

The Cure for the Common FDA Reform Legislation? House Committee Releases 21st Century Cures Discussion Draft

On January 27, 2015, the House Energy & Commerce Committee (“E&C Committee”) released a long-awaited [discussion draft](#) of comprehensive medical innovation reform legislation as part of its 21st Century Cures Initiative. Launched in April 2014 by E&C Committee Chairman Fred Upton (R-MI) and Rep. Diana DeGette (D-CO), the 21st Century Cures Initiative has sought ideas for legislative proposals to accelerate the pace of medical innovation in the United States through a series of committee hearings, roundtables held around the country, and white papers. According to supporting materials released by the E&C Committee, the key goals of the proposals in the discussion draft are to (1) incorporate patient perspectives into the development and approval processes for drugs and medical devices and help address patients’ unmet medical needs; (2) build the foundation for 21st century medicine; (3) streamline clinical trials; (4) support continued innovation at our federal public health agencies; and (5) modernize medical product regulation.

Two days after the release of the E&C Committee’s discussion draft, Senators Lamar Alexander (R-TN) and Richard Burr (R-NC) of the Senate Committee on Health, Education, Labor & Pensions released a [report](#) focused on identifying opportunities for meaningful legislative reforms to medical product discovery and development. The Senate report is intended as a parallel and complementary effort to the draft legislation under the 21st Century Cures Initiative, and it likewise focuses on the need for increased incentives for innovation and greater regulatory efficiency in the drug and device industry.

Both the Senate report and the E&C Committee’s discussion draft are early proposals intended to generate further debate, yet the authors of both suggest that they intend to introduce comprehensive legislation that they hope will be passed later this year. Given the far-ranging issues that would need to be considered and debated, the goal of passing such legislation this year is extremely ambitious. If comprehensive legislation does not pass this year, it is possible that ideas generated through these efforts may be further considered as the reauthorization process for drug and device user fee laws (“PDUFA/MDUFA”) gets underway.

Key Legislative Proposals to Watch for Drug and Device Manufacturers

The 21st Century Cures Initiative discussion draft contains wide-ranging legislative proposals that, if enacted, would significantly alter Food and Drug Administration (“FDA”) oversight of drugs and medical devices.

For both drug and device manufacturers, notable highlights include proposals to

- **Modernize Clinical Research Regulation** by clarifying the roles of central and local institutional review boards (“IRBs”), minimizing overlap in the regulatory requirements applicable to human subject research, and establishing a framework for companies to incorporate adaptive trial designs and Bayesian and other statistical methods into clinical trial designs in an effort to improve the efficiency of clinical trials. This provision would also empower the Secretary of Health and Human Services (“HHS”) to make any necessary changes to the HHS Human Subject Regulations or vulnerable-populations rules to avoid conflict with or duplication of the new law and its accompanying regulations.
- **Permit Re-evaluation of Post-approval Study Requirements** by creating a framework for FDA and sponsors to evaluate whether post-approval studies remain scientifically warranted, and to discontinue the studies or revise their scope where appropriate.
- **Redefine Rules for Communication of Scientific and Medical Developments by Manufacturers Regarding Their Products**, including so-called “off label” information. As Ropes & Gray has previously reported [here](#) and [here](#), FDA is re-evaluating its regulations and policies in

response to citizen petitions filed by the Medical Information Working Group, a coalition of drug and device manufacturers to which Ropes & Gray serves as outside counsel. The draft contains a “placeholder” for this proposal, which, although still being developed by the E&C Committee, is certain to be one of the most closely watched provisions in the bill.

- **Clarify FDA Regulation of Social Media** by, among other things, acknowledging that sponsors may present summary information concerning the safety and effectiveness of drugs and medical devices in character-limited applications like Twitter via hyperlinks. FDA has previously issued numerous draft guidance documents related to the regulation of social media promotion by drug and device manufacturers, as Ropes & Gray has previously reported [here](#) and [here](#).
- **Require FDA to Issue Guidance on Drug-Device Combination Products**, particularly with respect to the premarket review process for such products.
- **Launch the Precision Medicine Initiative.** The draft contains a placeholder that may serve as a vehicle to discuss the initiative recently announced by the President, which among other things would increase efforts to identify genomic drivers in cancer and direct FDA to develop a new approach for evaluating tests that rapidly sequence large segments of a person’s DNA.

For drug manufacturers specifically, notable highlights include proposals to

- **Incentivize Development of New Therapies, Dormant Therapies, New Antibiotics, and U.S.-Manufactured Generic Drugs** by extending exclusivity for two years for significant improvements to existing molecules; giving developers of “dormant therapies” (i.e., products that address an unmet medical need but have insufficient patent protection to encourage development) an option to choose a 15-year period of exclusivity as an alternative to the exclusivity available under current law; creating a new transferable exclusivity program for antibiotics; and extending exclusivity for generic and biosimilar drugs manufactured in the United States.
- **Facilitate Surrogate Endpoint Qualification and Utilization** by establishing a transparent process for qualifying surrogate endpoints, which can expedite drug approval by allowing drug effectiveness to be demonstrated by showing effect on a biomarker rather than clinical morbidity or mortality.
- **Expedite Approval of Breakthrough Therapies** by clarifying FDA’s authority to approve such drugs based on early-stage clinical data.
- **Streamline the Review of Supplemental New Drug Applications for New Indications** by permitting FDA to accept and review data summaries rather than full data packages.
- **Incorporate Real-World Evidence** by requiring FDA to issue guidance regarding the ability of drug manufacturers to rely on real-world use evidence to support approval of new indications and to satisfy post-approval study requirements.
- **Increase Transparency Regarding Expanded Access Programs for Unapproved Drugs** by requiring drug companies to disclose details on their expanded access programs.
- **Incorporate Patient Perspectives in Drug Approval Decisions** by requiring FDA to consider feedback from patients concerning desired benefits and tolerable risks associated with new treatments.
- **Foster 21st Century Manufacturing** by requiring FDA to issue regulations and guidance that take into account modern drug manufacturing technologies.
- **Streamline Regulatory Processes for New Vaccines** by establishing timelines for review of vaccines by the Centers for Disease Control (“CDC”) and creating a process for expedited FDA review of breakthrough therapies and therapies needed during public health emergencies.

For device manufacturers specifically, notable highlights include proposals to

- ***Incentivize the Development of Breakthrough Devices*** by establishing processes for priority review and accelerated approval of devices integrating breakthrough technologies that have the potential to address unmet medical needs, as well as transitional Medicare and Medicaid coverage of such breakthrough devices.
- ***Accelerate Innovation and Patient Access*** by enabling patients to obtain certain medical device treatments earlier than the treatments would typically be available by establishing a list of devices for which manufacturers have opted out of Medicare secondary payer payment coverage.
- ***Modernize the Regulation of Diagnostics***, especially in light of FDA’s 2014 proposal for the regulation of laboratory-developed tests, which was previously reported by Ropes & Gray [here](#). The discussion draft currently contains a placeholder for this proposal.
- ***Reform FDA Oversight of Medical Apps and Health IT*** by, among other things, distinguishing between “medical software” subject to FDA regulation and “health software” not subject to FDA regulation. FDA has previously issued numerous guidance documents related to the regulation of medical apps and health IT, as Ropes & Gray has previously reported [here](#).
- ***Secure the Medical Device Supply Chain*** by establishing a national framework for licensure of medical device wholesalers and third-party logistics providers.
- ***Clarify FDA Review Standards for Evaluating Effectiveness of Devices*** by recognizing that well-documented, real-world evidence from clinical registries and published studies can qualify as “valid scientific evidence.”
- ***Streamline the Process for Pre-Market Review of Class I Devices*** by permitting manufacturers to submit a determination by an accredited third party of current good manufacturing practices compliance instead of a traditional 510(k) (to the extent a particular Class I device is not 510(k)-exempt altogether).
- ***Provide Coverage for Disposable Medical Technologies*** by reforming the Medicare coverage and payment framework for certain disposable medical equipment.

Other highlights of interest to the drug and device industry include proposals to

- ***Establish a 21st Century Cures Consortium***, a public-private partnership that would bring together leaders from FDA, the National Institutes of Health, and the Centers for Medicare & Medicaid Services (“CMS”) with leaders from the drug and device industry, academic research institutions, patient groups, health plans, and other stakeholders to accelerate the discovery, development, and delivery of innovative cures through the award of contracts and grants and other means.
- ***Establish a Medicare Pharmaceutical and Technology Ombudsman within CMS*** to field complaints and requests from pharmaceutical and medical device companies.
- ***Create a 21st Century Data Sharing Framework*** to enable, among other things, patients and physicians to better identify ongoing clinical trials and researchers and developers to use Medicare data to improve the quality of patient care.

Next Steps in the Legislative Process

The E&C Committee has stated that the discussion draft is “neither perfect nor complete.” Several proposals are currently designated with placeholders, and the legislation has not yet been formally introduced in the House. The Senate’s efforts are even more preliminary.

Thus, despite the aspirations of both the E&C Committee's discussion draft and the report from Senators Alexander and Burr, it remains to be seen what reforms Congress will be able to enact in the near term. Since 1992, when Congress enacted the original Prescription Drug User Fee Act, most FDA reform legislation has been incorporated into user fee reauthorization legislation enacted every five years (*e.g.*, the FDA Modernization Act in 1997, the FDA Amendments Act in 2007, and the FDA Safety and Innovation Act in 2012). Given that the next user fee reauthorization legislation is not due to be enacted until 2017, any reform legislation enacted this year would depart from the typical five-year cycle.

As debate over the 21st Century Cures Initiative and complementary endeavors in the Senate continues in the coming weeks and months, legislative proposals that prove more controversial may be eliminated from the legislation to be enacted this year and may be deferred for consideration by FDA and industry as part of their statutorily mandated discussions over the next user fee reauthorization legislation slated for 2017.

Another key issue that will significantly affect the prospects of Congress enacting comprehensive reform legislation this year is funding. As initial ideas under the 21st Century Cures Initiative were discussed by the House last year, some House Republicans indicated that they would be unwilling to support increases in FDA funding.

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.