

HOUSE COMMITTEE REPORTS OUT 21ST CENTURY CURES BILL NEW FUNDING AND AUTHORITIES PROPOSED FOR NIH

On May 21st, the “21st Century Cures Act” was reported out of the House Energy and Commerce Committee by a vote of 51-0. This legislation has been under development by the Committee for nearly a year and a half and is the fruit of numerous hearings and roundtables as well as countless meetings between stakeholders and Members of Congress and their staffs. As Committee Chairman Fred Upton (R-MI) has explained, the purpose of the Cures Initiative is to explore steps which can be taken to accelerate the pace of cures in America. Accordingly, the Committee examined the “full arc of this process – from the discovery of clues in basic science, to streamlining the drug and device development process, to unleashing the power of digital medicine and social media at the treatment delivery phase.” Complete details on the 21st Century Cures Act are available at <http://energycommerce.house.gov/cures>.

Certain provisions of the 21st Century Cures Act will also be considered by the House Ways and Means Committee before it is taken to the House floor for a vote on final passage. The Committee has not yet scheduled a time for acting on the bill but the sponsors of the legislation would like to have it passed by the full House prior to the 4th of July recess.

Action on complementary legislation in the Senate is underway but lags many months behind the House. The Senate Health, Education, Labor and Pensions Committee is pursuing an initiative which it has titled “Innovations for Healthier Americans”. The effort has bipartisan support and thus far the Committee has conducted several hearings and intends to convene many more before it produces a legislative proposal. Senate HELP Committee Chairman Lamar Alexander (R-TN) has indicated that he would like to complete action on the legislation by the end of the year or early in 2016.

One of the primary purposes of the House 21st Century Cures Act is to increase funding for the National Institutes of Health and improve the function and operation of the agency. The bill also contains provisions to support young emerging scientists. A summary of the NIH provisions in the legislation follows.

Page 2 -- Funding for the National Institutes of Health

Page 2 -- NIH Planning and Administration

Page 2 - Strategic Plan

Page 3 - Accountability

Page 3 - Administrative Burden

Page 3 - NCATS Phase IIB Restriction

Page 3 - High-risk, high-reward research

Page 4 -- Supporting Young Emerging Scientists
Page 4 -- Capstone Grant Program
Page 4 -- Promoting Pediatric Research through NIH
Page 4 -- Advancing NIH Research and Data Access
Page 4 -- Facilitating Collaborative Research
Page 5 -- Council for 21st Century Cures

Funding for the National Institutes of Health

The bill funds NIH for FY 2016 (\$31,811,000,000), FY 2017 (\$33,331,000,000), and FY 2018 (\$34,851,000,000) and establishes an NIH Innovation Fund to support biomedical research through the financing of basic, translational, and clinical research. The Innovation Fund provides NIH with an additional \$2 billion per year for the next five years, FY 2016 – FY 2020—a total of \$10 billion in mandatory appropriations. The proposal requires that \$500 million be allocated each year to the Accelerating Advancement Program, a new program in which national research institutes and national centers match funding provided by the Innovation Fund to “accomplish important biomedical research objectives.” Of the remaining \$1.5 billion in annual funds at least 35 percent must be for early stage investigators and at least 20 percent must be for high-risk, high-reward research. No more than \$200 million can be allocated to intramural research in a given year.

The NIH Innovation Fund may only be used to conduct or support innovative biomedical research that falls into the following categories:

- Research in which a principal investigator has a specific project or specific objectives and funding is tied to pursuit of such project or objectives;
- Research in which a principal investigator has shown promise in biomedical research and funding is not tied to a specific project or specific objectives;
- Research to be carried out by an early stage investigator (defined as a principal investigator who has never been awarded, or has been awarded only once, a substantial, competing grant by the NIH for independent research and is within 10 years of having completed a terminal degree or medical residency);
- Research to be carried out by a small business concern (as defined in section 3 of the Small Business Act);
- Research under the Accelerating Advancement Program; and
- Development and implementation of the strategic plan (see below).

NIH Planning and Administration

Strategic Plan

The legislation requires the Director of NIH to develop and maintain a biomedical research strategic plan that (1) is designed to increase the efficient and effective focus of biomedical research in a manner that leverages the best scientific opportunities through a deliberative planning process; (2) identifies strategic focus areas, such as biomarkers, precision medicine, infectious diseases, and antibiotics; (3) includes objectives for each such strategic focus area; and (4) ensures that basic research remains a priority. The strategic plan is to be used

to identify research opportunities and should include individual plans for the research activities of each of the national research institutes and national centers.

The provisions of the bill direct that the strategic focus areas should be identified in a manner that considers their return on investment and includes overarching and trans-National Institutes of Health strategic focus areas, to be known as “Mission Priority Focus Areas” (“MPFA”). The MPFAs should best serve the goals of preventing or eliminating the burden of a disease or condition and scientifically merit enhanced and focused research over the next 5 years. The bill also requires the NIH Director to ensure that rare and pediatric diseases and conditions remain a priority. The initial plan provides NIH with 270 days to develop the first five-year plan and requires annual progress reviews for each strategic focus area.

Accountability

The 21st Century Cures Act sets five year terms for directors of national research institutes and national centers, permits the Director of the NIH to remove directors prior to the expiration of their term, and allows directors to be reappointed for an unlimited number of terms. In addition, the directors of national research institutes and national centers must review and approve “R-series” grants and requires the Institute of Medicine of the National Academies to complete a study on the extent to which biomedical research conducted or supported by Federal agencies is duplicative.

Administrative Burden

The legislation directs the NIH Director to implement measures to reduce the administrative burden of researchers funded by NIH and provides the agency with certain exemptions from Paperwork Reduction Act requirements.

NCATS Phase IIB Restriction

The Committee’s proposal allows the National Center for Advancing Translational Sciences (“NCATS”) to support clinical trial activities through the end of phase III, so long as (1) the Center gives public notice for a period of at least 120 days of its intention to support the clinical trial activities in phase III; (2) no public or private organization provides credible written intent to the Center that the organization has timely plans to further the clinical trial activities or conduct clinical trials of a similar nature beyond phase IIB; and (3) the Center ensures that support of the clinical trial activities in phase III will not increase the Federal Government’s liability beyond the award value of the Center’s support. Previously, NCATS could only support these activities through the end of phase IIB. The Director of NCATS is also provided with additional “transactions authority” when funding Cures Acceleration Network program projects.

High-risk, high-reward research

The bill requires the directors of national research institutes to establish programs to conduct or support research projects that pursue innovative approaches to major contemporary challenges in biomedical research that involve inherent high risk, but have the potential to lead to breakthroughs. Directors are to set aside a specific percentage of funding, as determined by the Director of NIH, for high-risk, high-reward research.

Supporting Young Emerging Scientists

Time and again during the Committee's hearings and roundtables, concerns were raised that young scientists were not entering the research field. In order to assist young emerging scientists, the 21st Century Cures proposal establishes a loan repayment program in which the Federal Government will pay up to \$50,000, adjustable for inflation, of the principal and interest of a health professional's student loans in return for research. Eligible health professionals must have "a substantial amount of educational loans relative to income." In addition, the proposal raises the cap on other NIH loan repayment programs from \$35,000 to \$50,000 and requires the Director to submit to Congress a report on NIH efforts to attract, retain, and develop emerging scientists.

Capstone Grant Program

The legislation establishes a "Capstone Award" program for "outstanding scientists who have been funded by the NIH" which is intended to facilitate the successful transition or conclusion of research programs. The duration and amount of each Capstone Award is determined by the Director of NIH in consultation with the directors of the national research institutes and centers.

Promoting Pediatric Research through NIH

The NIH Director is required to establish a National Pediatric Research Network comprised of, as appropriate, the pediatric research consortia, in order to more effectively support pediatric research and optimize the use of Federal resources. The bill calls on NIH to use grants, contracts, or cooperative agreements to "encourage a global pediatric clinical study network" and directs NIH to hold a workshop and publish guidelines addressing the consideration of age as an inclusion variable in research involving human subjects and identifying criteria for justifications for any age-related exclusion in such research.

Advancing NIH Research and Data Access

The 21st Century Cures legislation grants the Director of NIH authority, subject to certain limitations, to require recipients of NIH grants whose research was fully funded by NIH to share scientific data generated from their research. Data generated through NIH-funded research is to be standardized and "easily used by the public". Specifically, the information submitted to the registry and results data bank must include: (1) the disease or indication being studied; (2) inclusion criteria such as age, gender, diagnosis or diagnoses, lab values, or imaging results; and (3) exclusion criteria such as specific diagnosis or diagnoses, lab values, or prohibited medications. The Secretary of Health and Human Services is required to, within 90 days of the Act's passage, consult with stakeholders to receive advice on enhancements to the clinical trial registry data bank.

Facilitating Collaborative Research

The bill mandates the establishment of a seven-year Clinical Trial Data System pilot program through the Commissioner of Food and Drugs and the Director of the National Institutes

of Health. The program would release de-identified clinical trial data from "qualified clinical trials," including trials on drugs and medical devices, for purposes of registered users conducting further research.

The legislation also establishes a National Neurological Diseases Surveillance System and gives the Secretary of HHS the authority to enter into public-private partnerships focused on (1) cooperating with other entities to sponsor or maintain disease registries, including disease registries and disease registry platforms for rare diseases; (2) developing or enhancing a secure information technology system that has the capacity to support data needs across a wide range of disease studies and is easily modified as knowledge is gained during such studies; and (3) providing advice to clinical researchers, patient advocacy groups, and other entities with respect to the design and conduct of disease studies, the modification of any such ongoing studies, and, addressing associated patient privacy issues.

Council for 21st Century Cures

The Committee's proposal establishes a nonprofit corporation to be known as the Council for 21st Century Cures. The Council will be a public-private partnership created to "foster collaboration and coordination among the entities that comprise the Council, including academia, government agencies, industry, health care payors and providers, patient advocates, and others engaged in the cycle of discovery, development, and delivery of life-saving and health-enhancing innovative interventions." The Council will:

- undertake communication and dissemination activities;
- publish information on certain activities funded by NIH;
- establish a strategic agenda for accelerating the discovery, development, and delivery in the United States of innovative cures, treatments, and preventive measures for patients;
- identify gaps and opportunities within and across the discovery, development, and delivery cycle;
- develop and propose recommendations based on the gaps and opportunities so identified;
- facilitate the interoperability of the components of the discovery, development, and delivery cycle;
- propose recommendations that will facilitate precompetitive collaboration;
- identify opportunities to work with, but not duplicate the efforts of, nonprofit organizations and other public-private partnerships; and
- identify opportunities for collaboration with organizations operating outside of the United States, such as the Innovative Medicines Initiative of the European Union.

The Council will be comprised of the Director of the NIH; the Commissioner of Food and Drugs; the Administrator of the Centers for Medicare & Medicaid Services; the heads of five other Federal agencies deemed by the Secretary to be engaged in biomedical research and development; and 17 members appointed by the Comptroller General representing the biopharmaceutical industry (4), the medical device industry (2), the information and digital technology industry (2), academic researchers (2), patients (3), health care providers (2), and health care plans and insurers (2).