FDA Expanded Access Program

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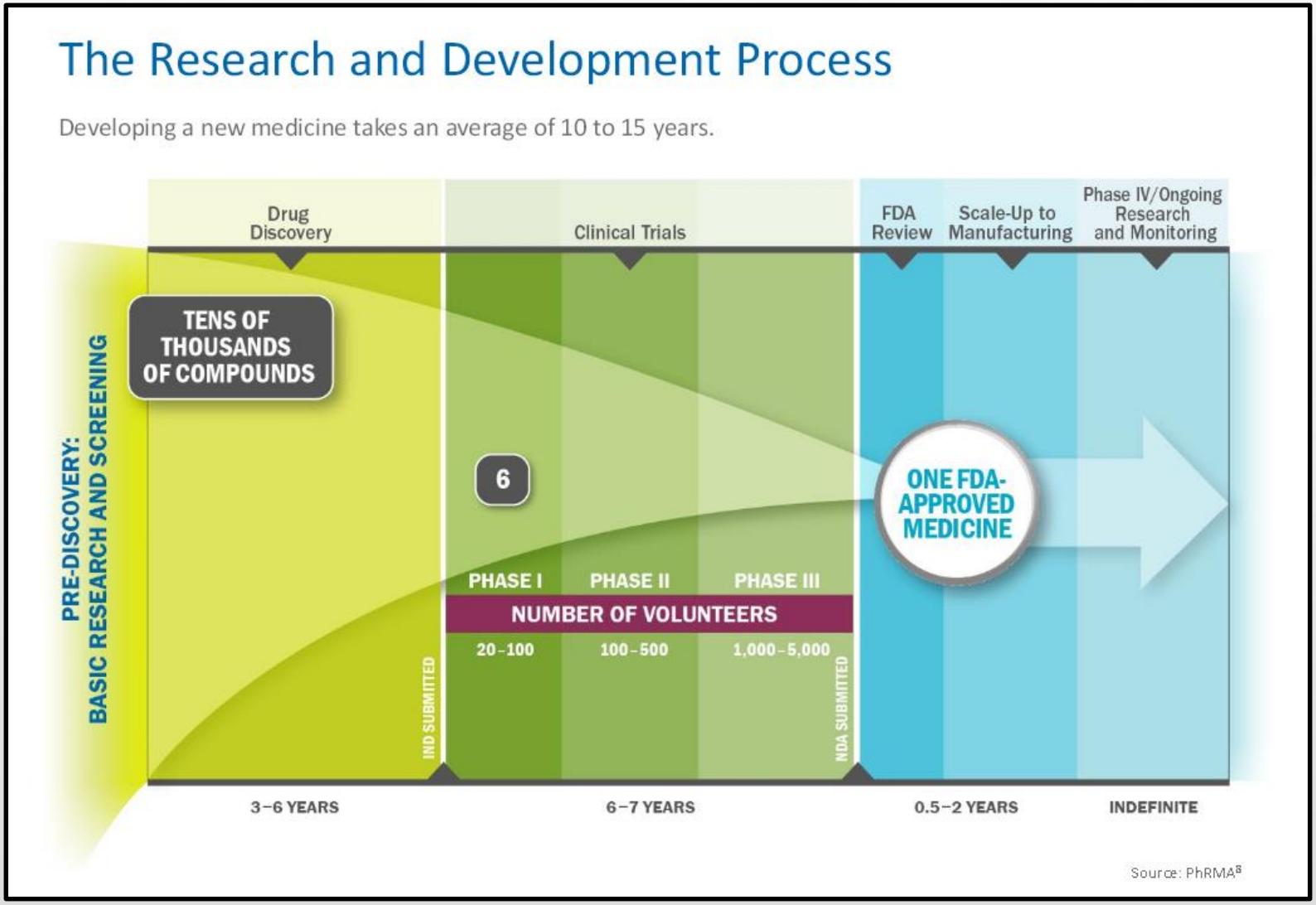
The Research and Development Process



Existing U.S. Expanded Access Process

- “Expanded access, sometimes called "compassionate use," is the use of an investigational drug outside of a clinical trial to treat a patient with a serious or immediately life-threatening disease or condition who has no comparable or satisfactory alternative treatment options.”[1]
- U.S. Food and Drug Administration (FDA) regulations establish three levels of expanded access programs depending on the size of the population seeking treatment use: single patient INDs, intermediate-size patient populations, and large patient populations (widespread use)

[1] http://www.fda.gov/ForConsumers/ByAudience/ForPatientAdvocates/AccesstoInvestigationalDrugs/ucm176098.htm

FDA Criteria for Expanded Access

Patients may obtain an unapproved, investigational drug for treatment use if specific criteria are met:

- The patient's physician determines that the patient has no comparable or satisfactory alternative therapy;
- The potential patient benefit justifies the potential risks of the treatment, and those risks are not unreasonable in the context of the condition being treated;
- FDA determines that there is sufficient evidence of safety and effectiveness to support use of the investigational drug;
- FDA determines that providing the investigational drug will not interfere with the initiation, conduct, or completion of clinical investigations that could support marketing approval; and
- The sponsor or clinical investigator submits information sufficient to satisfy the investigational new drug (IND) requirements.

Role of the Biopharmaceutical Company

- The initial step is for a patient's treating physician and medical staff at a biopharmaceutical company to determine whether expanded access to an investigational drug is the best possible treatment option for the individual patient
- The biopharmaceutical company must also determine whether expanded access would negatively impact the clinical development process, e.g. whether there is a sufficient supply of the investigational drug available
- If those conditions are met, the biopharmaceutical company or the patient's treating physician would submit an expanded access application to the FDA

Stakeholders Share the Same Goals

- Bring new, safe and effective medicines to patients as quickly as possible
- Identify ways to modernize the clinical trial, drug development, and FDA review processes to accelerate the availability of new medicines
- Help patients and physicians understand and navigate the full range of potential treatment options, including approved medicines, clinical trials, and expanded access, when appropriate

Developing New, Safe & Effective Medicines for Patients
Key Points to Consider – Expanded Access

- FDA approval remains the best way to ensure that new, safe and effective medicines are available to patients
  - Successful completion of the clinical trial process is required to demonstrate to the FDA that an investigational drug is safe and effective so that it can be made available to a broader patient population
  - Clinical trials are the primary route by which patients can participate in the drug development process, receive access to unapproved investigational drugs, and contribute to the collection of safety and efficacy data necessary for FDA approval
- For patients with a serious or life-threatening disease who are ineligible or unable to participate in a clinical trial, use of an unapproved investigational drug via an expanded access program may be an option
- Expanded access programs are part of the biopharmaceutical industry's commitment to patients. A variety of challenges must be addressed to avoid delays in the clinical development and FDA approval of new medicines for broader patient populations

Matching the Urgency of the U.S. Regulatory System to the Urgency of Patients' Unmet Medical Needs

- The development of new, safe, and effective medicines for serious or life-threatening diseases represents an urgent and unique challenge that requires special attention
- Current proposals to expand usage of unapproved investigational drugs jeopardize the FDA's critical role in the drug approval process and may undermine the essential process of investigating a drug's safety and effectiveness in a systematic way
- All stakeholders - patients, physicians, FDA, biopharmaceutical companies, academia, and policymakers at the state and federal level - must work together to optimize the federal expanded access process
- Patients facing serious and life-threatening diseases or conditions deserve earlier access to approved medicines that have satisfied FDA's standards for safety and effectiveness based on early stage data sets in areas of unmet medical need