The Drug Development Process

Patients and caregivers play an integral role in the drug development process. In rare disease, this role is particularly important because it can be especially difficult to develop and bring new investigational therapies to the market.

Once researchers identify a compound that has potential to benefit patients with a particular disease or condition, the development process begins.

BARRIERS TO RARE DISEASE DRUG DEVELOPMENT

What are Some Barriers to Patients Participating in Clinical Trials?

- Small and often diverse patient population
- Limited (or sometimes lack of) awareness, education, and understanding of the disease
- Few medical experts

Preclinical Trial Phases

Basic Research
- Select a compound that is efficacious in basic research for the target disease.

Preclinical Development
- Conduct extensive testing of the efficacy and safety of the compound.

Clinical Development
- Engage doctors and study centers to conduct clinical trials. Gain approval from their respective Institutional Review Boards (IRBs) and regulatory agencies.

Clinical Trials

Clinical Trial Phases

Phase 1: Is the Treatment Safe?
- Test the safety of the investigational therapy, often in healthy volunteers (not pateints with the specific disease)

Phase 2: Does the Treatment Work?
- Test the safety and efficacy of the investigational therapy in people with the disease.

Phase 3: Is the Treatment Better than What is Already Available?
- Compare outcomes of participants receiving the drug to those receiving a placebo or a different therapy

Regulatory Agency Submission
- If shown to be safe and effective, the regulatory agency may approve the investigational therapy for doctors to prescribe

Phase 4

APPROVED FOR PATIENT USE

Often referred to as “post-marketing monitoring,” the company continues to monitor the drug’s safety and effectiveness to keep track of long-term results.

Programs to Accelerate Approval

The Food and Drug Administration (FDA) in the U.S. and European Medicines Agency (EMA) in Europe have programs in place to help speed up the approval process of investigational therapies for diseases with high unmet medical need. Some examples include:

Fast Track Designation – Rolling reviews to help expedite the process.

Breakthrough Therapy Designation – Intensive guidance on efficient drug development and organizational commitment involving senior managers.

Accelerated Approval Pathway/Conditional Approval – Approval conditional on providing additional post-approval data. After confirmation, authorization is converted to a normal approval.

Approval Under Exceptional Circumstances – Approval with the understanding that complete data can never be provided.

Priority Review Designation/Accelerated Assessment – Reduced review time.

The drug development process is long, expensive, and requires a team working in tandem:

Researchers

Physicians

Academic centers

Biopharmaceutical companies

Patients

Families

Patient organizations

YOU ARE THE MOST POWERFUL VOICE!

Are you interested in learning how you can get involved? Check out www.ohf.org for how you can participate in the drug development process!

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