THE COMMITTEE ON ENERGY AND COMMERCE

MEMORANDUM

May 16, 2014

To: Members, Subcommittee on Health

From: Majority Committee Staff

Re: Hearing on "21st Century Cures: The President's Council of Advisors on Science and Technology (PCAST) Report on Drug Innovation"

On Tuesday, May 20, 2014, the Subcommittee on Health will hold a hearing entitled "21st Century Cures: The President's Council of Advisors on Science and Technology (PCAST) Report on Drug Innovation." The Subcommittee will convene at 10:00 a.m. in 2322 Rayburn House Office Building to hear from experts involved in the development of the PCAST *Report to the President on Propelling Innovation in Drug Discovery, Development, and Evaluation*, which was issued in September 2012 in response to President Obama's request for recommendations on this topic.

I. WITNESSES

- Garry A. Neil, M.D., Global Head of Research and Development, Medgenics, Inc.;
- Sara Radcliffe, Executive Vice President of Health Section, Biotechnology Industry Organization;
- Frank J. Sasinowski, Director, Hyman, Phelps & McNamara, PC;
- Jeff Allen, Executive Director, Friends of Cancer Research; and,
- Sean Tunis, M.D., Founder and Chief Executive Officer, Center for Medical Technology Policy.

II. BACKGROUND

The PCAST report 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]

---

[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.

After consulting with a wide range of stakeholders, PCAST identified opportunities and put forth constructive 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]

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

The PCAST report details eight broad recommendations listed below, along with numerous subsets of ideas and action items related to each, including many—though not all—that 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; and,
- Recommendation 8: Study Current and Potential Economic Incentives to Promote Innovation in Drug Development.

---

[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."
[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."

This hearing will provide an opportunity to hear from several experts involved in the development of the report and to explore the landscape of issues raised and proposals put forth.

III. STAFF CONTACT

If you have any questions regarding the hearing, please contact John Stone, Carly McWilliams, Paul Edattel, Brenda Destro, or Katie Novaria at (202) 225-2927.