Thanks to 21st Century Cures, the Sun is Coming out Tomorrow for Orphan Drugs and Pediatric Priority Review Vouchers

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The 21st Century Cures Act, a bill that includes major Food and Drug Administration reform, was passed by the Senate last week and was signed by President Obama on Tuesday, December 13, 2016.[1] While the new law addresses a wide variety of areas related to FDA regulation, it also includes an amendment to the Orphan Drug Act and reauthorization of the priority review voucher program for rare pediatric diseases.

The Orphan Drug Act of 1983 provided numerous incentives, including grants to defray clinical testing costs and seven years of non-patent marketing exclusivity, for sponsors to develop drugs to treat conditions that affect fewer than 200,000 people in the United States.[2] 21st Century Cures will expand access to orphan drug grants to include "observational studies" to help study a rare disease, in addition to studies that aid in the development of rare disease therapy.[3] No statutory definition of "observational studies" is given, beyond that they are studies to "understand the full spectrum of the disease manifestations, including describing genotypic and phenotypic variability and identifying and defining distinct subpopulations affected by a rare disease or condition."[4] Because grant funding will now be available for earlier stage research, this might lead to increased research focus on lesser understood rare diseases and, perhaps, the development of orphan therapies in the future.

It is noteworthy that the OPEN Act (Orphan Product Extensions Now Accelerating Cures and Treatments Act) did not make it into the final version of 21st Century Cures. The OPEN Act would have provided six-month extensions to orphan drug marketing exclusivities and would have prevented FDA from revoking orphan designation, absent an untrue statement in the application.[5]

Separately, in the rare disease space, the pediatric priority review program was created under the Food and Drug Administration Safety and Innovation Act (FDASIA) in 2012.[6] Under the program, a sponsor of a drug that receives approval to treat a rare pediatric condition is given a priority review voucher that can be used on a subsequent application for a different product. A manufacturer could use the voucher itself to have a future application reviewed in six months, instead of the standard ten months, or it could sell the voucher. Unlike other types of vouchers, pediatric priority review vouchers can be bought and sold an unlimited amount of times.[7]

The program was set to expire one year after the third voucher was granted, but President Obama signed the Advancing Hope Act in September, extending the program through the end of 2016.[8] Now, through 21st Century Cures, the program is extended through September 30, 2020.[9] An extension is also available for a drug that is designated as a drug for a rare pediatric disease no later than September 30, 2020 and is approved no later than September 30, 2022.[10]

1 http://docs.house.gov/billsthisweek/20161128/CPRT-114-HPRT-RU00-SAHR34.pdf
2 21 U.S.C. § 360ee
3 H.R. 34, 114th Cong. § 3015 (2016)
4 *Id.*
5 H.R. 971
6 21 U.S.C. § 360ff
7 21 U.S.C. § 360ff(b)(2)(A)
8 Public Law No: 114-229
9 H.R. 34, 114th Cong. § 3013 (2016)
10 *Id.*

21st Century Cures will require significant action from the FDA to implement and will certainly take some time. We will continue to monitor both the implementation of the Act and any guidance from FDA.

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