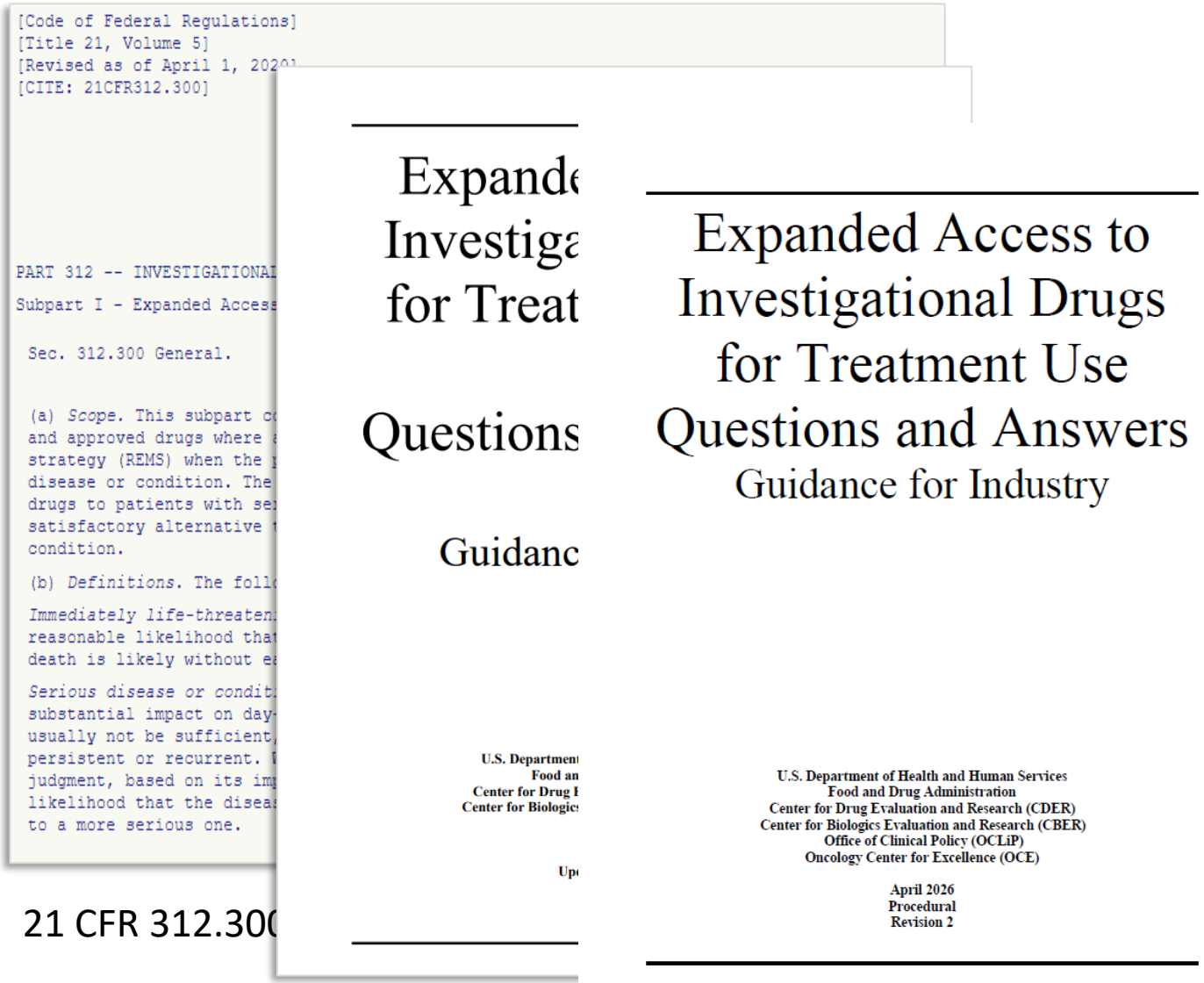
- Define expanded access
- Explain the expanded access background, regulations, and guidance
- Discuss the three categories of expanded access, submission requirements, and processes
- Identify resources for preparing expanded access requests for individual patients

What is Expanded Access (EA)?

- Expanded Access is the use of an investigational drug or biologic to treat a patient with a serious or immediately life-threatening disease or condition who does not have comparable or satisfactory alternative therapies to treat the disease or condition.
  - Primary intent is clearly treatment, not research

- Contrast with investigational drug in a clinical trial, whereby the primary intent is research
  - Systematic collection of data with the intent to analyze it to learn about the drug

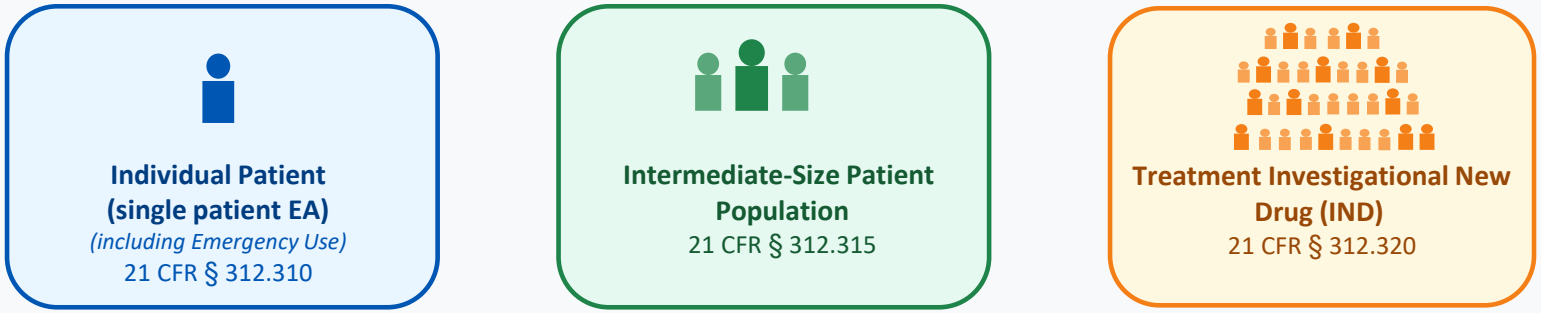
Expanded Access Regulations and Guidance



21 CFR 312.300

- Describes the general criteria applicable to all categories of access, and additional criteria that must be met for each access category
- Describes the requirements for submission
- Describes the safeguards applicable to Expanded Access Programs (EAP), such as informed consent, IRB review, and reporting requirements

Three General Categories of Expanded Access and Their Common Requirements



Common Requirements:*

1. Patients have serious or immediately life-threatening disease or condition
2. No comparable or satisfactory alternative therapy available
3. Potential benefits must justify the potential risks of the treatment
4. Providing the product under EA will not interfere with or compromise the investigational product development program

* Additional criteria, specific to each category, must be fulfilled

Individual Patient Expanded Access

Subcategories:

- ***Individual patient expanded access for emergency***

  Used for emergency situations (e.g., a patient needs to be treated before a written submission can be made)

  - The treatment is initially requested and authorized by telephone (or other means of electronic communication)
  - A written submission is required within 15 working days of the initial authorization and IRB should be notified within 5 working days of treatment initiation

- ***Individual patient expanded access for non-emergency***

  Used when patient does not need be to treated before written submission can be made

  - A written submission to FDA and IRB approval is required prior to treatment initiation

Intermediate-Size Patient Population

- For treatment of more than one patient, but generally fewer patients than are treated under a treatment IND or protocol
- Used to consolidate access to an investigational drug for multiple patients with the same disease or condition
- Can be used when the drug is
  - not being developed for marketing for the same indication as the expanded access use, or
  - being developed but the patients requesting access are unable to participate in the trial (e.g., have a different stage of disease than the one being studied)

Treatment IND and Protocol

- For providing access to large (widespread) populations
- Only used when the drug is being investigated in a controlled clinical trial under an IND to support a marketing application for the same use

OR

- If all clinical trials of the drug are complete and the sponsor is actively pursuing marketing approval of the drug for the expanded access use

Two Paths for Regulatory Submission

Expanded Access IND

- A new IND submission
- Separate and distinct from any existing INDs
- Can be submitted by a patient's physician or the pharmaceutical company or drug manufacturer

Expanded Access Protocol

- Submitted as a protocol amendment to an existing IND
- Generally used when the sponsor of an existing IND (typically the pharmaceutical company or drug manufacturer) is sponsoring the EA use

Who can submit EA requests to FDA?

- Pharmaceutical company or manufacturer: Submits an EA IND, if there is no IND in place, or a protocol to its existing IND (sponsor)
  - The patient's physician acts as the investigator
- Licensed Physician: Submits a new EA IND and acts as the sponsor-investigator

Submission Process: Forms and App

Form FDA 3926

- Streamlined form for EA
- For licensed physicians only
- Can only be used to submit individual patient IND (including emergency use)

Form FDA 1571

- Standard IND form
- Required for the commercial sponsors submitting:
  - An individual patient IND or protocol (including emergency use)
  - Intermediate-size patient population IND/protocol
  - Treatment IND/protocol
- May be used:
  - By licensed physicians
  - For individual patient IND/protocol (including emergency use)

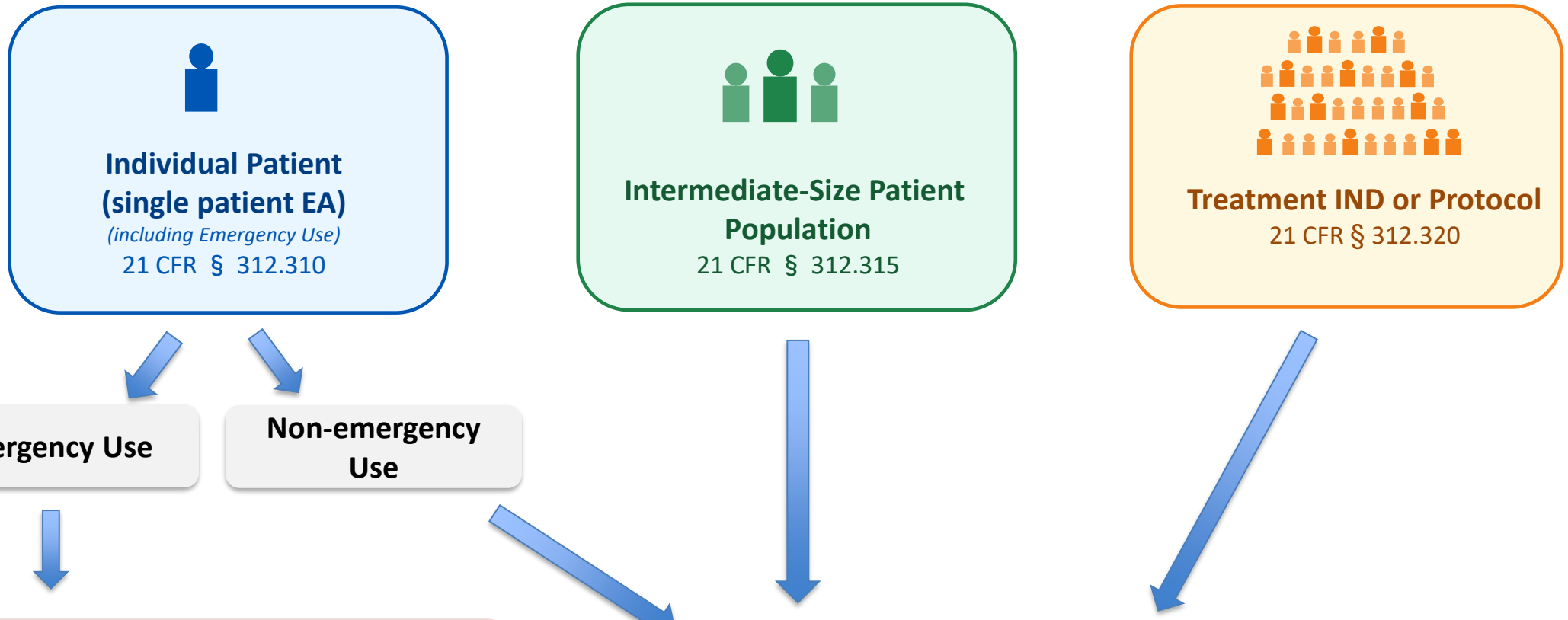
eRequest app

- Web application
- For licensed physicians only
- Can only be used to submit individual patient expanded access IND in non-emergency settings
- Compatible with multiple devices

Submission Process: IRB Review

- IRB review and approval is required before treatment with the investigational drug may begin (21 CFR part 56), unless it is for emergency use
  - Regardless of whether the submission is a new IND or a protocol to an existing IND (21 CFR § 312.305(c)(4))
- The IRB reviews the expanded access use at a convened meeting at which a majority of the members are present (full IRB review) (21 CFR § 56.108(c))
- The IRB's primary purpose is to ensure the rights and welfare of human subjects are protected

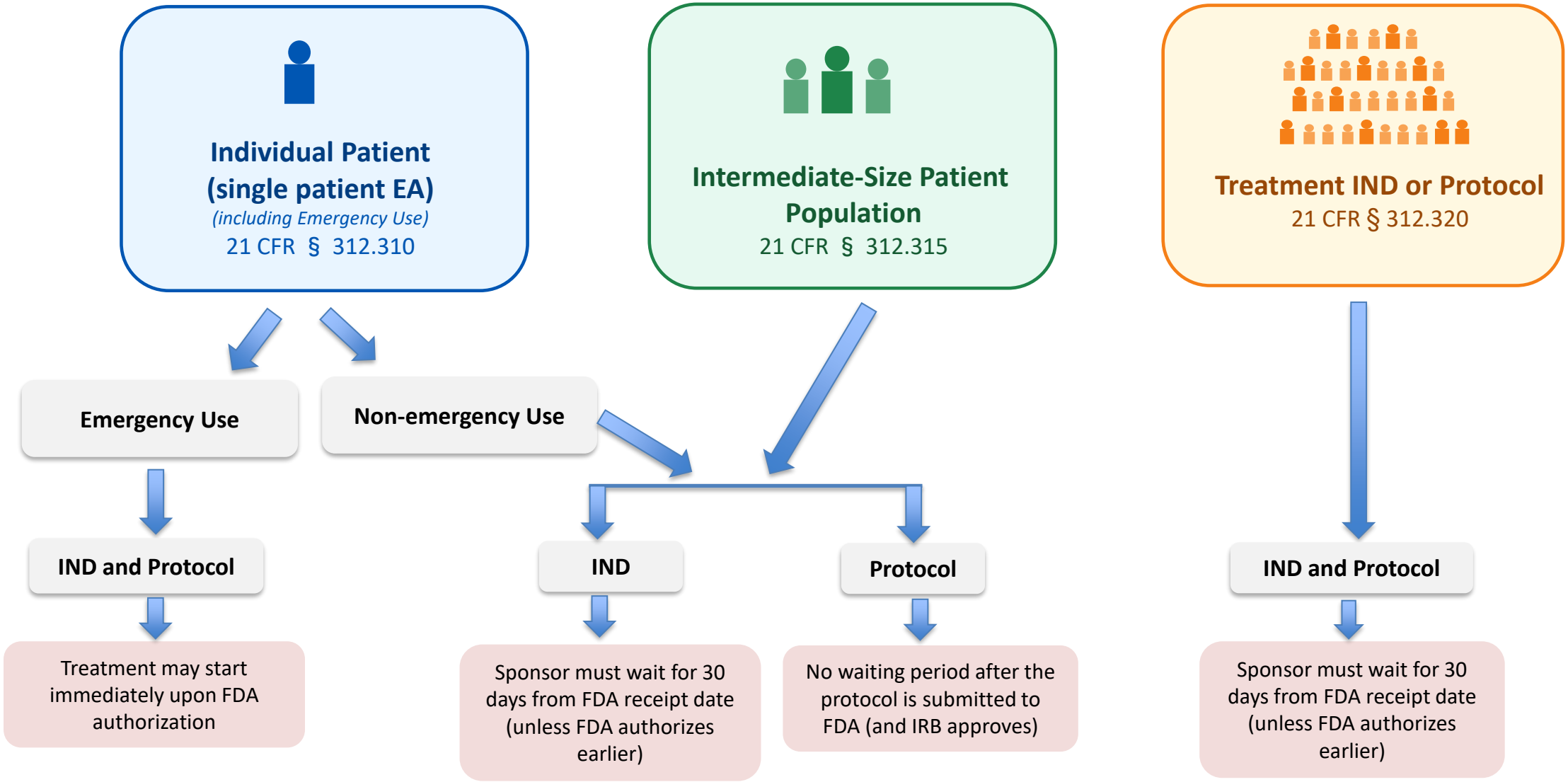
Timing for IRB Review and Approval



Waiver of the Requirement for Full IRB Review and Approval

- Upon request, FDA intends to allow for waivers of the requirement for review and approval at a convened full IRB meeting for individual patient non-emergency EA requests
  - The IRB chairperson or another designated IRB member provides concurrence
- A waiver is appropriate for:
  - initial submission
  - any amendments to the IND
  - continuing review
- A waiver can be requested by checking the box 10b of Form FDA 3926 or by a written request

Submission Process:
Waiting Period Before Treatment May Begin



Informed Consent

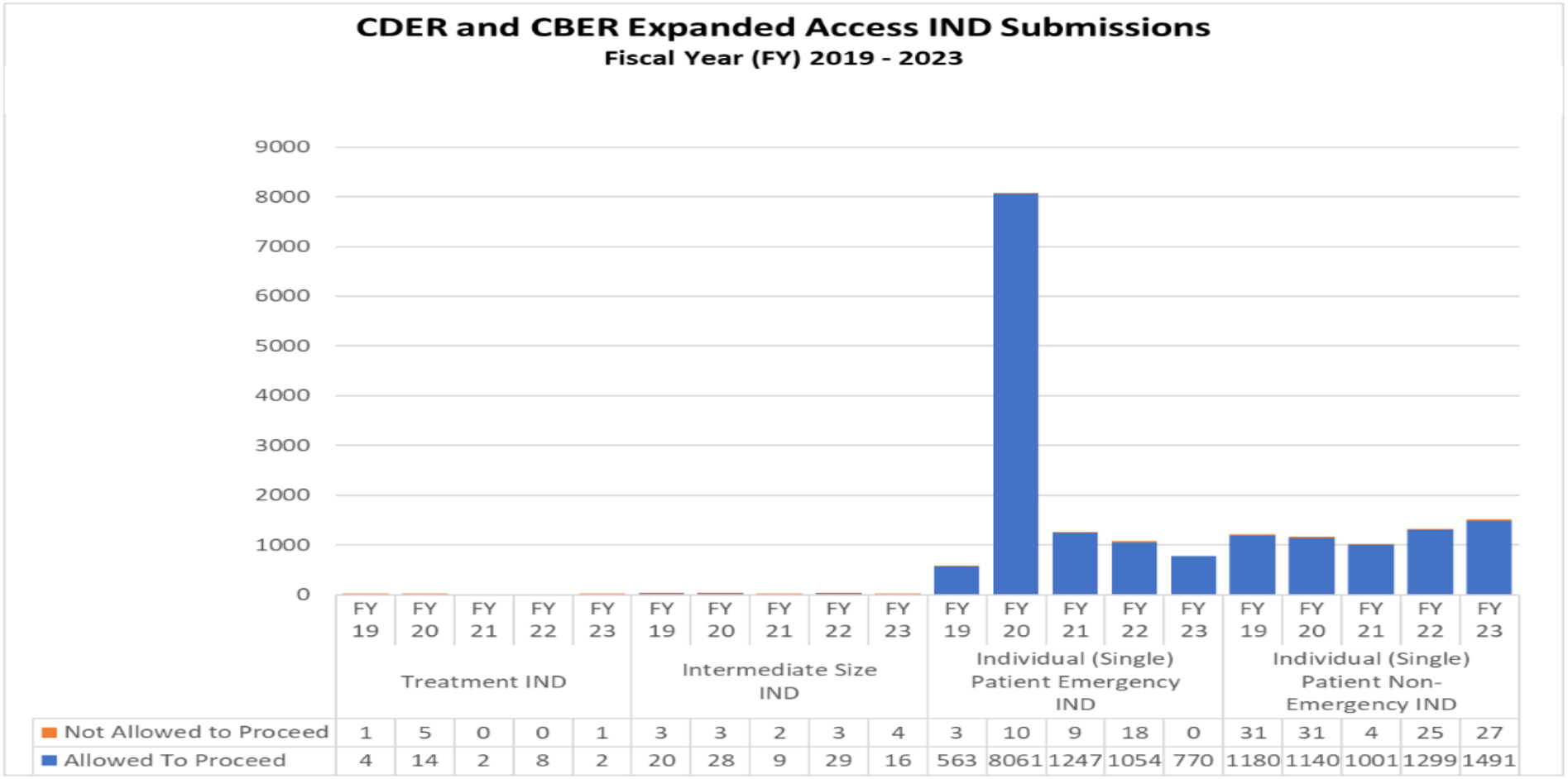
- Informed consent is required (as described in 21 CFR part 50) for expanded access, unless one of the exceptions found in part 50 applies
- The consent form must contain information to enable the patient to make an informed decision about receiving experimental treatment (21 CFR § 50.20 and § 50.25)
- Sponsors are not required to include informed consent as part of an EA submission, unless FDA requests
- The April 2026 guidance includes a template for developing informed consent for individual patient INDs

Expanded Access Policy: Public Availability

- Manufacturers or distributors of investigational drugs must make their policies for evaluating and responding to EA requests readily available to the public
- Timeline for posting: Earlier of the following dates:
  - At the first initiation of a phase 2 or phase 3 study
  - 15 days after the drug receives a designation as a breakthrough therapy, fast track product, or regenerative advanced therapy
- Note: Posting does not guarantee access

How many Expanded Access Requests Are Authorized by FDA?

Combined CDER and CBER Expanded Access Submissions and Protocols (2019–2023)



	Treatment IND					Intermediate Size IND					Individual (Single) Patient Emergency IND					Individual (Single) Patient Non-Emergency IND				
	FY 19	FY 20	FY 21	FY 22	FY 23	FY 19	FY 20	FY 21	FY 22	FY 23	FY 19	FY 20	FY 21	FY 22	FY 23	FY 19	FY 20	FY 21	FY 22	FY 23
Not Allowed to Proceed	1	5	0	0	1	3	3	2	3	4	3	10	9	18	0	31	31	4	25	27
Allowed To Proceed	4	14	2	8	2	20	28	9	29	16	563	8061	1247	1054	770	1180	1140	1001	1299	1491

FDA Expanded Access Program Data

FDA Resources

- Guidance Expanded Access to Investigational Drugs for Treatment Use: Questions and Answers (2026)
- Guidance Institutional Review Board (IRB) Review of Individual Patient Expanded Access Submissions for Investigational Drugs and Biological Products (2023)
- FDA's Expanded Access webpage with information for patients, physicians, industry, IRBs
- Example template for Informed Consent for Individual Patient IND
- Example of wording for letter requesting authorization to use alternative IRB review procedure
- Example of wording for Letter of Authorization (LOA) for individual patient expanded access IND
- Charging for Investigational Drugs Under an IND: Questions and Answers (2024)

Acknowledgements

- Division of Medical Policy Development (DMPD) Staff
- Atasi Poddar (Health Science Policy Analyst, DMPD)
- Dorothy West (Supervisory Health Science Policy Analyst, DMPD)
- Stephanie O. Omokaro (Deputy Director, DMPD)
- CAPT Mathilda Fienkeng (Director, DMPD)
- Mary Than Hai (Director, OND)
- David Faranda (ORP)
- Jean Kim (OMP)
- CAPT Dianne Paraoan (Director, OMP)

Thank you!


FDA
U.S. FOOD & DRUG
ADMINISTRATION

REAGAN-UDALL FOUNDATION FOR THE FDA

FDA & Foundation Resources

Jessica Palfreyman

Senior Program Associate
Reagan-Udall Foundation for the FDA

REAGAN-UDALL FOUNDATION FOR THE FDA

FDA & Foundation Resources

Jessica Palfreyman

Reagan-Udall Foundation for the FDA

What we'll cover

REAGAN-UDALL FOUNDATION FOR THE FDA

01

Expanded Access Navigator

Step-by-step guides, a searchable company directory, and a plain-language resource library

02

Expanded Access eRequest

Completing, signing, and submitting Form FDA 3926 to FDA online

03

Where to go for help

Technical support, FDA contacts, and reference materials

REAGAN-UDALL FOUNDATION for the Food and Drug Administration

ABOUT GUIDES COMPANY DIRECTORY RESOURCES eREQUEST COVID-19

Expanded Access Navigator

Expanded Access (EA) may be considered for patients who have exhausted their treatment options and are not eligible for, or able to participate in, a clinical trial.

Expanded Access eRequest

Physicians: Electronically request non-emergency use of medical products for your patients

GET STARTED

navigator.reaganudall.org

Navigator Tools & Resources

REAGAN-UDALL FOUNDATION FOR THE FDA

Step-by-Step Guides

For patients/caregivers, for physicians/healthcare providers, and for companies/sponsors

Company Directory

Searchable, company-provided expanded access policies, criteria, and contact information

Resource Library

User guides, videos, checklists, and a plain-language guide to ClinicalTrials.gov

Expanded Access eRequest

Complete, sign, and submit Form FDA 3926 to FDA online

Questions? Email navigator@reaganudall.org

Guides tailored to each audience

REAGAN-UDALL FOUNDATION FOR THE FDA

Roadmaps for patients, caregivers, and physicians through the request process and answering the most common questions we get about expanded access

For patients & caregivers

ABOUT

For physicians & healthcare providers

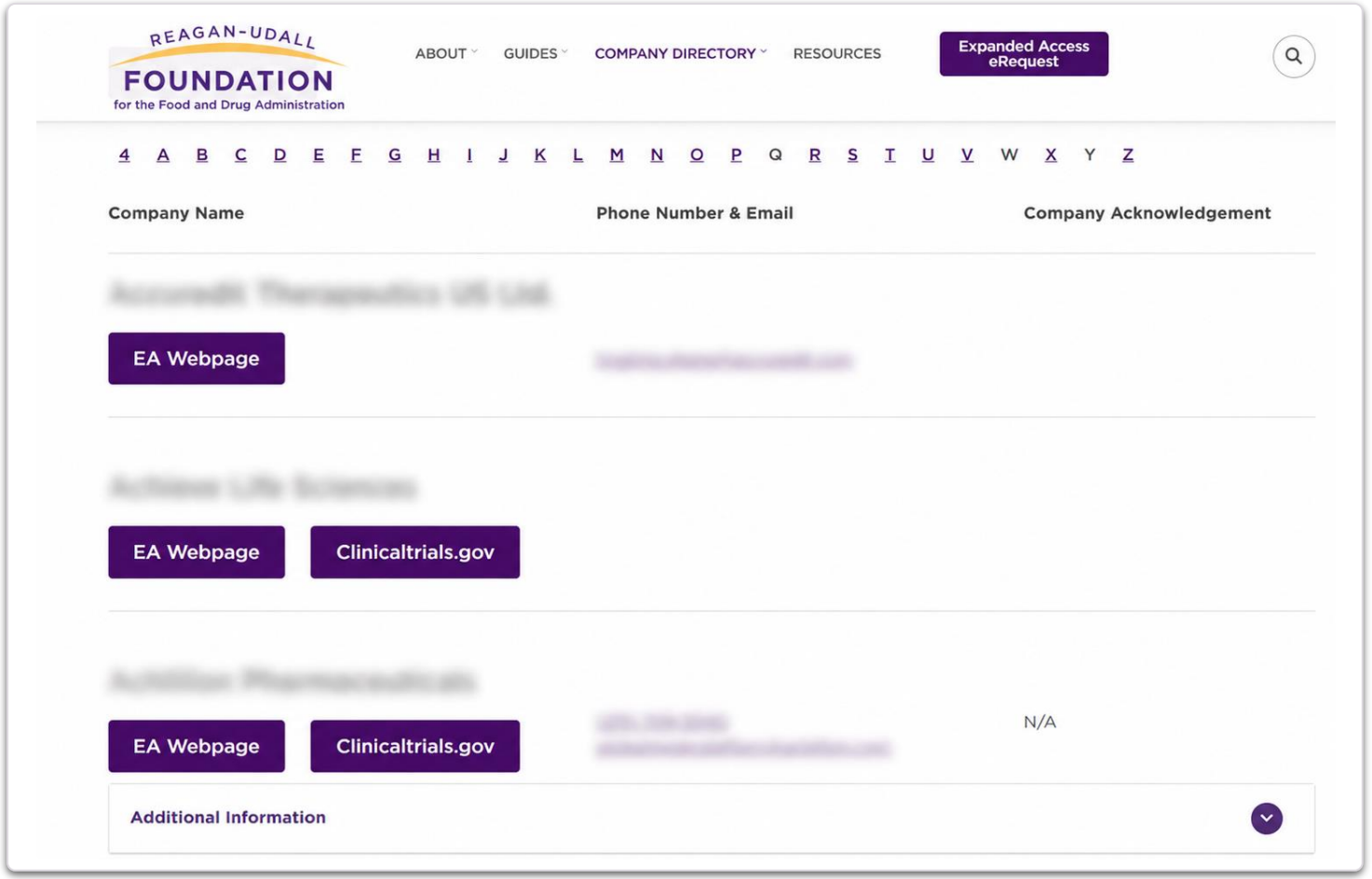
Expande eRec

For companies & sponsors

Select the step-by-step guide best matching your needs.

Company Directory

Searchable, company-provided expanded access policies, criteria, and contacts – with direct links to each company's expanded access webpage and its studies on ClinicalTrials.gov



For patients & providers

Find each company's policy, criteria, and contact – plus links out to their expanded access page and open trials

For companies

Publish expanded access policies & contacts – a way to meet 21st Century Cures disclosure

Resource Library

REAGAN-UDALL FOUNDATION FOR THE FDA

Guidance for every role in the expanded access process

One searchable hub brings together practical resources, forms and trusted guidance—organized around what each audience needs

01 **Patients & caregivers**
Understand options and next steps

02 **Healthcare providers**
Find guidance, support and submission tools

03 **Companies & sponsors**
Explore policies and practical resources

04 **Forms**
Go directly to the documents you need

navigator.reaganudall.org/resources

BROWSE BY AUDIENCE

ALL | FOR PATIENTS/CAREGIVERS | FOR PHYSICIANS/HEALTHCARE PROVIDERS | FOR COMPANIES/SPONSORS | FORMS

Expanded Access eRequest App

Expanded Access eRequest allows physicians and other prescribers to request non-emergency use of medical products for patients. The app allows physicians to complete, sign, and submit Form 3926 online to FDA.
READ MORE »

Glossary of Terms

Active clinical trialA trial open for enrollment of patientsActive, not recruiting (clinical trial)The study is ongoing but new participants are not currently being enrolledAdverse eventA medical problem that happens during treatment with a drug or other therapy, which may be mild, moderate, or...
READ MORE »

Expanded Access to Investigational Drugs for Treatment Use Questions and Answers

Expanded Access to Investigational Drugs for Treatment Use Questions and Answers Guidance for Industry
READ MORE »

Institutional Review Board (IRB) Review of Individual Patient Expanded Access Requests for Investigational Drugs and Biological Products During the COVID-19 Public Health Emergency

FDA plays a critical role in protecting the United States from threats such as emerging infectious diseases, including the Coronavirus Disease 2019 (COVID-19) pandemic. FDA is committed to providing timely guidance to support response efforts to this pandemic. Read FDA's Guidance for IRBs and...
READ MORE »

Patients Matter: What is Expanded Access?

Find answers and hear a family's experience with Expanded Access in this new "Patients Matter" video from FDA.
READ MORE »

Project Facilitate

Project Facilitate assists medical professionals seeking expanded access for patients with cancer FDA's Oncology Center for Excellence is

REAGAN-UDALL FOUNDATION FOR THE FDA

Expanded Access eRequest

Physicians: Electronically request use of medical products for your patients

To submit a new request, you will need to provide patient information and a Letter of Authorization from the manufacturer (or CMC information).

Get Started

Submit emergency IND request for

- Brincidofovir (TEMBEXA) to treat

navigator.reaganudall.org

EXPANDED ACCESS eREQUEST

Submit requests to FDA online

The eRequest app takes physicians screen-by-screen through the process of completing, signing, and submitting a single-patient access request

Guided screen-by-screen

From determining whether expanded access fits the patient to submitting the request to FDA

Initiate non-emergency requests

Start and complete a standard single-patient request online

Upload documentation for emergency requests

Add documentation for authorized emergency requests (with an IND)

Follow-up reminders

Prompts to upload the required follow-up submissions on time

REAGAN-UDALL FOUNDATION FOR THE FDA

Get Started | Submit Application | Contact Us | Resources | Sign In

Registration

Already have an account? Sign In

Personal Information

Name *
Enter Name

Phone *
Enter Phone Number

Fax
Enter Fax Number

Address 1 (Street address, no P.O. box) *
Enter Address 1

Address 2 (Suite, building, floor, etc.):
Enter Address 2

City *
Enter City

State *
Select State

Zip Code *
Enter Zip Code

Country *
USA

Account Information

Email (Username) *
Enter Email

Password *
Enter Password

Confirm password *
Confirm Passsword

I agree to the Terms of Use and Privacy Policy associated with using this portal

REAGAN-UDALL FOUNDATION FOR THE FDA

A one-time setup

Registration collects the physician's information **once** – then reuses it for every future expanded access request, so there's far less duplicated effort

EXPANDED ACCESS eREQUEST

Submitted securely to FDA

REAGAN-UDALL FOUNDATION FOR THE FDA

When the request is complete, the eRequest site submits it securely to FDA — no printing, faxing, or emailing

Complete in eRequest

Fill out and sign Form FDA 3926 online

Packaged & encrypted

The site assembles a secure request package

Delivered to FDA

Submitted securely to FDA for review

Before you submit: have the Letter of Authorization from the manufacturer ready

FDA Resources

REAGAN-UDALL FOUNDATION FOR THE FDA

fda.gov/expanded-access

FDA Key Contact Information

During normal business hours (8 a.m.–4:30 p.m. ET, weekdays)

Investigational drugs
301-796-3400 | druginfo@fda.hhs.gov

Investigational medical devices
301-796-7100 | DICE@fda.hhs.gov

General questions
ForPatients@fda.hhs.gov

Oncology drugs
240-402-0004 | ONCProjectFacilitate@fda.hhs.gov

Investigational biologics
240-402-8020 or 800-835-4709
industry.biologics@fda.hhs.gov

After 4:30 p.m. ET weekdays and all day on weekends
Emergency requests for all medical products: 866-300-4374

Locate IRB Resources

Information about Institutional Review Board review, approval, and reporting

Contact Us

REAGAN-UDALL FOUNDATION FOR THE FDA

navigator.reaganudall.org

Expanded Access Navigator – guides, Company Directory, & resource library, and Expanded Access eRequest

navigator@reaganudall.org

Questions about any of the Foundation's expanded access resources

TECH SUPPORT

erequest@trazeredge.com

Technical questions or problems using the eRequest site

Reagan-Udall Foundation for the FDA · (202) 849-2075
1333 New Hampshire Ave NW, Suite 420, Washington, DC 20036

Stakeholder Dialogue

Patient Advocacy, Academic, Health Care & Industry Experts

REAGAN-UDALL FOUNDATION FOR THE FDA

Alison Bateman-House, PhD, MPH
Associate Professor, NYU Grossman School of Medicine

Mitchell Chan, PharmD, BCPS
Team Lead, Clinical Analyst, Oncology Center of Excellence, FDA

Pat Furlong, BSN, MS
Founding President & CEO, Parent Project Muscular Dystrophy

Allison Martin, MS
Director, Regulatory Science & Policy, Sanofi

Fabian Sandoval, MD
President & CEO, Emerson Clinical Research Institute

REAGAN-UDALL FOUNDATION FOR THE FDA

Expanded Access: Insights & Resources

Thank you for joining!

The webinar recording will be posted on our website at www.ReaganUdall.org

PhRMA provided funding for this webinar

REAGAN-UDALL

FOUNDATION

FOR THE FDA