

Clinical Trials Modernization Act

As part of the effort to improve the development of 21st Century Cures, Rep. Chris Collins is introducing legislation to streamline clinical trials. The Clinical Trials Modernization Act will free up innovators, allowing them to get life-saving drugs and medical devices more quickly to those in need. This legislation is a step forward to reducing regulatory overlap and administrative inefficiency, encouraging broader utilization of efficient, flexible trial designs, and modernizing the development and assessment of potential new treatments.

Bayesian Statistics and Adaptive Trial Provision

Problem: Clinical trials today require immense resources and large sample sizes to abide by FDA regulations and ensure safe products are available to all patients. Although recent scientific breakthroughs have allowed innovators to prevent, treat, and cure many diseases and conditions, the methods used to evaluate investigational drugs and devices have remained the same for decades. Within FDA, acceptance of emerging statistical models that take into account prior data are not adjudicated evenly, leading to uncertainty among the producing community regarding the acceptance of these new, more efficient methods of research. In order to bring innovative therapies to patients faster, drug and medical device producers must be able to rely on clear, streamlined guidance regarding new statistical approaches to clinical trials.

Solution: This provision requires the Secretary of HHS to draft guidance and establish uniform standards on the regulatory review and approval process of drugs, biological products, or devices that are tested through adaptive trial designs and Bayesian methods. Traditional statistical methods for clinical trials use information from previous studies at the design stage and use the information acquired from these studies at the conclusion of the study to complement the formal data analysis. Bayesian statistics and adaptive trials are approaches for learning from evidence as it accumulates and consider prior information and trial results as part of a continual data stream. Inferences are updated each time new data become available and the trial is able to adapt as new information is uncovered. While currently applied to such observational studies as predicting hurricane paths, advances in technology have made practical application of Bayesian statistics to biometric research feasible only in the last few decades. Uniform guidance from HHS on innovative clinical trial designs will allow for trials of smaller-sizes and, in some cases, shorter duration, which are more accurate in identifying how individuals of different backgrounds and genetic dispositions react to certain therapies.

Post-marketing Provision

Problem: After approval of a drug, FDA has the authority to require the sponsor to conduct certain types of post-marketing clinical trials or other studies. While some post-marketing studies are necessary, others lack justification and their purpose is vague and not subject to periodic reviews. The number of post-marketing clinical trial requirements has nearly doubled since the enactment of the Food and Drug Administration Amendments Act in 2007, while the yield of new, unprecedented drugs has remained steady. These post-marketing studies cost innovators more and more in expensive additional clinical trials and should be periodically reevaluated due to the changing circumstances within the medical field.

Solution: This provision requires the Secretary of Health and Human Services (HHS) to establish a process under which the Secretary or sponsors can request a periodic evaluation of a post-approval study or clinical trial. This evaluation will be used to determine whether the trial or study is still scientifically warranted and whether the design and timelines of the study or trial should be renegotiated. Ultimately, after evaluating new evidence, changes to medical practices and standards of care, and the approval of new drugs, the Secretary determines whether a post-approval study or clinical trial should continue as planned, be terminated, or whether the terms of the trial should be renegotiated. This reevaluation process will ensure redundant and unnecessary post-marketing studies are not forced to continue in the face of sound scientific evidence.