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Preventing Patient Harm in 21st Century Cures

Congress should not rush to pass this nearly 1,000 page bill before there is time to thoroughly review it and understand the public health consequences. While some harmful provisions have been improved or removed, there are still many provisions in the renegotiated bill that remain problematic for public health. Moreover, the bill fails to deliver on its original promise of mandatory NIH funding, as appropriators will have discretion whether to release the promised funding each year, starting the bargaining process over again and potentially forcing further public health concessions.

Some of the worst remaining provisions in the 21st Century Cures Act (as amended on November 29, 2016) include:

SEC. 3022. Real World Evidence (pages 165-169): Requires the FDA to develop a program to evaluate the potential use of "real world evidence" to support the approval of a new indication of an FDA-approved drug and to support or satisfy postapproval study requirements. “Real world evidence” means data regarding the usage, or the potential benefits or risks, of a drug derived from sources other than randomized clinical trials. The sources of real world evidence include ongoing safety surveillance, observational studies, registries, claims, and patient-centered outcomes research activities. This will weaken the standards for FDA review and approval of supplemental new drug applications (sNDAs) and lower the bar for the type of evidence needed to meet post-approval requirements.

SEC. 3031. Summary Level Review (pages 174-177): Allows the FDA to rely upon “qualified data summaries” to support approval of a supplemental application for an FDA-approved drug for a “qualified indication,” if there is existing data demonstrating the safety of the drug. The term “qualified indication” means an indication for a drug that the Secretary determines to be appropriate for summary level review; and the term “qualified data summary” means a summary of clinical data that demonstrates the safety and effectiveness of a drug for a qualified indication. This will weaken the standards for FDA review and approval of sNDAs.

SEC. 3033. Accelerated Approval for Regenerative Advanced Therapies (page 179 *et seq*): Creates an expedited review pathway for “regenerative medicine” products that allows use of surrogate endpoints and post-approval studies. This was not in previous versions of Cures and should not be rushed through without adequate discussion.

SEC. 3037. Health Care Economic Information (page 188 *et seq*): Allows pharmaceutical companies to promote off-label uses to insurance providers, allowing them to dramatically expand their markets while evading the requirement of FDA approval for new indications.

SEC. 3038. Combination Product Innovation (page 190-204): This language will still result in pressure on the FDA to regulate combination drug/device products as devices by allowing the manufacturer to challenge FDA’s initial classification and demand documented scientific proof that the “primary mode of action” of the product is that of a drug in cases where the product has been classified as a drug. The primary mode of action is often unclear because the product is complex, not sufficiently studied, or otherwise difficult to discern. Placing the burden on the FDA to provide a scientific rationale will result in more products being classified as devices in such cases of uncertainty.

SEC. 3042. Limited Population Pathway (pages 208-213): While this bill represents an improvement over prior versions of the PATH act, it still pressures the FDA to approve antibiotics based on smaller trials in a limited group of patients, meaning more safety risks will go undetected. Also, actual use in the real world of antibiotics approved under this pathway will likely be much more extensive, meaning such antibiotics will likely be used inappropriately in contexts where the benefits do not outweigh the risks.

SEC. 3051. Breakthrough Devices (pages 233-242): Creates overly-broad category of “breakthrough” devices and pressures the FDA to rush approval of these devices, potentially leading to poor decisions.

Additional bad provisions include:

SEC. 3013 & 3086. Reauthorization and Expansion of Priority Review Voucher Programs (pages 155 *et seq* and 296 *et seq*): These two sections renew the priority review voucher program for pediatric drugs and expand it to treatments for agents that present national security threats. This program contained large loopholes that allowed drug companies to extract windfall profits from vouchers awarded without requiring any new research. These should be fixed before the program is renewed.

SEC. 3053. Recognition of Standards (page 243 *et seq*): Compels the FDA to review and adopt or reject international standards, which may be drafted by industry, within an extremely brief review timeframe of 60 days. Once adopted, these standards will be used to regulate cleared medical devices in the United States.

SEC. 3058. Least Burdensome Device Review (page 252 *et seq*): Requires FDA employees to be drilled in a standard requiring them to ask for the minimum possible amount of information when approving new medical devices, inappropriately focusing attention away from other important standards, such as the standard for device safety and effectiveness.

SEC. 3060. Clarifying Medical Software Regulation (page 257 *et seq*): Exempts certain types of medical software from FDA oversight without providing for regulation by another federal agency, meaning problems with medical software will be difficult for regulators to address when they arise.

SEC. 4011. Medicare Pharmaceutical and Technology Ombudsman (pages 409-410)**:** Requires the Centers for Medicare and Medicaid Services to set up an ombudsman office, the sole purpose of which will be to respond to complaints, grievances and requests from pharmaceutical, biologic, medical device and diagnostic manufacturers about the coverage, coding and payment decisions made regarding the products of those industries. This would allow for greater industry influence over decisions impacting beneficiaries' health.