

ONE HUNDRED THIRTEENTH CONGRESS  
**Congress of the United States**  
**House of Representatives**  
COMMITTEE ON ENERGY AND COMMERCE  
2125 RAYBURN HOUSE OFFICE BUILDING  
WASHINGTON, DC 20515-6115  
Majority (202) 225-2927  
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July 1, 2014

Mr. Alexis Borisy  
Partner  
Third Rock Ventures  
29 Newbury Street  
Boston, MA 02116

Dear Mr. Borisy:

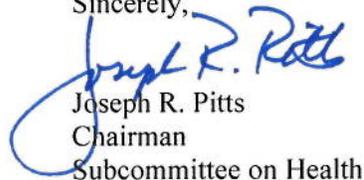
Thank you for appearing before the Subcommittee on Health on Wednesday, June 11, 2014, to testify at the hearing entitled "21st Century Cures: Examining the Role of Incentives in Advancing Treatments and Cures for Patients."

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.

To facilitate the printing of the hearing record, please respond to these questions with a transmittal letter by the close of business on Wednesday, July 16, 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](mailto: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

Attachment

## Attachment—Additional Questions for the Record

### The Honorable Joseph R. Pitts

1. The size and cost of clinical trials is an impediment to investment and innovation, particularly for products treating diseases that impact large patient populations. How can advances in technology make trials more efficient?
2. Understanding that lengthy clinical trials with a large number of participants are currently the norm for drugs treating chronic diseases such as heart diseases and stroke, what processes does FDA have in place to provide the necessary certainty to sponsors up front so that, when resources are devoted to drug development in these areas, investors and companies can plan accordingly?

### The Honorable Michael C. Burgess

1. Would you comment on some of the barriers that Class III medical device manufacturers face when seeking coverage and payment from CMS for innovative cutting edge technology that improves the lives of patients?

### The Honorable Cathy McMorris Rodgers

1. Your testimony specifically references the length of clinical trials as being an impediment to investment. What are some specific ideas on what we could do to streamline the way trials are conducted? How would this affect investment in the bio-pharma space?
2. How can we improve our existing research structure in a way which incentivizes more investment? What is the possibility for clinical trial networks? Or more partnerships with NIH? How about the interaction of the SBIR/STTR program with NIH?
3. You mention the need for FDA to allow for the utilization of modern tools—such as biomarkers and personalized medicine to diagnostically define subsets of a disease. Do you think the FDA and its current regulatory framework is equipped to approve these types of products? Do you think there are adequate incentives in the market for these types of innovative diagnostics?

### The Honorable Gus Bilirakis

1. Your testimony mentioned that FDA allows for the use of novel endpoints, biomarkers and non-traditional clinical trial designs, but lacks transparency and consistency in their approach. How can we improve the process and encourage regulators to use every tool in their proverbial toolbox?
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. What barriers are currently in place that limit that 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?

4. In your testimony, you touch on the need for certainty after approval and the challenge of ensuring that there is coverage of a new drug or device by Medicare, Medicaid or private insurance. Typically, commercial insurers cover something that Medicare covers. What are the challenges that are faced getting covered and reimbursed under Medicare?
5. You mentioned that in Europe they have something called the adaptive licensing pilot and that could help modernize our regulatory system. Would you talk more about this program and how it could be used in the United States?