

**Opening Statement of Chairman Fred Upton**  
**Subcommittee on Health Legislative Hearing on 21<sup>st</sup> Century Cures**  
**April 30, 2015**

First, I'd like to talk about how we got here today. These two little girls (FSU holds up photo with the girls), my friends Brooke and Brielle Kennedy, served as an inspiration for 21<sup>st</sup> Century Cures. They are battling SMA, and are two of the brightest stars I know.

At our inaugural 21<sup>st</sup> Century Cures roundtable last spring I commented, "I think we all agree that we can always be doing more to help biomedical innovation." We've come a long way, but those words still hold true. In fact, since our launch one year ago today, we have heard from our colleagues in the Senate that they are interested in these same goals, and President Obama even included Precision Medicine as part of his State of the Union Address in January. There is clearly an opportunity to make a real difference.

At that first roundtable we asked, "What steps can Congress take to accelerate the discovery-development-delivery cycle in the U.S. to foster innovation, bring new treatments and cures to patients, and keep more jobs in the U.S.?" The bipartisan discussion draft released yesterday, which makes meaningful investments and still will be *fully* paid for, includes a number of policies that seek to answer those questions.

We started this journey because all of us know patients and families who are desperate for hope. We've also seen and read about the incredible advances made in science and technology. But it has become increasingly clear in recent years that our regulatory policies have not kept pace with innovation and there is much more we can be doing to provide that hope to folks. That's what this bill does.

This discussion draft, the product of eight hearings, more than two-dozen roundtables, and several white papers, incorporates the patient perspective into the regulatory process. It will increase funding for NIH. It modernizes clinical trials,

including allowing for more flexible trial designs so we can customize trials based on the unique characteristics of patients most likely to benefit. 21<sup>st</sup> Century Cures will unlock the wealth of health data available so patients, researchers, and innovators can communicate and keep the cycle of cures constantly moving and improving.

We still have important issues to resolve over the next few weeks. One placeholder included in the draft is on rescuing and repurposing drugs for serious and life-threatening diseases and disorders. As we move through the process to markup, we will continue to work on a policy to provide incentives to develop drugs that, while they may have failed in trials for one indication, show promise to treat patients facing other serious or life-threatening diseases. We need to ensure the scientific promise to help patients plays a more important role than patents in drug development. This policy also will include incentives for doing research on drugs that are FDA-approved but can be repurposed to help patients with different types of illnesses.

On the important issue of diagnostics, we remain committed to developing a modernized regulatory framework for these products and services. We look forward to working with Dr. Shuren and stakeholders with hopes of having a legislative hearing by July.

This hearing and the one-year anniversary of 21<sup>st</sup> Century Cures are important milestones, but much more work remains to get this bill to the president. Along with the wealth of ideas and support shared over the last year, we heard repeatedly that patients can no longer wait. We must get this done this year.

I want to thank all of my colleagues who have participated in this effort, thank the patients who have shared their stories with us, as well as the administration officials, staff, and other experts. Yes, we still have work to do, but it is important to recognize the incredible progress of the past year and remain focused on our common goal of helping patients. We have a chance to do something big, and this is our time. And it is Brooke and Brielle's time.