

DEPARTMENT OF HEALTH AND HUMAN SERVICES

NATIONAL INSTITUTES OF HEALTH

Hearing on 21st Century Cures Implementation: Updates from
FDA and NIH

Witness appearing before the
House Committee on Energy and Commerce Subcommittee on Health

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Accompanied by

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and

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ChairmanBurgess

Inclusion

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drugs and devices over the years that were tested in clinical trials. WULD OV KDYHQ ¶ W DO
included the full spectrum of humanity and this limits the applicability of study results. It also
limits our ability to target therapies and address disparities. Congress helped NIH address this
issue through the Cures Act at three focus areas: inclusion of children and seniors; inclusion of
pregnant and lactating women; and continuing our focus on women and racial and ethnic
minorities

On June 12, 2017 as required by the Cures Act, NIH held a workshop on inclusion
across the lifespan. It might seem easy to include all age ranges but both children and older
adults require special considerations. At the workshop, investigators with expertise in
conducting clinical studies with pediatric and older populations, ethics experts, and other
stakeholders had a robust discussion about barriers and facilitators to the inclusion of volunteers
of all ages in research. The findings and recommendations were presented at an Advisory
Committee meeting on December 14-15, 2017, and on December 19, 2017 we announced that
we were revising the NIH Policy and Guidelines on the Inclusion of Children to apply to
individuals across the lifespan.³ The revisions broaden the policy to address inclusion of
research participants of all ages and will apply beginning in January 2019 to all supported
research involving human subjects.

The Cures Act also asks NIH to continue making progress on the inclusion of women and
ethnic and racial minority populations in research. This has been a partnership of the Congress
and NIH for many years. In the Congress authorized both the NIH Office of Research on

³ <https://grants.nih.gov/grants/guide/notifications/NOT-OD-18-116.html>

Based on the outcome of the Task Force meeting report with the final recommendations has been developed and will be sent to the HHS Secretary and Congress by September 2018. NIH is grateful to the Congress for recognizing the need for careful consideration in this area of research and looks forward to addressing any recommendations made by the Task Force as determined by the Secretary.

Strengthening Biomedical Workforce

NIH and its stakeholder community have for many years been concerned about the long term stability of the biomedical research enterprise. Since 2003, more than 20 percent of its purchasing power from 2003 to 2015, researchers were forced to vie for limited resources, leading to a hypercompetitive environment. With success rates below 20 percent, many highly meritorious applications were going unfunded. This too often resulted in misaligned incentives and unintended consequences for talented researchers at all career stages who were trying to succeed and stay in science. This kind of environment can be particularly challenging for many new and mid-career investigators.

Over the last several years, NIH has taken numerous steps to balance, strengthen, and stabilize the biomedical research workforce, but these measures have only taken us so far. By 2015, the percentage of NIH awards that support early career investigators went from declining to flat, those gains were offset by a decline in the percentage of NIH awards that supported mid-career investigators.

As a direct result of the Cures Act, in June 2017 NIH launched the Next Generation Researchers Initiative⁵ aimed at strengthening the biomedical workforce with a focus on early career investigators or investigators who are at an early stage in their career. NIH intends to take a multi-pronged approach, which we outlined in an article published on November 2017,⁶ to increase the number of NIH-funded early stage investigators and stabilize the career trajectory of scientists at all stages.

NIH is developing evidence-based, data-driven strategies to assure that NIH investments are directed in ways that maximize scientific output. We are being aided in these efforts by an expert Working Group of the Advisory Committee to the Director, who will present recommendations in December 2018. But several important steps are already being taken: Institutes and Centers are placing greater emphasis on current NIH funding programs to identify, grow, and retain new and early-career investigators across these critical career stages. The NIH

⁵ <https://grants.nih.gov/ngri.htm>

⁶ Michael Lauer, Lawrence Tabak, and Francis Collins. [Opinion: The Next Generation Researchers Initiative at NIH](#). *NIH*. 11/20/17

EUREKA prize competition. The purpose of soliciting additional public comments was to seek
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activity duplicates other ongoing activities in any relevant sector (e.g., academia, business), (2)
the attractiveness of this question to a broad audience of possible solvers, (3) the length of time
solvers would need to develop a prize submission, and (4) metrics that judges might use to
identify a winner. Comments were due on July 1, 2018. The NIA incorporated relevant input
received into a final prize announcement for Challenge.gov, planned for release prior to
September 31, 2018.

NIH also formed the EUREKA Prize Coordination Committee to review future proposals
for future EUREKA prize competitions and funded the National Academy of Sciences (NAS) to
study Innovation Prizes and Federal R&D with specific emphasis on strategies to determine
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also consider the st

The All of Us Research Program (described below) aims to enroll one million individuals in a decade-long research project. That ambitious goal requires flexibility, complex and dynamic interactions, and ways to engage traditional NIH awardees to advance the mission. For example, All of Us has used OAA to make awards to the Healthcare Provider Organizations to help build the research protocols, test enrollment procedures, and collect essential health data and biological specimens.

The Cures Act also recognizes that two of the cornerstones of scientific advancement are rigor in designing and performing scientific research and the ability to reproduce biomedical research findings. In recent years, the scientific community has become aware of the need to improve rigor and reproducibility. In 2014, NIH worked with scientific publishers to develop a set of principles and goals that publishers have now endorsed. As the Cures Act requires, my Advisory Committee has convened a Working Group on Rigor and Reproducibility, and they are reviewing the experience of the last few years, leading to the development of recommendations for a formal policy. I look forward to updating you as this effort takes shape.

NIH Innovation Fund

Last, but certainly not least, the Cures Act provided multi-year funding through the NIH Innovation Fund for four highly innovative scientific research initiatives: the Precision Medicine Initiative (PMI), the Brain Research through Advancing Innovative Neurotechnologies® (BRAIN) Initiative, the Cancer Moonshot, and the Regenerative Medicine Innovation Project. As required by the Cures Act, on March 28, 2017, I solicited recommendations from my Advisory Committee on how to allocate the funds. We had a robust conversation about each of the initiatives and the Advisory Committee members provided critical advice on how to move

forward. As a result of that discussion, and conversations with NIH colleagues, we drafted the NIH Innovation Fund Work Plan which was submitted to Congress in September 2017 outlining how the agency will use the NIH Innovation Funds for each of the four initiatives. I would like to tell you a bit about each of these initiatives and how the NIH Innovation Fund is helping to move each initiative forward.

The Precision Medicine Initiative

Precision medicine is a revolutionary approach for disease prevention and treatment that takes into account individual differences in lifestyle, environment, and biology. While some applications of precision medicine have found their way into practice over the years,

diverse communities to join and remain in the program, with a focus on those traditionally underrepresented in biomedical research.

We began a robust, year-long beta phase in May 2017, during which each of our partners were able to test their systems and processes to ensure a good experience for participants. We are excited to tell you that All of Us launched nationally on May 6, 2018 with events across the country. As of July 10, 2018, 85,360 individuals have started the enrollment process, and 2,415 have completed all the steps in the protocol. Of those, 75% are from communities that have been historically underrepresented in biomedical research and almost 50% are specifically African American.

brain that, for the first time, shows how individual cells and complex neural circuits interact in both time and space. Long desired by researchers seeking new ways to cure, and even prevent brain disorders, this picture is filling major gaps in our current knowledge and providing unprecedented opportunities for exploring exactly how the brain enables the human body to record, process, utilize, store, and retrieve vast quantities of information, all at the speed of thought.

NIH leveraged the Cures Act, FY 2017 Innovation Funds, in addition to our annual appropriation, to launch 10 exciting new research projects and in FY 2018 NIH expects to fund approximately 150 new BRAIN Initiative projects. Cures funds will support critical areas including data infrastructure and sharing, the BRAIN Initiative Cell Census Network (which is developing an atlas of brain cell types), the Team Research Brain Circuits Program, and human brain studies. In human studies, the BRAIN Initiative is advancing brain imaging and non-invasive brain stimulation, and public-private partnerships are investigating implanting implanted brain stimulation therapies that are already showing promise. Ultimately, this will lead to an increased understanding of brain health, and means of preventing brain disorders such as stroke, Alzheimer's disease, and traumatic brain injury.

The Cancer MoonshotSM

The Cancer Moonshot¹² funded in the Cures Act, has an ambitious goal to dramatically speed advances in cancer prevention, diagnosis, treatment, and care. To identify the most promising and innovative strategies, the National Cancer Institute (NCI) solicited direct input from the

¹¹ <https://www.braininitiative.nih.gov/funding/fundedAwards.htm>

¹² <https://www.cancer.gov/research/initiatives/moonshotcancerinitiative>.

public and convened a Blue Ribbon Panel (BRP)

for carrying out scientific research and protecting patient safety. The \$2 million Cures provided for this initiative in FY 2017 were amplified through matching funds and NIH Institute contributions to support research totaling \$5.4 million. NIH has worked in close collaboration with the FDA to implement the RMIP. In September, NIH made eight clinical research awards that cover a broad spectrum of science and new technologies, and have the potential to advance understanding and treatment of common diseases including diabetes, anemia, corneal and other eye diseases, and chronic skin ulcers, as well as rare diseases, including idiopathic pulmonary fibrosis, inherited skin diseases, and sickle cell disease.

Several awards will explore the use of adult disg1 72.024 681.58 Tm 0 g [(for)6(thi)-aMC 11nt

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depth stem cell characterization, support to address regulatory requirements, manufacturing
assistance for preparation of clinical grade stem cell products, and storage and sharing of clinical
data. NIH looks forward to the opportunity the Cures Act provides to significantly advance this
field of science.

Conclusion

Thank you for your leadership and dedication that resulted in enacting the Cures Act 18
months ago. The Cures Act has provided NIH with critical resources and tools to advance our
mission to seek fundamental knowledge about the nature and behavior of living systems and
the application of that knowledge to enhance health, lengthen life, and reduce illness and
disability. We appreciate & R Q J U H V V ¶ V V X S S R U W I R U 1 , + W K U R X J K W K H
implement the law to accelerate scientific discoveries and develop new approaches to the
prevention, treatment, and cure of disease