Progressing Toward a Cure: House Committee Unveils Revised, Streamlined 21st Century Cures Discussion Draft

On April 29, 2015, the House Energy & Commerce Committee ("E&C Committee") released a revised discussion draft of the medical innovation reform legislation that has been in the works for over a year as part of the 21st Century Cures Initiative. As Ropes & Gray previously reported, the E&C Committee released its initial discussion draft, which included a variety of legislative proposals organized in five titles spanning nearly 400 pages, at the end of January 2015.

The revised discussion draft continues to target the "the complete cycle of cures," organized in three broad titles—discovery, development, and delivery. Nonetheless, the revised discussion draft has been significantly pared down. A number of proposals, including those relating to drug exclusivity and laboratory developed tests, have been removed. The revised draft also includes several new proposals, including a provision proposing several modifications to Section 114 of the Food and Drug Administration Modernization Act (FDAMA). These revisions and the pared down nature of the draft likely are the product of negotiations among E&C Committee members. Significantly, unlike the original discussion draft noting no sponsors, the revised version lists five bipartisan sponsors, including the Chairs and Ranking Members of the full E&C Committee and the Health Subcommittee.

On April 30, 2015, the E&C Committee held a legislative hearing regarding the revised discussion draft at which representatives from FDA's Center for Drug Evaluation and Research and Center for Devices and Radiological Health, as well as the National Institutes of Health (NIH) testified. Committee mark-ups of the discussion draft are expected to occur soon, and E&C Committee chairman Fred Upton and others spearheading the 21st Century Cures Initiative have expressed their intent to enact legislation this year.

The key provisions that have been omitted from, added to, or substantially changed in the revised discussion draft are noted below.

What Has Been Cut?

A number of legislative proposals contained in the initial discussion draft have been omitted from the revised version. Notable omissions include proposals that would have:

- Created or modified drug exclusivity programs, including a new exclusivity program for "dormant therapies," extended exclusivity for significant improvements and reformulations to existing drugs, extended exclusivity for American-manufactured generic drugs and biosimilars, extended exclusivity for orphan drugs, and transferable exclusivity for qualified infectious disease products
- Clarified FDA regulation of social media
- Modernized FDA regulation of diagnostics and laboratory-developed tests
- Enhanced FDA's ability to approve breakthrough therapies based on early stage clinical data
- Created an accelerated approval pathway and establishment of CMS coverage for breakthrough devices
- Addressed supply chain security for medical devices
- Updated current good manufacturing practice regulations and guidance accounting for modern drug manufacturing technologies
- Created a program for re-evaluation of required post-approval studies and clinical trials
- Established a 21st-century data-sharing framework
- Clarified the regulation of drug-device combination products
- Established a list of devices for which manufacturers have opted out of Medicare secondary payer payment coverage

What Is New?

The revised discussion draft also includes several proposals that were not offered in the earlier version. Highlights include proposals relating to:

- *Communication of Health Care Economic Information by Drug Manufacturers*: The revised draft proposes several modifications to FDAMA 114, the statutory provision that permits drug manufacturers to convey health care economic information (HCEI) to formulary committees and other similar entities so long as the information is directly related to an approved indication and is based on competent and reliable scientific evidence. In particular, the draft (1) clarifies that payers are included within the audience permitted to receive health care economic information; (2) permits sharing HCEI that is "related to" the approved indication, as opposed to "directly related"; and (3) indicates that all components of the economic analysis, including the data, inputs, clinical or other assumptions, methods, results, and other components comprising the analysis, are included with the definition of HCEI (and thereby subject to the competent and reliable scientific evidence standard).
- *Guidance on CLIA Waiver Studies*: A provision calling for FDA to revise its 2008 "Recommendations for Clinical Laboratory Improvement Amendments of 1988 (CLIA) Waiver Applications for Manufacturers of In Vitro Diagnostic Devices" guidance document to include information concerning the appropriate use of comparable performance to demonstrate device accuracy.
- *Accelerated Approval Development Plans*: A provision allowing for the submission of accelerated approval development plans containing study parameters for the use of a surrogate endpoint intended to serve as the basis for the accelerated approval of drugs beyond the current scope of drugs intended to treat patients with life-threatening illnesses and unmet needs.
- *NIH Funding*: The revised draft includes a new provision authorizing nearly $100 billion to be appropriated for NIH for fiscal years 2016 through 2018. Additionally, the draft establishes the NIH Innovation Fund, which would appropriate $2 billion for fiscal years 2016 through 2020 for the purpose of funding "Precision Medicine" and "Young Emerging Scientists" initiatives denoted in the previous draft with placeholders.
- *IT System for Data on the Natural History of Diseases*: A provision has been added establishing an FDA public-private partnership to create a publicly available, secure information technology system with a focus on the natural history of rare diseases.
- *Repurposing Drugs*: A placeholder for "repurposing drugs for serious and life-threatening diseases and conditions" has been added to the draft. If comments submitted by patient advocacy groups are incorporated into the language of this provision, incentives such as exclusive marketing rights could be included to encourage drug manufacturers to pursue therapies for conditions for which there currently are no treatments.

What Else Has Changed?

- *Precision Medicine*: A placeholder has been replaced with a detailed provision directing FDA to publish guidance concerning the definition of precision drugs and methods for identifying subpopulations for biological characteristics research. Data obtained from research targeting such subpopulations may, in turn, be used by drug companies to seek orphan drug exclusivity.
- *Surrogate Endpoints and Biomarkers*: Provisions related to surrogate endpoint qualification and utilization have been streamlined and modified to address the qualification of biomarkers, including surrogate endpoints that may be used for accelerated product approval. The streamlined provision also calls on FDA to publish guidance documents concerning the biomarker qualification.

- *Clinical Experience Evidence*: A provision calling for FDA to incorporate real-world evidence into its review of drugs for new indications and post-approval study requirements has been revised to focus on evidence based on "clinical experience" and to provide FDA with detailed instructions for establishing a framework for the program.
- *Expanded Access Programs*: A provision calling for drug manufacturers to disclose details on their expanded access programs for certain limited categories of drugs has been modified to require manufacturers to disclose their expanded access policies for any investigational new drug used in a phase 2 or phase 3 "human safety study."
- *Health Software*: Provisions addressing FDA oversight of "medical" and "health" software have been modified to eliminate the term "medical software" altogether. "Health software" such as administrative, mobile fitness, and other software not intended to serve patient-monitoring purposes and not an "integral part of a device" would remain exempt from FDA regulation.
- *Valid Scientific Evidence for Devices*: A provision clarifying that well-documented evidence from clinical registries and published studies can constitute "valid scientific evidence" for purposes of FDA's effectiveness evaluation has been modified to permit FDA to request in certain circumstances the underlying data from a study published in a peer-reviewed journal to confirm its validity. The prior provision would have presumed the validity of data from studies published in peer-reviewed journals.
- *Premarket Review of Class I Devices*: A provision that would have streamlined the process for premarket review of Class I devices (to the extent such devices are not already 510(k)-exempt) has been replaced with a placeholder.
- *Disposable Medical Technologies*: The details of a provision addressing Medicare coverage for disposable medical technologies have been replaced with placeholder text.

Prospects for the Legislation

The prospects of passage for 21st Century Cures legislation remain uncertain. A key issue discussed at the E&C Committee's April 30 hearing was the need for additional FDA funding to support and implement the legislative proposals in the revised discussion draft. FDA officials expressed concern about the potential for unfunded mandates, and some House Republicans have previously indicated that they would be unwilling to support funding increases for FDA.

In addition to the House's efforts with 21st Century Cures, the Senate is separately considering the need for medical innovation legislation. On April 28, 2015, the Senate Health, Education, Labor & Pensions ("HELP") Committee held its own hearing on medical innovation with participation from FDA and NIH officials. Unlike the House E&C Committee, the Senate HELP Committee has not released any legislative proposals, so it remains to be seen how similar or different the Senate and House bills will be, provided the Senate's recent hearings on Precision Medicine and Medical Innovation ultimately result in a bill.

Any legislative proposals that are considered too politically controversial or too technically complex for medical innovation legislation this year could be reassessed as part of the negotiations for the next reauthorization of the drug and device user fee laws (PDUFA and MDUFA, respectively). Those negotiations for the sixth version of PDUFA are slated to begin in June. Acting FDA Commissioner Stephen Ostroff, in public statements made in April, acknowledged that FDA is already advising members of the E&C Committee as to which proposals are appropriate for the 21st Century Cures legislation and which should be deferred for discussion as part of PDUFA-VI negotiations.

Ropes & Gray will continue to monitor legislative developments in this area. If you have any questions, please contact any member of Ropes & Gray's FDA regulatory practice or your usual Ropes & Gray advisor.