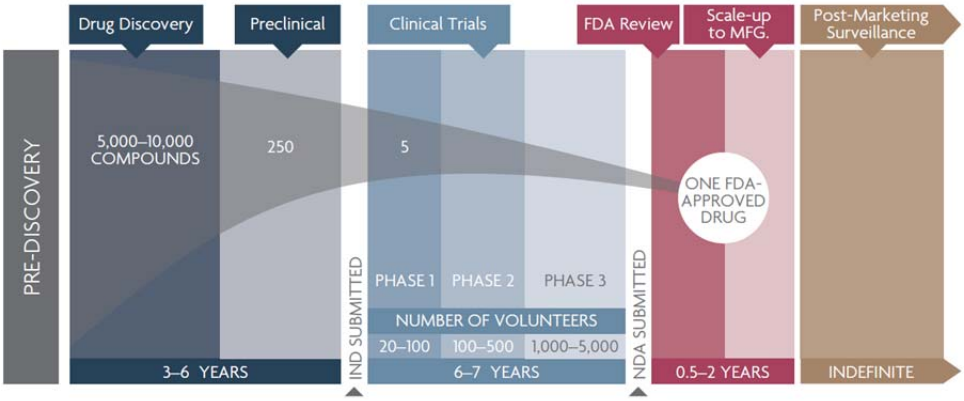
Clinical Trials and the Development of New Medicines

Developing new therapies to treat diseases and improve quality of life is a long and complex process that is regulated by the U.S. Food and Drug Administration (FDA) and its counterparts in other countries. A critical part of that process is clinical development – the study of potential new medicines in humans. Clinical trials are carefully controlled tests of new medicines designed to evaluate their safety and effectiveness and compile the evidence needed by the FDA and other regulators around the world to approve new treatments.



The modern clinical trial program is the most scientifically rigorous and clinically productive in history, requiring carefully balanced and regulated collaboration among sponsoring companies, regulatory agencies, investigative site staff, medical professionals, hospitals, academia, institutional review boards and ethics committees. Clinical testing of new medicines for approval is conducted in three phases in which the number of clinical trial participants increases as investigators learn more about the medicine under investigation. At the beginning of a clinical trial and throughout the testing itself, companies and clinical investigators inform the FDA and other regulators about plans, outcomes and any issue regarding the trial. Even after a new therapy reaches the market, companies are required to submit periodic reports to the FDA, including all cases of adverse reactions and new information learned about the medicine. For some medicines, clinical studies may be required after it is approved for marketing in order to evaluate safety and efficacy in a broader patient population and over a longer period of time than what is required in the pre-approval studies.

The Productive Clinical Trials Process

- The biopharmaceutical research sector is the global leader in medical innovation, with more than 340 new medicines approved by the Food and Drug Administration in the last decade, and 39 in 2012 alone.
- More than 5,000 new medicines are in the pipeline globally. Of these medicines in various phases of clinical development, 70 percent are potential first-in-class medicines, which could provide exciting new approaches to treating disease for patients.

Source: Analysis Group report, "Innovation in the Biopharmaceutical Pipeline: A Multi-Dimensional View." 2012.

Clinical trials involve the participation of thousands of volunteer patients and the generation of thousands of pages of scientific, medical and technical data. They account for seven of the 10 to 15 years and 45 to 75 percent of the $1.2 billion average cost of developing one new cutting-edge medicine.[i]

In accordance with law, PhRMA member companies post information on new trials for patient recruitment at the time of recruitment and also post summaries of clinical trials at the time of approval. Companies have made the further commitment – voluntarily under the PhRMA Clinical Trial Principles – to post summaries of research conducted under discontinued research programs.

When conducting multinational, multi-site trials, in both the industrialized and developing world, PhRMA member companies follow standards based on the Guideline for Good Clinical Practice of the International Conference of Harmonization (GCP). The GCP is an international standard for designing, conducting, recording and reporting clinical research involving human participants. Compliance with the GCP assures that the rights, safety and well-being of human participants are protected and that clinical trial data are credible. The GCP was developed using best practices from many countries as well as the World Health Organization. They were published in 1996 as part of the International Conference of Harmonization and are intended to apply in the European Union, Japan and the United States.[ii] However, PhRMA encourages its members to apply the GCP to studies

conducted in all countries, including the developing world. Applying the GCP broadly helps assure that certain minimum ethical standards are consistently applied in countries that may not have rules or laws governing clinical trial conduct.[iii]

Clinical Trials and Sharing of Patient Data

Critics have recently called into question the integrity and effectiveness of the current clinical trial regime and regulatory process. Faulting companies, researchers, academics and government regulators alike, these critics call for the public release of results from all clinical trials and access to patient-level data for all trials conducted globally. The unregulated release and access to such data will severely threaten the integrity, validity and future of critical clinical trials testing new medicines with potential to solve some the world's most challenging health issues. There will be negative effects on recruitment, enrollment and participation in clinical studies; the clinical trial process will be slowed; and there will be confusion among patients and care givers.

Negative Effects on Recruitment, Enrollment and Participation

Without adequate security of data and patient information, the development process is compromised. Without protection of patient data security, there are fewer incentives for patients to volunteer for clinical trials. A recent Tufts Center for the Study of Drug Development report found that currently, 48% of clinical trial sites miss enrollment targets and study timelines often slip, causing extensions that are nearly double the original duration in order to meet enrollment levels for all therapeutic areas.[iv] Increased releases of data may compromise participant privacy, decreasing trial participation and slowing the development of new medicines.

To assure past, current and future patients, biopharmaceutical companies take great care to respect and protect the safety and individual privacy of research participants and have had a longstanding commitment to sponsoring clinical studies that fully comply with all legal and regulatory requirements. Release of patient-level data sets, as some critics have sought, could threaten patient privacy, even if data are "anonymized."

Delayed Clinical Trial Process

The current regulatory regime is designed and implemented to provide the highest quality oversight of the clinical trial process. Throughout the process there are opportunities for input and review by diverse stakeholders. In order to ensure that patients have access to safe medicines as quickly as possible, FDA approval should represent the final step. Release of all trial data threatens to create regulatory second-guessing potentially slowing the approval process and delaying introduction of life-saving medicines to market.

Documents of Clinical Trials Principles

- PhRMA's Principles on Conduct of Clinical Trials and Communication of Clinical Trial Results (2009)
- Joint Position on the Disclosure of Clinical Trial Information via Clinical Trial Registries and Databases(2009; IFPMA, EFPIA, PhRMA, JPMA)
- Joint Position on the Publication of Clinical Trial Results in the Scientific Literature (2010; IFPMA, EFPIA, PhRMA, JPMA)

Increased Confusion among Patients and Care-Givers

The current structured and regulated release of clinical trial outcomes and data is designed to provide the best information and guidance to patients and physicians alike. The biopharmaceutical sector is committed to providing relevant and context-specific information about potential new medicines so that patients and their clinicians can make informed decisions about participation in clinical trials and use of future new medicines.

The current clinical trial system prioritizes patient safety and privacy, and facilitates the approval of innovative new medicines. The process has significant oversight by government regulators, and the biopharmaceutical industry holds itself to strict principles governing the conduct of clinical trials, the communication of clinical trial results and the disclosure of clinical trial information through FDA submissions, presentations at medical convergences, publication in journals and participation in clincaltrials.gov.[v, vi]

Sources:

[i] Pharmaceutical Research and Manufacturers of America, Clinical Research Trials 2012 (Washington, DC: PhRMA, 2012), http://phrma.org/research/clinical-research-trials

[ii] International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use (Geneva, Switzerland: ICH, 1996), Harmonised Tripartite Guideline – Guideline for Good Clinical Practice E6(R1), http://www.ich.org/fileadmin/Public_Web_Site/ICH_Products/Guidelines/Efficacy/E6_R1/Step4/E6_R1__Guideline.pdf

[iii] Pharmaceutical Research and Manufacturers of America, Principles on Conduct of Clinical Trials and Communication of Clinical Trial Results (Washington, DC: PhRMA, 2009), http://phrma.org/sites/default/files/105/042009_clinical_trial_principles_final.pdf

[iv] Tufts Center for the Study of Drug Development, "89% of trials meet enrollment, but timelines slip, half of sites under-enroll," *Impact Report* 15, no. 1 (2010).

[v] International Federation of Pharmaceutical Manufacturers and Associations, Joint Position on the Disclosure of Clinical Trial Information via Clinical Trial Registries and Databases (Geneva, Switzerland: IFPMA, 2009), http://clinicaltrials.ifpma.org/clinicaltrials/fileadmin/files/pdfs/EN/November_10_2009_Updated_Joint_Position_on_the_Disclosure_of_Clinical_Trial_Information_via_Clinical_Trial_Registries_and_Databases.pdf

[vi] International Federation of Pharmaceutical Manufacturers and Associations, Joint Position on the Publication of Clinical Trial Results in the Scientific Literature (Geneva, Switzerland: IFPMA, 2010), http://clinicaltrials.ifpma.org/clinicaltrials/fileadmin/files/pdfs/20100610_Joint_Position_Publication_10Jun2010.pdf