Essential Tremor (ET)

The drug development & approval process

The United States (US) system of new drug approvals is perhaps the world's most rigorous. The mean cost of developing a new drug is estimated to range from $314 million to $2.8 billion, according to a 2020 study published in the Journal of the American Medical Association (JAMA).

According to the industry group PhRMA (Pharmaceutical Research and Manufacturers of America), it takes 10-15 years on average to develop one new medicine from initial discovery through regulatory approval. Their research shows only 12 percent of new compounds that enter clinical trials eventually receive U.S. Food and Drug Administration (FDA) approval.

Once a new compound has been identified in the laboratory, medicines are developed as follows:

Pre-clinical testing: A pharmaceutical company conducts laboratory and animal studies to show biological activity of the compound against the targeted disease, and the compound is evaluated for safety.

Investigational New Drug Application (IND): After completing pre-clinical testing, a company files an IND with the US Food and Drug Administration (FDA) to begin to test the drug in people. The IND becomes effective if FDA does not disapprove it within 30 days. The IND shows results of previous experiments; how, where and by whom the new studies will be conducted; the chemical structure of the compound; how it is thought to work in the body; any toxic effects found in the animal studies; and how the compound is manufactured. All clinical trials must be reviewed and approved by the Institutional Review Board (IRB) where the trials will be conducted. Progress reports on clinical trials must be submitted at least annually to FDA and the IRB.

Clinical Trials, Phase I: These tests involve about 20 to 80 normal, healthy volunteers. The tests study a drug's safety profile, including the safe dosage range. The studies also determine how a drug is absorbed, distributed, metabolized, and excreted as well as the duration of its action.

Clinical Trials, Phase II: In this phase, controlled trials of approximately 100 to 300 volunteer patients (people with the disease) assess a drug's effectiveness.

Clinical Trials, Phase III: This phase usually involves 1,000 to 3,000 patients in clinics and hospitals. Physicians monitor patients closely to confirm efficacy and identify adverse events.

New Drug Application (NDA): Following the completion of all three phases of clinical trials, a company analyzes all of the data and files an NDA with FDA if the data successfully demonstrates both safety and effectiveness. The NDA contains all of the scientific information that the company has gathered. NDAs typically run 100,000 pages or more. After an NDA is received, FDA has 60 days to decide whether to file it so it can be reviewed.

Approval: Once FDA approves an NDA, the medicine becomes available for physicians to prescribe. A company must continue to submit periodic reports to FDA, including cases of adverse reactions and appropriate quality-control records. For some medicines, FDA requires additional trials (Phase IV) to evaluate long-term effects.

International Essential Tremor Foundation

Your Voice for Essential Tremor

Our Mission:
The IETF provides hope to the essential tremor (ET) community worldwide through awareness, education, support and research.

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