

# Summary of Provisions in 21<sup>st</sup> Century Cures Act (H.R. 6)

as passed by full House of Representatives, July 10, 2015

## Pediatric-Specific Provisions

- Requires the NIH to complete a strategic plan, and in the plan requires NIH to “ensure that rare and pediatric diseases and conditions remain a priority.” (Sec. 1021)
- Increases the maximum yearly support from the NIH pediatric loan repayment program (and other loan repayment programs) from \$35,000 to \$50,000, indexes it for inflation. (Sec. 1041)
- Requires the NIH to implement the National Pediatric Research Network Act (NPRN). (Sec. 1081)
  
- Establishes the “sense of Congress” that NIH and FDA should support the development of a global pediatric clinical trials network. (Sec. 1082)
  
- Requires the NIH to report every two years on the number of children, disaggregated by the pediatric age group, race, and gender, included in NIH-funded trials. Also requires an NIH workshop and the publication of NIH guidance on inclusion criteria for children. (Sec. 1083)
  
- Reauthorizes the rare pediatric disease priority review voucher program through December 31, 2018, and requires a GAO report to evaluate the effectiveness of the program for incentivizing drug development for rare pediatric diseases. (Sec. 2152)

AAP supports this provision that would require that children remain a priority in NIH strategic planning process.

Existing caps on NIH loan repayment programs are an obstacle to incentivizing the generation of physician researchers. AAP supports this provision.

Congress reauthorized the NPRN in 2013 but has yet to fund it. H.R. 6 would require NIH to implement the NPRN even if it is not funded. AAP believes that dedicated funding should be provided to the NPRN if NIH will be required to implement it so that it does not supplant other existing pediatric research networks.

This provision indicates that Congress is supportive of collaborative efforts to improve global infrastructure for industry-sponsored pediatric drug research. AAP supports the establishment of this network, but believes the network should be privately funded.

This provision is identical to the AAP-championed *Children Count Act* and AAP strongly supports it. Children have been required to be included in NIH studies since 1997 but NIH has never appropriately monitored the implementation of this provision by collecting and reporting on the actual numbers of children enrolled in NIH studies.

While there is a potential for the program to ultimately prove to be a helpful incentive for the development of drugs for children, it is not yet clear whether it will be able to incentivize the development of pediatric drugs that would not have otherwise been developed.

Public policy should ensure that any incentive is appropriately targeting the creation of truly new drugs and not rewarding those already in the pipeline. Any extension of this program must have accompanying requirements that demonstrate it is working appropriately. AAP is concerned that FDA is not adequately resourced to implement the program and modifications to it may be necessary to demonstrate that the added strain on FDA resources is justified.

The language does modify the program to ensure its applicability to important therapeutic areas including pediatric cancer and sickle cell disease.

## National Institutes of Health Provisions

- Reauthorizes the NIH and increases authorization levels to \$31.8 billion in 2016, \$33.3 billion in 2017, and \$34.9 billion in 2018. (Sec. 1001)
- Creates a separate NIH innovation fund to provide \$8.75 billion in mandatory funding to NIH over 5 years. (Sec. 1002)
- Directs the NIH to compile research and implement recommendations on how to streamline the grant process for researchers and reduce administrative burdens. (Sec. 1023)
- Establishes the “sense of Congress” that attendance at scientific conferences and meetings is important to the mission of NIH. (Sec. 1025)
- Eases restriction on participating in phase IIB and phase III studies at the NIH National Center for Advancing Translational Science (NCATS). (Sec. 1027)
- Requires NIH institutes and centers to set up programs and to set aside a percentage of funding for high-risk, high-reward research. (Sec. 1028)
- Establishes a public-private partnership called the Council for 21<sup>st</sup> Century Cures to accelerate the discovery, development, and delivery of innovative cures, treatments, and preventive measures for patients. Authorized at \$10 million per year for 8 years. (Sec. 1141)
- Requires NIH to finalize guidance on single IRBs for multi-site studies within 1 year. (Sec. 2261)

AAP strongly supports increased funding levels for NIH, however, they should be accompanied by language guaranteeing that it will not be supplanted by the NIH Innovation Fund.

AAP strongly support increased mandatory funding for NIH.

Travel to scientific meetings is essential for NIH employees and funded researchers, however, in recent years increasing travel restrictions have served as a significant barrier to scientific collaboration. AAP supports this provision.

AAP supports this effort to quickly update guidance to streamline IRBs for multi-site studies which are commonly needed in pediatrics.

## Food and Drug Administration Provisions

- Requires FDA to establish a structured framework for the incorporation of patient experience data into the regulatory decision-making process, including the assessment of desired benefits and tolerable risks associated with new treatments (Sec. 2001)
- Requires FDA to issue guidance on the development of biomarkers, with input from public-private partnerships, and establish a codified process for qualification of biomarkers and other drug development tools. (Sec. 2021)
- Requires FDA to publish guidance on the development of precision medicines. (Sec. 2041)

More inclusion of patient input into FDA’s and industry’s drug development and review process can be important. Questions remain about how FDA would incorporate patient experience data and how useful it can be in drug approvals. FDA must be careful not to lower the approval standard for drugs.

While innovation in the development of biomarkers may help increase access to important therapies for children, FDA must be careful not to lower the approval standard for drugs.

- Requires FDA to hold a public meeting and issue guidance on incorporating adaptive designs and Bayesian statistical modeling into clinical protocols and drug applications. (Sec. 2061)
- Requires FDA to establish a program to evaluate the potential use of evidence from clinical experience to help support the approval of a new indication for a drug and to help support or satisfy post-approval study requirements. (Sec. 2062)
- Places transparency requirements on certain drug companies regarding their expanded access programs (programs for patients to access drugs before they are approved) and requires FDA to finalize guidance regarding how it interprets and uses adverse drug event data resulting from drug use under such expanded access programs. (Sec. 2082-2083)
- Allows drug companies to share certain data and health care economic information with payers and formularies regarding FDA-approved uses of drugs. (Sec. 2101)
- Requires FDA to establish an approval pathway to speed patient access to new antibacterial drugs and biological products for use in a limited population of patients in order to address antimicrobial resistance. (Sec. 2121)
- Allows FDA to utilize appropriate outside expertise to more rapidly update breakpoints to help clinicians determine if a particular infection will be susceptible or resistant to a particular drug. Breakpoints must be kept updated to keep pace with developing resistance to guide appropriate prescribing. (Sec. 2122)
- Establishes a new exclusivity incentive for existing drugs that seek a new indication for a rare disease. Six months of additional marketing exclusivity (additive of any exclusivity awarded under the Best Pharmaceuticals for Children Act). (Sec. 2151)
- Modifies the humanitarian device exemption (HDE) approval pathway by raising the limit on disease prevalence from 4,000 to 8,000 per year. Devices approved under the HDE pathway need only show safety and “probable benefit,” not efficacy. In 2007 and 2012, FDA law was changed to allow devices approved under this pathway to make a profit (rather than merely recouping R&D costs) only if the devices are labeled for pediatrics (or not relevant to pediatrics). (Sec. 2227)

Adaptive study designs and Bayesian modeling are often needed in pediatric trials with small sample sizes. AAP supports this provision to increase attention to these methodologies at FDA.

While clinical experience and data may help inform the drug review process, it must not be considered a substitute for clinical trials and FDA must be careful not to lower the approval standard for drugs.

This provision would allow drug companies to share data on off-label uses of drugs with payers and formularies. Allowing drug companies additional leeway in their ability to communicate about off-label uses may serve as a disincentive for them to pursue expanded FDA-approved labeled indications.

It is often not feasible to conduct a large clinical trial for these drugs because of the small populations on which they will be used. This provision allows drugs to be approved with smaller clinical trials for serious or life-threatening infections. Drugs would have to be labeled in a way that states they are approved for a limited population. This provision is similar to a provision in the AAP-supported ADAPT Act. AAP supports this provision.

AAP supports this provision which will help promote innovation for antimicrobial drugs. This provision is also similar to a provision in the AAP-supported ADAPT Act.

While this provision has the potential to increase the number of devices that seek approval through the HDE pathway, a pathway that can require the pediatric study of devices, it should be carefully monitored to ensure that it does not decrease the number of devices seeking approval under the PMA pathway, which requires more robust data on effectiveness.

- Changes the law to state that research subject to FDA’s human subjects protections is no longer subject to the Common Rule, thereby removing the role of the HHS Office of Human Research Protections (OHRP) in overseeing FDA-regulated research. (Sec. 2261)
- Changes FDA law to allow “minimal risk” research to possibly avoid notifying participants and providing for informed consent (Sec. 2263)
- Expresses congressional support for FDA staff attending scientific meetings. (Sec. 2282)
- Allows FDA additional flexibility to recruit and retain highly qualified employees at higher salaries (Sec. 2281 and Sec. 2285)
- Exempts certain FDA user fees from sequestration. (Sec. 2301)
- Provides FDA with \$550 million over 5 years to implement provisions of the bill. (Sec. 4041)

OHRP plays an important role in protecting patients engaged in medical research. AAP opposes this provision.

It is often ethically necessary for even “minimal risk” research to require informed consent. AAP opposes this provision.

AAP supports this provision and notes the importance of the presence of FDA employees at scientific meetings.

AAP supports this provision to ensure that the FDA has sufficient salary flexibility to recruit and retain highly qualified pediatricians and pediatric subspecialists to work at the agency.

If FDA is not appropriately resourced to carry out the responsibilities required of it by this bill, the agency would have to divert resources from its core mission to ensure the safety and efficacy of drugs.

## Other Provisions

- Requires the Advisory Committee on Immunization Practices (ACIP) to consider the use of a vaccine at the next scheduled ACIP meeting following the vaccine licensure, requires a CDC review of the ACIP process to evaluate consistency in formulating and issuing recommendations for vaccines, and allows for meetings between CDC and vaccine developers upon request of the developer. (Sec. 2141-2143)
- Requires enhances Medicare payment for newly developed antimicrobial therapies and a Government Accountability Office report on barriers to antimicrobial development. (Sec. 2123)
- Modifies functions and responsibilities at HHS, the Office of the National Coordinator for Health Information Technology, and other federal agencies in order to enhance efforts to make electronic health care systems interoperable and increases penalties for blocking or otherwise inhibiting the flow of patient information throughout our healthcare system. (Sec. 3001)
- Establishes a Lyme and Tick-Borne Disease Working Group in HHS to review all efforts within the department concerning Lyme disease and other tick-borne diseases to ensure interagency coordination, minimize overlap, and examine research priorities. (Sec. 4061)

ACIP is already extremely transparent, and undertakes a very deliberative, scientific process to reach their recommendations. Sometimes the data are not understandable within a certain timeframe, so imposing such a timeline would inappropriately allow the sponsor or manufacturer to drive the process. It is also unreasonable to require CDC to gather information for manufacturers that is already in the public domain. Because it compromises the independence of ACIP, the AAP opposes this provision.

This provision is meant to encourage interoperability of data in health IT systems. It would penalize EHR vendors who block the interoperability of data. Unfortunately, it does not contain provisions to encourage more pediatric providers to take up health IT in their practice.

This provision would allow the Lyme and Tick-Borne Disease Working Group to include non-scientists or medical professionals that promote the idea of chronic lyme disease.

- Exempts certain transfers of value related to medical education from the Physician Payment Sunshine Act. (Sec. 3041)

Provision of continuing medical education for pediatricians is essential to promote the best science in pediatric practice. AAP supports this provision which clarifies the interpretation of the PPSA to permit funding for provider education activities unrelated to specific transfers covered under the Act.