A new medicine takes, on average, 10 to 15 years to move from initial research through the approval of regulatory authorities such as the U.S. Food & Drug Administration (FDA) and European Medicines Agency (EMA). In the United States, the average cost to research and develop a successful drug has been estimated at $2.6 billion.¹

The discovery process includes the early phases of research to identify an investigational drug and perform initial tests in a laboratory. This first stage can take three to six years. By the end, researchers hope to identify a promising drug candidate and conduct tests for safety and effectiveness both in the lab and in animal models. If these early studies show promise, the FDA gives approval to move to testing in humans, known as clinical trials.¹

Clinical trials are conducted in three phases to test a treatment, find the appropriate dosage and uncover side effects. Clinical trials alone can take six to seven years and may require the participation of thousands of people. Because clinical trials must produce positive results related to safety, side effects and efficacy, less than 12% of the possible medicines that enter the first phase of clinical trials are approved by the FDA.¹

Successful drug development requires immense resources — the best scientific minds, sophisticated technologies – and very importantly, engaged patients.

As essential stakeholders in the drug development process, patients, family members, caregivers and patient advocacy organizations can provide unique and valuable perspectives on what it is like to live with a disease. Understanding what’s most important to a patient and incorporating these viewpoints into early stage research and clinical trials through FDA approval can help ensure new therapies are developed that truly meet patient’s needs.

Recently, there has been a significant increase in the study of gene therapies, many of which are being researched to treat rare and serious genetic diseases. The FDA predicts between 40 to 60 gene therapy products will be launched by 2030, leading to new treatments for more than 500,000 people.²

Speeding up the availability of drugs that treat serious diseases is in everyone’s interest. For gene therapies and other drugs that are intended to treat rare conditions, the FDA has created programs to accelerate the development and review of new medicines.

For example, as opposed to the thousands of patient volunteers needed to participate in a clinical trial for a more common condition like hypertension or diabetes, the FDA may approve a new therapy for a rare disease with fewer clinical trial participants because the patient population is so small. In many of these cases, the FDA may require post-approval monitoring of clinical trial participants to make sure the drug continues to be safe and effective long-term.

No matter the condition or therapy being studied, patients are the experts when it comes to their own health conditions. And this expert perspective is critical to advance therapies.

Incorporating Patient Experience into the Research and Development Process

During the early phases of research, scientists want to learn about patient and caregivers’ experiences with a disease or condition and gather information such as:

- Symptoms of the condition and how it has progressed
- Impact on quality of life
- Individual experience with treatments
- Impacts from treatment on activities of daily living
- Preferences for outcomes and treatments

Patient input is also incorporated into each phase of clinical trials to make it easier for a patient to participate and remain engaged with the clinical trial. Patients and patient advocacy organizations can help by:

- Providing input on clinical trial site locations
- Reviewing the timing of study procedures and number of study visits
- Making recommendations on patient education material format and design
- Supporting recruitment for clinical trials to ensure there is broad representation from all demographics

During FDA review:

- Patient and caregiver input regarding a medicine’s benefits and risks informs the FDA’s regulatory decision making

New medicines are developed that better reflect patient and caregiver needs.

Learn More

Your health care team can be an excellent resource to start the discussion about identifying and participating in clinical trials. Patient advocacy organizations can also share information about clinical trials in which they have helped provide the perspective of patient communities to researchers and the FDA.