ROPES & GRAY ALERT

FDA Regulatory ▪ Health Care ▪ Digital Health

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21st Century Cures Act – Provisions Relating to Development Incentives for Certain Classes of Drugs

On December 13, 2016, President Obama signed into law the 21st Century Cures Act (the Act), just days after it passed in the U.S. House of Representatives and Senate. With an overarching goal of advancing biomedical innovation, the Act makes numerous changes to laws that govern Food and Drug Administration (FDA) programs, clinical research regulations, and Medicare coverage and reimbursement rules.

To see Ropes & Gray's analysis of key provisions of the Act, please click on the hyperlinks below:

- Promoting Drug Development
- Medical Device Innovation
- Digital Health
- Regulation of Clinical Research
- Reimbursement & Fraud and Abuse

Partners in Ropes & Gray's FDA Regulatory practice have also recorded a podcast to discuss some key implications of the Act for biopharmaceutical and medical device manufacturers. Click here to listen to the podcast.

This Alert highlights select provisions aimed at incentivizing the development of certain types of drugs through statutory and programmatic mechanisms such as priority review vouchers, accelerated approval, and flexible study and data requirements. Incentives are provided under the Act for genetically targeted and variant protein-targeted drugs intended to treat rare diseases, drugs intended to treat rare pediatric diseases and orphan diseases, regenerative therapies (e.g., stem cell therapies), antimicrobial products intended to treat serious infections in a limited population, and medical countermeasures.

Targeted Drugs for Rare Diseases [Section 3012].[1]

The Act allows for more streamlined development of genetically targeted drugs and variant protein-targeted drugs that address unmet medical need in rare disease subgroups. Under this provision, sponsors of targeted drugs may rely on data from studies previously conducted by the same sponsor (or another sponsor with a contractual right of reference) and submitted in previously approved applications for drugs using the same or similar targeted technology. By explicitly authorizing FDA to approve genetically targeted drugs and variant protein-targeted drugs under these circumstances, this provision is expected to accelerate the development process for these products, though, importantly it does not alter the existing NDA approval standard.

Reauthorization to Encourage Treatments for Rare Pediatric Diseases [Section 3013].

The Act reauthorizes the pediatric rare disease priority review voucher program through September 30, 2020. This provision allows drugs designated by September 30, 2020 to continue to receive a voucher under this program if approved by September 30, 2022.

[1] Sections cited in this Alert refer to the relevant provisions in the Act.

Amendments to the Orphan Drug Grants [Section 3015].

Under the Federal Food, Drug, and Cosmetic Act (FDCA), the Secretary of Health and Human Services (HHS) is authorized to make grants and enter into contracts with public and private entities to help defray the cost of developing orphan drugs. This provision would expand the types of tests eligible for governmental financial assistance by expanding the scope of "qualified testing" conducted to develop orphan drugs. Specifically, the provision would broaden "qualified tests" to include prospectively planned and designed observational studies conducted to assist in the understanding of the natural history of a rare disease and in the development of the therapy.

Accelerated Approval for Regenerative Advanced Therapies [Section 3033].

The Act includes a number of provisions to speed approval of new classes of products and therapies through the expedited approval framework under Section 506 of the FDCA. This provision creates an accelerated approval designation for regenerative advanced therapies, including cell therapies, therapeutic tissue engineering products, human cell and tissue products, and combination products using any of these therapies or products demonstrated by preliminary clinical evidence to have the potential to address unmet needs. In reviewing and approving these therapies, FDA may consider granting accelerated approval based on surrogate or intermediate endpoints with the possibility of FDA requiring confirmatory or post-market studies.

Antimicrobial Products: Limited Population Pathway [Section 3042].

The Act introduces a new "Limited Population Pathway" to expedite approval of antimicrobial products intended to treat a limited patient population. The provision allows FDA to approve antibacterial or antifungal drugs that treat serious or life-threatening infections in a limited population of patients with unmet needs, even if the benefit-risk profile may not justify approval for a broader patient population. Drugs approved under this provision would be required to adhere to special labeling requirements, including a prominent "Limited Population" statement. Additionally, in recognition of increasing concerns about drug-resistant infections, the Act requires the U.S. Government Accountability Office (GAO) to compile a report on antimicrobial resistance by 2021, which would include a review of any effect of the new Limited Population Pathway on antibacterial or antifungal resistance.

Encouraging Treatments for Agents that Present a National Security Threat [Section 3086].

The Act creates a new priority review voucher program for "material threat medical countermeasures." The Act defines the term to be drugs or vaccines intended to treat biological, chemical, radiological, or nuclear agents that present a national security threat, or to treat harm from a condition that may be caused by administering a drug or biological product against such an agent. As is the case under existing priority review voucher programs for tropical diseases and rare pediatric diseases, the voucher is transferable. The program is scheduled to sunset on October 1, 2023.

If you have any questions, please contact any member of Ropes & Gray's FDA regulatory or health care practices or your usual Ropes & Gray advisor.