UNIVERSITY of ROCHESTER MEDICAL CENTER

November SCORE meeting

What Coordinators Need to Know About Regulatory Support

Joan E. Adamo, PhD
Office of Regulatory Support
Wednesday, 20 November 2024

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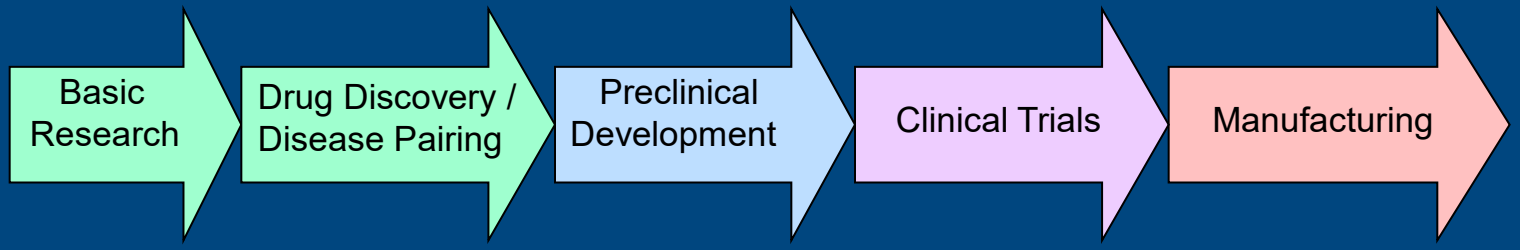
What is the mission of the Office Regulatory Support?



To guide investigators and their study teams through FDA processes

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URMC CTSI
Office of Regulatory Support

https://www.urmc.rochester.edu/clinical-translational-science-institute/clinical-research/regulatory-support.aspx

"Sponsor-Investigator"

Office of Regulatory Support

The Office of Regulatory Support (ORS) offers services to help investigators navigate and comply with a range of governing requirements. In addition to general assistance with requirements as they arise, we provide expertise to support specific FDA-regulated processes, including research involving experimental drugs and devices, as well as preclinical laboratory studies.

Leaders

Joan E. Adamo, Ph.D., Director of the Office of Regulatory Support
Reshma Ravilla, Pharm.D., Regulatory Affairs and Compliance Specialist

reg_support@urmc.rochester.edu

Services & Support

We can help you navigate FDA-regulated processes, including Investigational New Drug and Investigational Device Exemption applications and Expanded Access treatment uses.

Search ORS Services

Education & Career

The UR CTSI has launched a range of programs focused on advancing training, career development, and research in regulatory science.

Explore ORS Education

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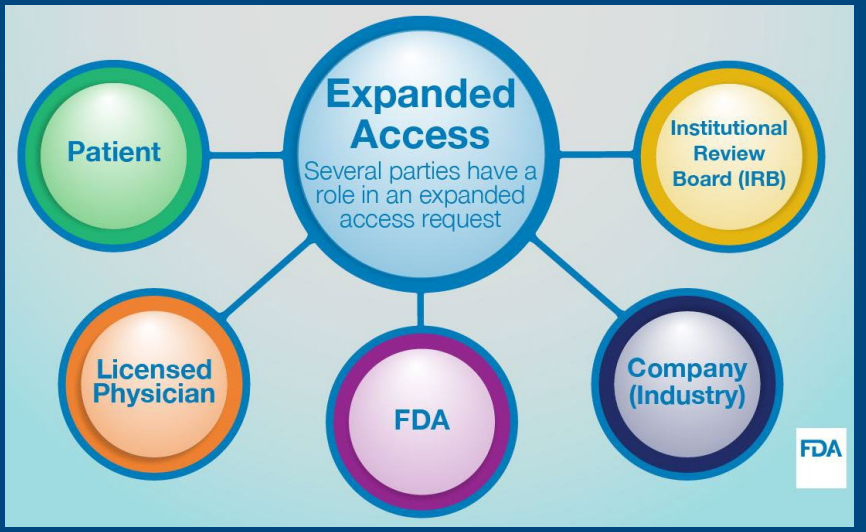
Clinical Trial Process

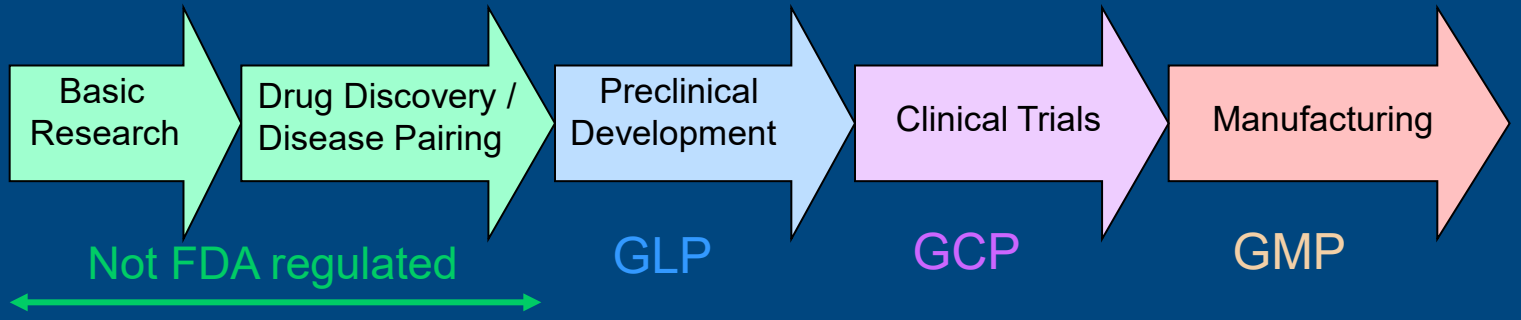
1. Do you need to interact with the FDA?
2. When do you engage the FDA?
3. Do you need an IND (or IDE) to conduct your study?



What is the FDA role in Clinical Trials?

Institutional Review Boards (IRB)
Informed Consent
Good Clinical Practices (GCP)

Good Laboratory Practices (GLP)
Good Clinical Laboratory Practices (GCLP)
Good Manufacturing Practices (GMP)



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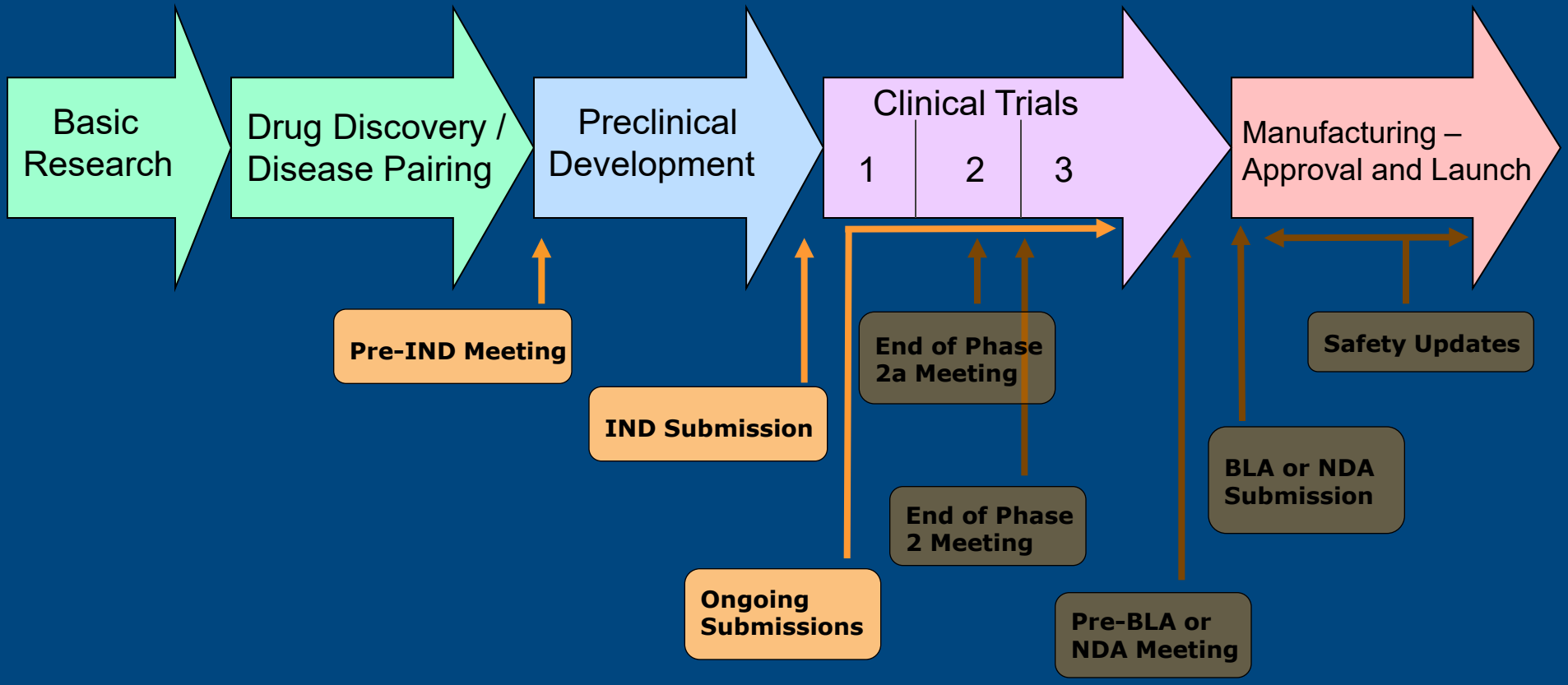
Clinical Research Pathway –
Meetings with the FDA



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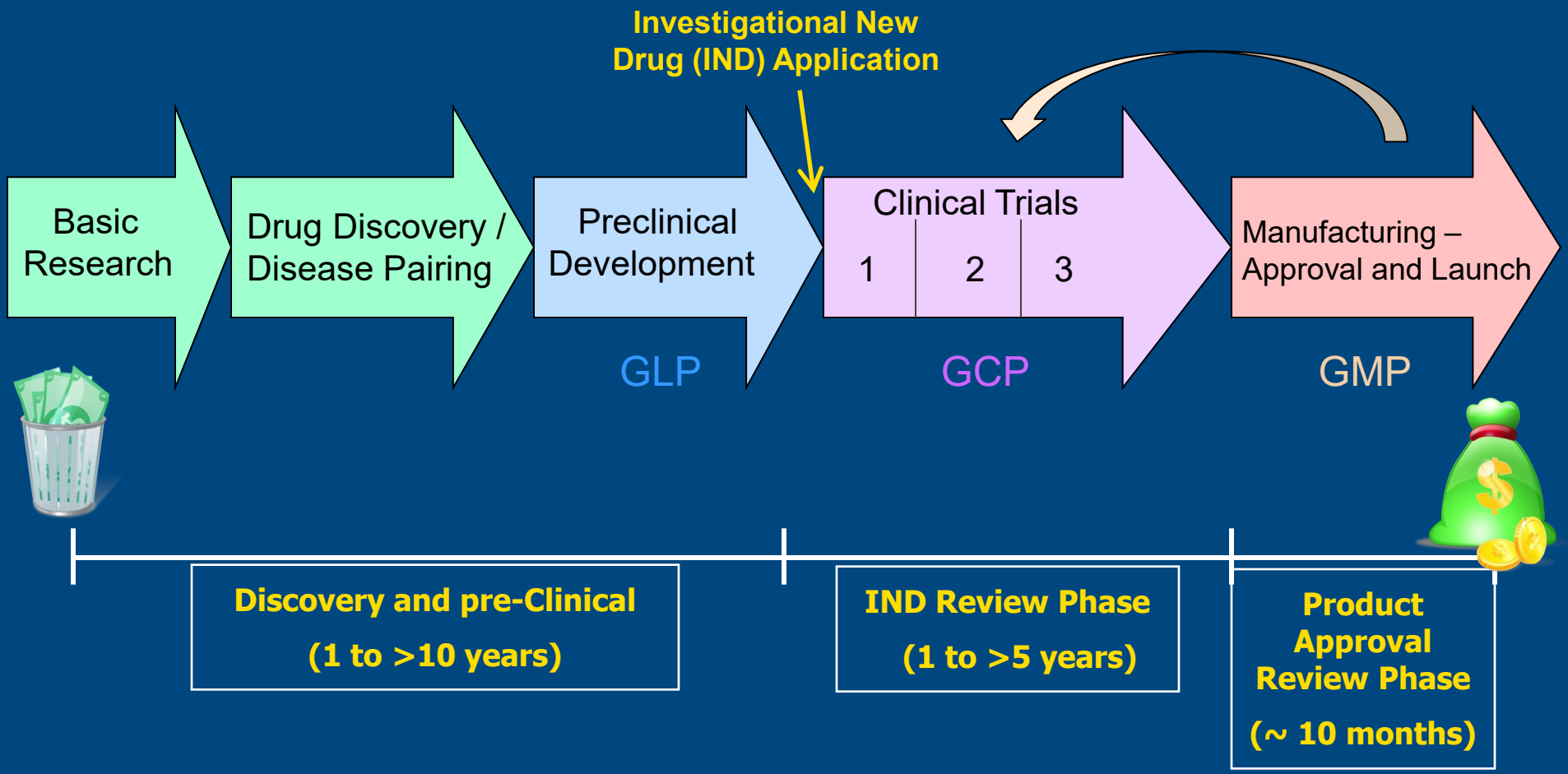
Clinical Research Pathway

(Drugs or Biologics)



Phases for drug studies

(Phases for drug studies are not written in Roman Numerals!)

- Phase 0 – exploratory, often first-in-human, single sub-therapeutic doses
- Phase 1 – initial safety trials, establish dose range, often healthy volunteers
- Phase 2 – evaluate safety and efficacy, ~100-300 patients with disease
- Phase 3 – large, multicenter studies, generate additional efficacy and safety data
- Phase 4 (Post-marketing studies) – often required as a follow-on to approval, further safety, detect / define previously unknown SAE, risks

IND Exemption?

21 CFR 312.2(b)

ALL CONDITIONS MUST APPLY

1. Not intended to change labeling;
2. Not intended to support a change in advertising;
3. Study of the drug product will not significantly increase risk or decrease the acceptability of risk due to a change in the:
   - Route of administration
   - Dose
   - Population
4. Conducted in compliance with the requirements for institutional (IRB) review and informed consent; **AND**
5. Conducted in compliance with rules for the promotion of investigational drugs

Components of an IND

(21 CFR 312.23)

Cover Letter

1. Form **FDA 1571**
2. Table of Contents
3. Introductory Statement
4. General Investigational Plan
5. Investigator's Brochure (for studies with more than one site)
6. Protocol
   a. Study Protocol and Informed Consent
   b. Investigator data – Form **FDA 1572** and CV for the PI
   c. Facilities data
   d. IRB data
7. Chemistry, Manufacturing and Control data (CMC)
   - composition, stability and controls used in manufacture
8. Pharmacology and Toxicology data (GLP standards)
9. Previous Human Experience (if any)
10. Additional Information – Including Form **FDA 3674**

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Components of an IND

(21 CFR 312.23)

Cover Letter
Form **FDA 1571**

This list is greatly reduced for repurposed investigational drug studies!

Study Protocol and Informed Consent
Form **FDA 1572** and CV for the PI

IRB data

Package Insert

Form **FDA 3674**

FDA's 30-day process of non-objection for INDs

SUN	MON	TUE	WED	THU	FRI	SAT
29	30	31	1 ORS uploads IND to FDA	2	3	4
5	6 *May or may not make it to the review division until some time this week*	7	8	9	10	11
12	13 *Potentially insurmountable issues might be communicated in advance . . .*	14	15	16	17	18
19	20	21	22	23	24	25
26	27 FDA internal meeting 	28 	29 	30 30-day review is up	1 Clinical Hold or Enroll	2

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Supplements / Neutraceuticals

Research with a dietary supplement or botanical

- ✓ look at its effects on disease (to cure, treat, mitigate, prevent or diagnose a disease including associated symptoms)
- ➢ then you need to engage the FDA for an IND (or IND exemption) to govern the clinical study

The product is acting as a DRUG!

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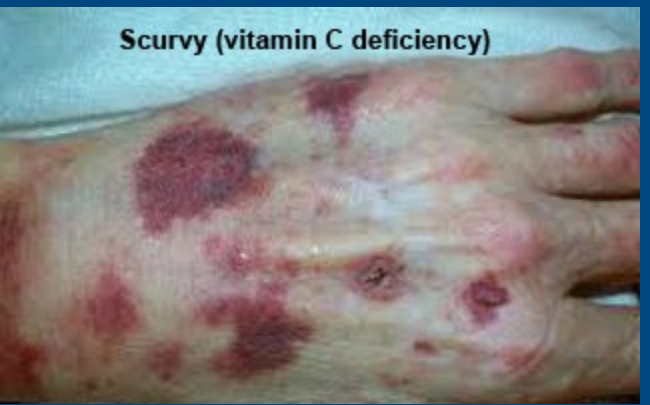
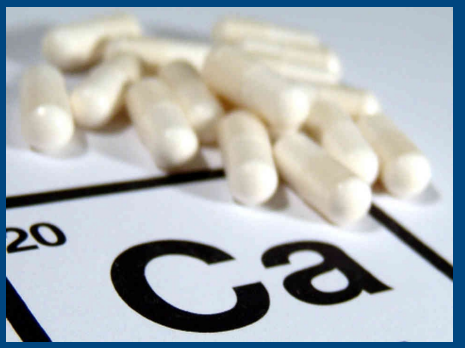
Supplements / Neutraceuticals

Evaluating the product as a dietary supplement

- ✓ structure / function claims
- ✓ then it is not a drug, an IND is not likely necessary

The product is acting as a Dietary Supplement!





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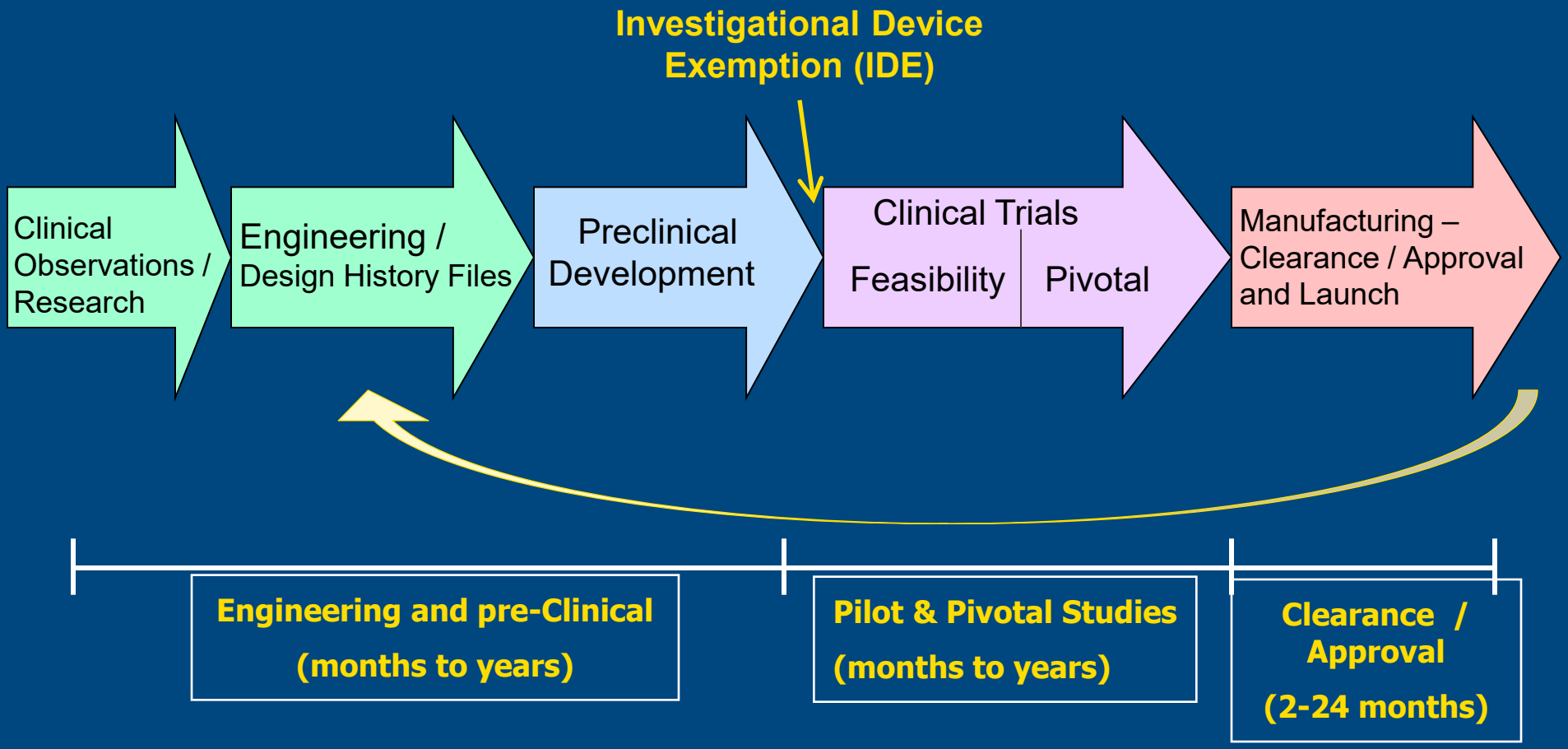
Clinical Research Pathway
(Devices)



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Device concept / prototype → Medical Device → Risk and Device Class Determination

Risk and Device Class Determination → 510(k) Exempt

Risk and Device Class Determination → Is there a substantially equivalent predicate device on the market?

Is there a substantially equivalent predicate device on the market? → yes → 510(k) Premarket Notification

Risk and Device Class Determination → Clinical studies performed under IDE

Clinical studies performed under IDE → Publications and other support → Premarket Approval

Phases for Device Studies

Feasibility Studies – proof-of-concept, initial human experience, small numbers, finalize and confirm device design

Pivotal Studies – multi-center, larger number of patients, use data to support marketing application

- Post-Marketing studies – may be required after approval

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When an IDE is needed

If studying a non-significant risk (NSR) device in an NSR study, then the IRB may potentially serve as the surrogate overseer.

- Abbreviated IDE or 'considered approved' IDE

If the device is classified as a significant risk (SR) device, then it is necessary to file an IDE with the FDA.

Investigational Device Exemption (IDE)

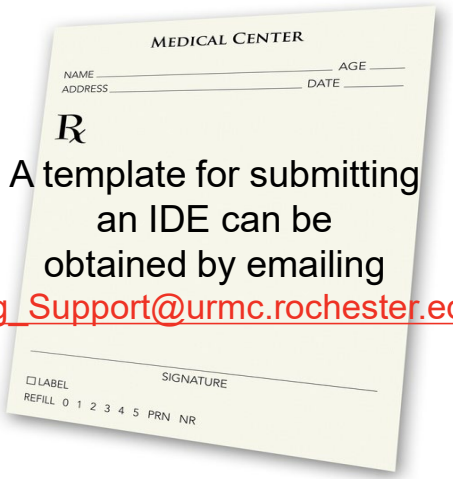
Governed by 21 CFR part 812

Clinical Investigation of a Medical Device – to determine safety and effectiveness

Can be filed with the FDA or can be filed with the local IRB

IDE Application Contents (21 CFR 812.20)

- ✓Cover letter
- ✓Table of contents
- ✓Report of prior investigations
- ✓Investigational plan
- ✓Manufacturing information
- ✓Investigator information
- ✓Sales information
- ✓Environmental impact statement
- ✓Labeling
- ✓Informed consent materials



A template for submitting an IDE can be obtained by emailing Reg_Support@urmc.rochester.edu

Submitting an IDE

eCopy submit your complete IDE application to the CDRH document control center

- FDA has 30 days to review
- Approve, Disapprove or Approve with Modifications

Common reasons for disapproval:

- Risks outweigh the potential benefits
- Inadequacy of a critical step
- Failure to respond to FDA request for information

Expanded Access

What about Expanded Access or Compassionate Use?

Expanded Access

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Sometimes called “compassionate use”, expanded access is a potential pathway for a patient with an **immediately life-threatening condition or serious disease or condition** to gain access to an **investigational medical product** (drug, biologic, or medical device) for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available.

Expanded Access

https://redcap.urmc.rochester.edu/redcap/surveys/?s=7RCWF4TC3N

Expanded Access

Expanded access, sometimes called compassionate use, is the use of an investigational medical product that has not been approved by the FDA outside of a clinical trial. The FDA allows these uses on a case-by-case basis, when submitted by a qualified physician. The University of Rochester has formalized a process for requesting use of a non-FDA-approved investigational drug outside of a clinical trial that expedites the coordination of all groups that need to be involved.

Request Expanded Access

Jump to: Features Get Started Contact
Return to: Services and Support Directory Office of Regulatory Support

Features

In order for the FDA to permit expanded access to an investigational drug, all of the following criteria (found in 21 CFR 312.305(a)) must be met:

- The patient to be treated has a serious or immediately life-threatening disease or condition, and there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition.
- The potential patient benefit justifies the potential risks of the treatment and those potential risks are not unreasonable in the context of the disease or condition to be treated.
- Providing the investigational drug for the requested use will not interfere with the initiation, conduct, or completion of clinical investigations that could support marketing approval of the expanded access use or otherwise compromise the potential development of the expanded access use.

This process involves the careful coordination of information with several groups at the University:

- Office of Regulatory Support
- Office of Research and Project Administration
- Office of Counsel
- Research Subjects Review Board

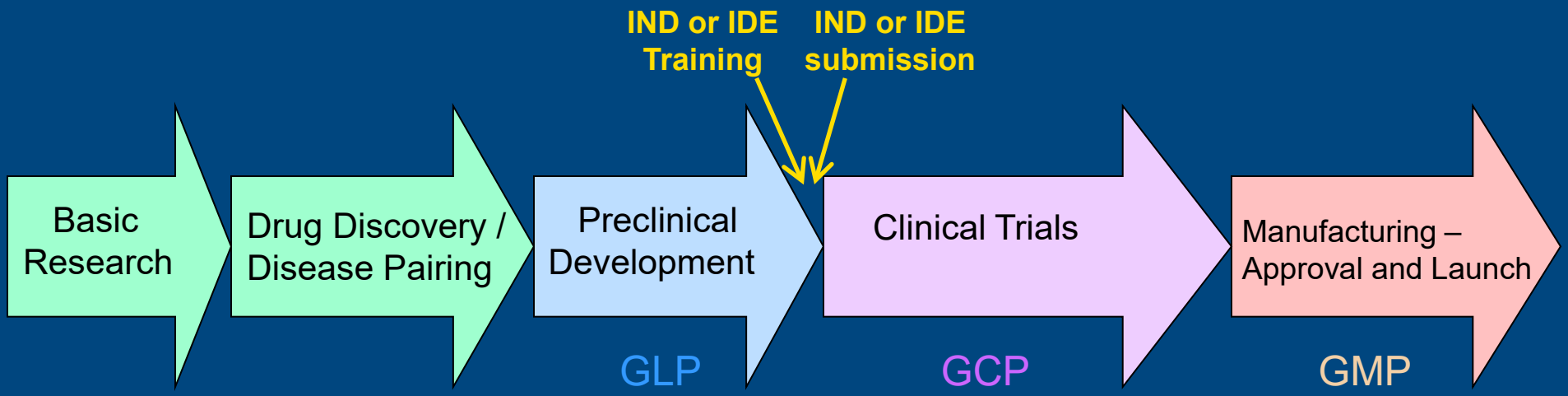
bit.ly/Expanded-Access

Get Started

To use a non-FDA-approved drug through the expanded access pathway, please submit an Expanded Access Application. Someone from the Office of Regulatory Support will be in touch with you soon to provide assistance so that the regulatory process and reporting obligations are understood and fulfilled.

Online Training - MyPath

Clinical Research Pathway



IND Training: "Orientation to the Requirements for Filing an Investigational New Drug (IND) Application"

IDE Training: "Orientation to Medical Devices and the Requirements for an FDA Investigational Device Exemption (IDE)"

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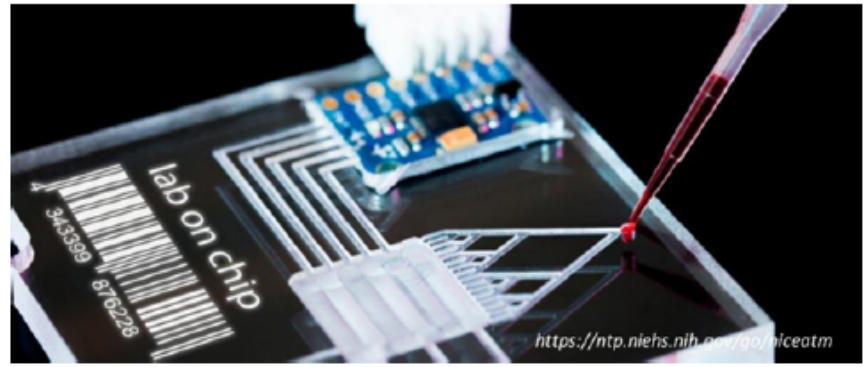
America's Got Regulatory Science Talent

Student Competition



Open to all university students

Competition is a 5-minute presentation

Propose a solution to a Regulatory Science Focus Area

Wednesday, 4 Dec 2024 @ 12noon

School of Nursing HWH Auditorium

This annual competition provides an opportunity for students to develop and present a proposed solution to a current FDA regulatory science need in areas ranging from personalized medicine and digital health to advanced manufacturing and risk communication.

Current FDA regulatory science Focus Areas can be found here.

Solutions will focus on the science of developing new tools, standards, and approaches to assess the safety, efficacy, quality, and performance of FDA-regulated products.

For registration, competition guidelines, examples of proposed solutions, and access to faculty or FDA advisors, visit the America's Got Regulatory Science Talent page.

WHEN & WHERE:

Brief entry form due by 5:00 p.m. on Wednesday, November 6, 2024

Competition at noon on Wednesday, December 4, 2024
School of Nursing Auditorium (1W-304)
Lunch provided

Winning team may present findings at the FDA in Spring 2025

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Co-Sponsored by:
University of Rochester
Clinical & Translational Science Institute

University of Rochester
Center for Medical Technology & Innovation

University of Maryland
Center of Excellence in Regulatory Science and Innovation

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Top Five ORS Requests

5. Do I need an IDE for this study?

4. Can I treat this patient without going to the FDA?

3. Can I get a template for my IND Annual Report?

2. The IRB says to talk to you, but I do not think I need an IND for this study.

1. Do I need an IND for this study?

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