# 21ST CENTURY CURES ACT: NEW CLINICAL RESEARCH TOOLS



"Title III — Development" of the 21st Century Cures Act (the "Act") contains several significant provisions devoted to facilitating new pathways for both drug and medical device development. Patient-focused drug development, new drug development tools, new approaches to clinical trial design, and the use of real-world evidence for certain clinical purposes all represent a theme in the Act to allow for new and flexible approaches to research, testing, and approval of drugs in appropriate circumstances.

## PATIENT-FOCUSED DRUG DEVELOPMENT

Section 3002 of the Act establishes a framework to collect and use "patient experience data" and related information in support of applications submitted under Section 569C of the Federal Food, Drug, and Cosmetic Act (the "FDCA") or Section 351(a) of the Public Health Service Act (the "PHSA"). Patient experience data is intended to provide information about patient experiences with a health condition, including the impact of the health condition or related therapy on patients' lives, and patient preferences with respect to treatment of a health condition. Patient experience data may be collected from patients, family members and caregivers of patients, patient advocacy organizations, disease research foundations, researchers, and drug manufacturers.

The Secretary of Health and Human Services (the "Secretary") is required, pursuant to Section 3002 of the Act, to develop a plan to issue draft and final guidance documents, over a period of five years, regarding collection of patient experience data and the use of such data in drug development. A draft version of at least one such guidance document must be issued within 18 months after the enactment of the Act, and no later than 18 months following the end of the public comment period, either revised draft guidance or final guidance must be issued.

The guidance is intended to be used by any person seeking to collect patient experience data for submission to and proposed use by the Secretary in regulatory decision-making. The guidance documents must, among other requirements, address specific methodological approaches that are relevant and objective and ensure that data is accurate and representative of the intended population. The guidance documents must include methods to collect meaningful patient input throughout the drug development process and methodological considerations for data collection reporting, management, and analysis. Finally, at least 180 days following enactment of the Act, the Secretary is required to provide a brief public statement regarding the patient experience data and related information, if any, submitted and reviewed as part of an application.

## ADVANCING NEW DRUG THERAPIES

Section 3011 of the Act amends Chapter V of the FDCA to include a new Section 507 addressing a process of qualification for "drug development tools." A drug development tool is intended to be used for supporting or obtaining approval or licensure (as applicable) of a drug or biological product, or supporting the investigational use of a drug or biological product under section 505(i) of the FDCA or section 351(a)(3) of PHSA. The term "drug development tool" is defined to include a biomarker, a clinical outcome assessment, and any other method, material, or measure that the Secretary determines aids drug development and regulatory review. The term "biomarker" means "a characteristic that is objectively measured as an indicator of normal biologic processes, pathologic processes, or biological responses to a therapeutic intervention, and includes a surrogate endpoint." A surrogate endpoint is a marker such as a laboratory measurement, radiographic image, physical sign, or other measure that is known to predict or reasonably likely to predict clinical benefit and could be used to support traditional approval

or accelerated approval of a drug or biological product. A "clinical outcome assessment" means "a measurement of a patient's symptoms, overall mental state, or the effects of a disease or condition on how the patient functions and includes a patient-reported outcome." A patient-reported outcome is reported by the patient regarding a health condition without interpretation by a clinician or other person.

The Act directs that drug development tools be approved by the Secretary through a defined process of qualification whereby the drug development tool is deemed a "qualified" drug development tool. A drug development tool is "qualified" if in its proposed context of use the tool can be relied upon to have a specific interpretation and application in drug development and review under the Act. The qualification process will consist of several steps, beginning with submission of a letter of intent by a requestor to the Secretary for review. Upon acceptance of a letter of intent by the Secretary, the requestor would then submit a qualification plan to the Secretary for review, and upon acceptance, a full qualification package would be submitted. The Secretary is required to develop draft guidance on the implementation of this section not later than three years following enactment of the Act, and final guidance must be developed not later than six months following closing of the comment period for the draft guidance.

## NOVEL CLINICAL TRIAL DESIGNS

Section 3021 of the Act requires the Secretary to conduct a public meeting and issue guidance that addresses the use of complex adaptive and other novel trial designs in the development and regulatory review and approval or licensure for drugs and biological products. The guidance must specifically include how such proposed clinical trials help satisfy the substantial evidence standard under section 505(d) of the FDCA. Additionally, the guidance must address