21st Century Cures: An Update on the President's Council of Advisors on Science and Technology 2012 Report on Propelling Innovation

Overview

In September 2012, the President's Council of Advisors on Science and Technology (PCAST) issued a *Report to the President on Propelling Innovation in Drug Discovery, Development, and Evaluation* responding to President Obama's request for recommendations on this topic.

In the report, the president's council acknowledges that while "[t]he past quarter-century has seen tremendous progress in biomedical research" and such "breakthroughs are beginning to pay off in terms of new therapies for American patients," much work remains to be done since "the pace of new therapeutic development has not kept up with the explosion in scientific knowledge."[1] PCAST identifies opportunities and puts forth proposals to achieve an ambitious goal for our nation:

> Double the current annual output of innovative new medicines for patients with important unmet medical needs, while increasing drug efficacy and safety, through industry, academia, and government working together to double the efficiency of drug development, by decreasing clinical failure, clinical trial costs, time to market, and regulatory uncertainty.[2]

In its report, PCAST comments that "such a goal is attainable over the next 10-15 years," but that it "will require advances in: the science of drug development; the execution of clinical trials; the development pathways used for innovative medicines; the mechanisms for drug

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[1] PRESIDENT'S COUNCIL OF ADVISORS ON SCIENCE AND TECHNOLOGY, EXECUTIVE OFFICE OF THE PRESIDENT, REPORT TO THE PRESIDENT ON PROPELLING INNOVATION IN DRUG DISCOVERY, DEVELOPMENT, AND EVALUATION iii (Sept. 2012) *available at* http://www.whitehouse.gov/sites/default/files/microsites/ostp/pcast-fda-final.pdf.

[2] *Id. at* 51. The report clarifies that such a goal means that "the time and cost of projects that begin in the 2020s will be two-fold lower than costs and development times for current projects."

approval, surveillance and communication of risk; and management at the FDA."[3]

We are seeking feedback on the proposals put forth by PCAST, and whether any of them have been further discussed or implemented. Further, we are seeking to learn how PCAST's ideas could potentially advance the committee's 21st Century Cures initiative. How can these proposals help accelerate the discovery, development, and delivery of innovative new treatments and cures, creating more jobs, and maintaining our nation's role as the innovation capital of the world?

The report details the eight broad recommendations listed below, along with numerous subsets of ideas and action items related to each. Many of these - though not all - would require congressional authorization or involvement to effectuate.

Recommendation 1: Support Federal Initiatives to Accelerate Therapeutics

Recommendation 2: Catalyze the Creation of a Broad-Based Partnership to Accelerate Therapeutics

Recommendation 3: Expand the Use in Practice of FDA's Existing Authorities for Accelerated Approval and Confirmatory Evidence[4]

Recommendation 4: Create a New Pathway for Initial Approval of Drugs Shown to be Safe and Effective in a Specific Subgroup of Patients

Recommendation 5: Explore Approaches for Adaptive Approval Via Pilot Projects Under Existing Pathways, but Do Not Create New Adaptive Approval Pathways Through Legislation

Recommendation 6: Improve FDA's Tools for Monitoring and Communication of Clinical Benefits and Risks

Recommendation 7: Reform Management Practices at FDA

Recommendation 8: Study Current and Potential Economic Incentives to Promote Innovation in Drug Development

Input on the PCAST report in general and as it may relate to any or all of these specific recommendations, ideas, and action items is welcome. We request all submissions and suggestions be sent to cures@mail.house.gov by June 10, 2014.

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[3] *Id.*

[4] The PCAST report notes on page 61 that in 2012 the Food and Drug Administration Safety and Innovation Act (FDASIA) authorized expanded use of the Accelerated Approval pathway and that "FDA should make fuller use of authorities previously granted by legislation and not yet fully utilized."