



21st Century Cures – Patients

***“There are 7,000 known diseases.
We have treatments for only 500 of them.
We have work to do.”¹***

For patients and their families, the gap between the number of diseases and the number of treatments is not a statistic; it is their daily struggle. Despite the medical breakthroughs of recent years, for many diseases, treatments or research simply do not exist.

Through legislation such as the Food and Drug Administration Safety and Innovation Act and the PREEMIE Reauthorization Act, which included the National Pediatric Research Network Act, the Energy and Commerce Committee has sought bipartisan solutions to facilitate and accelerate patient access to innovative treatments. Although progress has been made, significant work remains. That is why we launched the 21st Century Cures initiative, and the involvement and guidance of those patients on the frontlines and their advocates is critical. Our goal of accelerating the cycle of discovery, development, and delivery of promising new treatments and cures is shared by many, but perhaps most of all by patients and their families.

The committee appreciates that certain aspects of the discovery, development, and delivery cycle have different meanings for different patients. Depending on the condition or disease at issue, the state of biomedical research, and the translation of such research into treatments and cures, varies. We want to hear about the state of biomedical research and therapeutic innovation for specific diseases and better understand how Congress can help move the ball forward.

To help the committee’s effort, we are seeking input from the patient community on the following questions:

- What is the state of discovery of cures and treatments for your disease? Are there cures and treatments now or on the horizon?

¹ Statement of Margaret Anderson, Executive Director of FasterCures, at the 21st Century Cures Roundtable held on May 6, 2014, <http://www.youtube.com/watch?v=Fr4Re7sfDzE&t=33m21s>.

- What programs or policies have you utilized to support and foster research, such as patient registries, public-private partnerships, and venture philanthropy?
- How can Congress incentivize, coordinate, and accelerate basic research for diseases we know relatively little about?
- How can we work together to better translate advances in science into safe and effective new therapies for patients?
- How do you coordinate your research and outreach with other patients?
- How do you learn about new treatments and cures? How do you communicate with other patients regarding treatments and cures?
- What can we learn from your experiences with clinical trials and the drug development process?
- What is the role of government in your work, including any barriers to achieving your goals and advancing breakthroughs?
- How should regulators evaluate benefit-risk? How do you work with regulators regarding benefit-risk? Can this process be improved?
- What is the role of public and private funding in the research and development of cures and treatments?
- Are there success stories the committee can highlight and best practices we can leverage in other areas?
- How have you worked with other patients to support one another?
- What is the financial burden of your disease? How would better treatments and cures help save money for your family and the federal government?
- How can Congress help?

Public input is critical to the 21st Century Cures initiative, especially from the patient community. With the staggering gap between the number of diseases and available treatments, there are undoubtedly countless untold stories that will provide guidance and perspective in this effort. We request all submissions and suggestions be sent to cures@mail.house.gov by June 13, 2014.