ROADMAP 101 Warm-up:
An Introduction to Drug Development

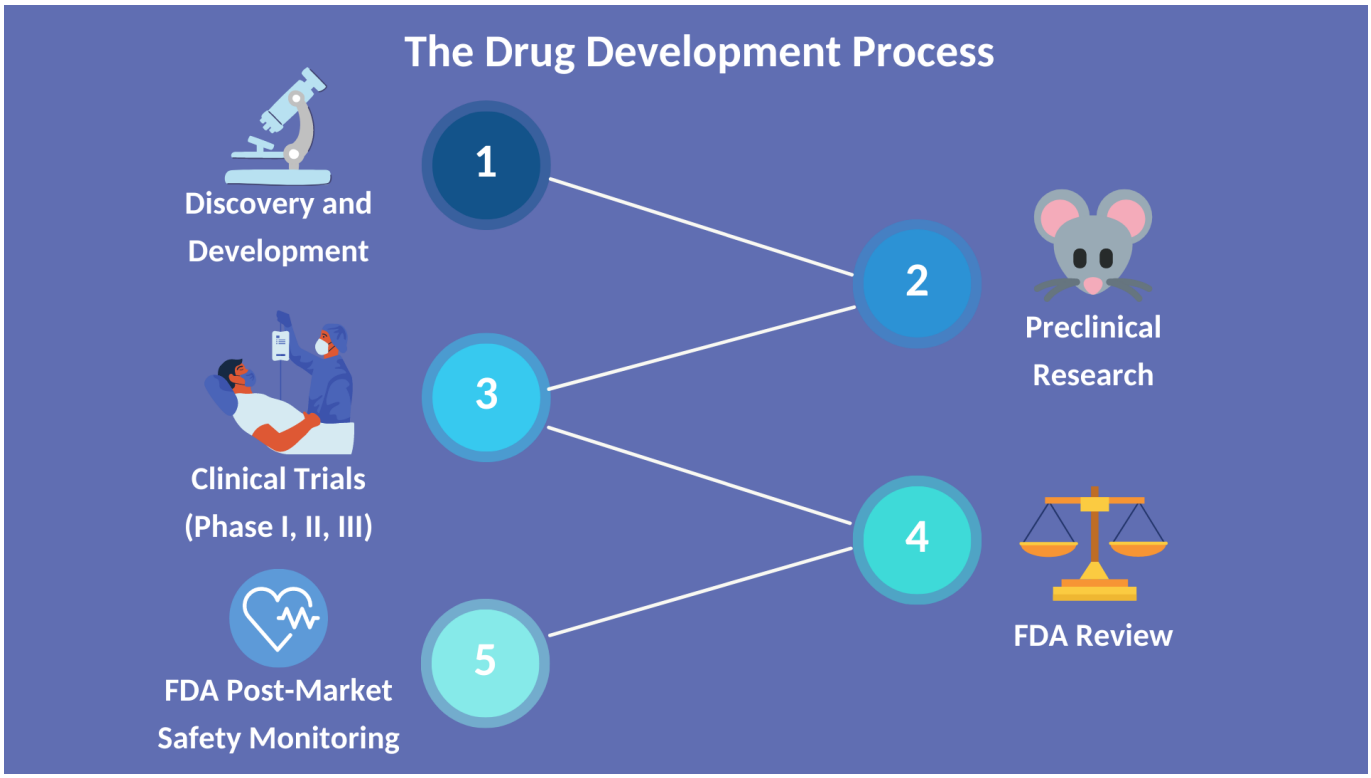
During the ROADMAP 101 session on December 8th, Frank Sasinowski will provide us with some basic terminology and concepts in drug development. Afterwards, we'll have time in breakout rooms to ask questions of the experts. We hope you will leave the Drug Development 101 session with a clear understanding of the process required to develop a drug as a treatment for PSC. Then, during the Drug Development 201 session on January 11th, we will discuss the challenges that our community faces in drug development and chart a path forward on our pursuit of a cure for PSC.

PSC Partners put this document together as a reference to get us started.

What is "drug development"?

Drug development refers to the process that brings a drug to patients in order to meet an unmet medical need. Oftentimes, the goal is for a drug to receive "FDA approval".

While there are multiple pathways to develop a treatment for PSC, most drugs will be developed using the following five steps[1]:



What is the FDA?[3]

The Food and Drug Administration (FDA) is a government regulatory agency. The FDA regulates certain food, drugs, cosmetics, and medical products.

The FDA is also responsible for advancing public health by "helping to speed innovations that make medical products more effective, safer, and more affordable and by helping the public get the accurate, science-based information they need to use medical products and foods to maintain and improve their health."[3]

<u>All of this is to say that the FDA plays a critical role in drug development.</u>
While the FDA is based in the US, other regulatory agencies exist worldwide, such as the European Medicines Agency (EMA) in Europe.

Who are the players driving drug development for PSC? What do they do?

Patients: Patients are at the center of the drug development process. They provide biosamples to enable research, communicate unmet needs, participate in clinical trials, and provide valuable feedback to guide drug development.

Scientists: Scientists perform experiments to identify and study drugs (Step 1), measure safety in experimental models (Step 2), and provide expertise to analyze and interpret results throughout the drug development process (Steps 3-5).

Pharmaceutical Companies: Pharmaceutical companies may develop drugs (Steps 1-2) and decide whether to pursue clinical trials to test the safety and efficacy of the drug in humans for a given condition, like PSC (Step 3). These companies invest very large funds towards clinical trials as they seek FDA approval for their drug (Step 3). Importantly, pharmaceutical companies rely on all other stakeholders in order to successfully develop a drug.

Who are the players driving drug development for PSC? (continued)

Regulatory Agencies: Once there is sufficient data to suggest a drug may be safe in humans, pharmaceutical companies may submit paperwork to the FDA to initiate a clinical trial to test the safety and efficacy of the drug to treat a specific condition, like PSC (Step 3). The regulatory agency sets standards, reviews conditions of the study, and ensures the safety and protection of public health throughout the drug development process. Once the trial is completed, data are submitted by the pharmaceutical company and the FDA decides whether there is enough evidence that the drug is safe and effective to receive full FDA approval[2] for the specific condition (Step 4).

Clinicians: Clinicians, or medical providers, are trained to care for patients and are often involved in recruiting patients to join clinical trials (Step 3). Oftentimes, patients will receive the experimental drug from this clinician and the clinician will submit important data to the pharmaceutical company. This then contributes to a growing body of evidence about the safety and efficacy of the experimental drug in question, which may be reviewed by the FDA (Step 4).

Patient Advocacy Groups: Finally, let's discuss the role that PSC Partners plays in this process. Patient groups support clinical trial design, patient recruitment, physician education, and magnify patient needs and patient voice. PSC Partners has expanded our efforts to support every step of the drug development process through our research programs. We look forward to sharing more at the Drug Development 201 session.

Additional Resources to Explore:

1. Tell me more about each step of the Drug Development process
2. What does "FDA approval" mean?
3. Tell me more about the FDA