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ONE HUNDRED THIRTEENTH CONGRESS

Congress of the United States

House of Representatives

COMMITTEE ON ENERGY AND COMMERCE

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June 10, 2014

Ms. Sara Radcliffe
Executive Vice President, Health
Biotechnology Industry Organization
1201 Maryland Avenue, S.W., Suite 900
Washington, D.C. 20024

Dear Ms. Radcliffe:

Thank you for appearing before the Subcommittee on Health on Tuesday, May 20, 2014, to testify at the hearing entitled "21st Century Cures: The President's Council of Advisors on Science and Technology (PCAST) Report on Drug Innovation."

Pursuant to the Rules of the Committee on Energy and Commerce, the hearing record remains open for ten business days to permit Members to submit additional questions for the record, which are attached. The format of your responses to these questions should be as follows: (1) the name of the Member whose question you are addressing, (2) the complete text of the question you are addressing in bold, and (3) your answer to that question in plain text.

Also attached are Member requests made during the hearing. The format of your responses to these requests should follow the same format as your responses to the additional questions for the record.

To facilitate the printing of the hearing record, please respond to these questions and requests with a transmittal letter by the close of business on Tuesday, June 24, 2014. Your responses should be mailed to Sydne Harwick, Legislative Clerk, Committee on Energy and Commerce, 2125 Rayburn House Office Building, Washington, D.C. 20515 and e-mailed in Word format to **Sydne.Harwick@mail.house.gov**.

Thank you again for your time and effort preparing and delivering testimony before the Subcommittee.

Sincerely,

Joseph R. Pitts
Chairman
Subcommittee on Health

cc: The Honorable Frank Pallone, Jr., Ranking Member, Subcommittee on Health

Attachments

Attachment 1—Additional Questions for the Record

The Honorable Leonard Lance

1. Thank you for your testimony during the May 20 Health Subcommittee hearing on the President's Council of Advisors on Science and Technology Report on Drug Innovation. At the hearing, you testified about the extraordinary expense to bring a new drug, biologic or diagnostic to market and the critical importance of developing and ensuring policies that incentivize investment in the "next generation of biomedical discoveries, treatments and cures." I also agree with BIO's testimony that to ensure investments by venture capitalists, we need an "FDA regulatory framework that is predictable, consistent and well-resourced." The Special Protocol Assessment (SPA) process was created by Congress to create such a framework, and until recently, has promoted innovation and investment in the development of new medicines essential for patient well-being. Do you believe the integrity of the SPA process is essential to ensuring continued investment in biomedical research? If the Agency were to stray from statutory guidance in the SPA decision-making process, what impact would such actions have on the availability of private funding for research and development?

The Honorable Gus Bilirakis

1. In the world of rare diseases or orphan drugs, there are almost 7,000 diseases affecting about 30 million Americans. Most of these diseases have no treatment. In the last FDA user fee agreement, Congress rewrote the statute on Accelerated Approval and directed FDA to expand the Accelerated Approval pathway beyond HIV/AIDS and oncology.

   The FDA has released guidance on the implementation of this expedited approval pathway. What is BIO's opinion of the guidance that was released? Do you feel confident that accelerated approval is being implemented the way Congress intended?

2. One mechanism drug companies have to improve certainty about the Agency's acceptance of certain trial designs is to enter into a Special Protocol Assessment (SPA) agreement, which was first authorized in 2007 for that very purpose. Have these agreements generally brought the intended certainty to companies and has the Agency always held up its end of the binding contract?

3. For Accelerated Approvals to work, the FDA needs to be comfortable using surrogate endpoints that are reasonably likely to predict a clinical benefit. The Report to the President talks about how the biomedical research community should take a more active role in determining endpoints. How can FDA work with stakeholders to determine new endpoints that are reasonably likely to predict a clinical endpoint? Has the FDA been receptive to working with stakeholder on this?

4. What barriers are currently in place that limit the potential of using clinical and outcomes data to learn more about how therapies are working on patients in the real world? How should we address them?

5. Once a drug is on the market, PCAST asserts that the economic incentives for drug companies to conduct further clinical trials to obtain formal approval for additional indications may be low. The report also points to the many difficulties of enrolling patients in clinical trials after the drug is already on the market. That being said, data about how the drug is working on patients in the real world is not confined to the indications approved for marketing. How can this real world data be leveraged for supplemental applications?

6. As a Member of Congress, we hear tales about how companies meet with FDA on drug approval, and about their frustration with the process sometimes. Reviewers change during the approval process or may lack expertise about the latest science in a given area. How can FDA work with stakeholders to ensure that their management and review team is knowledgeable about the latest science?

The Honorable Jan Schakowsky

1. A recent NPR story discussed a gentleman who is very sick with Hepatitis C but who is unable to afford the new Hepatitis C treatment. According to the report, the new Hepatitis C drug treatment costs about $100,000 per year. This is an example of a widespread disease where a treatment exists but cannot be accessed by all who need it. What can we do to develop a system where everyone can access and afford the new treatment and cures developed through investments in drug innovation?

2. I have been a long-time advocate for increasing funding for the National Institutes of Health. Our investment in research saves lives and improves health. Adequately funding the NIH is also critical in helping to train our next generation of scientific leaders as well as supporting jobs in communities throughout this country. As you know, total inflation-adjusted funding for NIH peaked in fiscal year 2003, meaning that NIH had its largest purchasing power that year. As compared to 2003, inflation-adjusted funding is down 22.1% for fiscal year 2014. Would you explain what this dramatic reduction in purchasing power at the NIH means to the pace of drug innovation? How has this reduction affected our ability to develop our future scientific workforce and how does this harm our biomedical research capacity? Are there other ways that this reduction is affecting the pace of discovery of new cures and treatments?

   Thank you for your important testimony. Your testimony makes clear the harm caused by inadequately funding the NIH. I hope that we can work together to ensure that NIH has the resources it needs to ensure that we remain the world's leader in innovation and that we accelerate our ability to discover new treatments and cures that save lives and improve health.

Attachment 2—Member Requests for the Record

During the hearing, Members asked you to provide additional information for the record and you indicated that you would provide that information. For your convenience, descriptions of the requested information are provided below.

The Honorable H. Morgan Griffith

1. What legal barriers currently exist that limit the potential for doctors, researchers and drug companies to communicate on how therapies are working for patients in the real world? What can we do to break down some of those legal barriers that are preventing reasonable and valuable treatments from getting to the patients?