

## New Legislative Initiative to Promote 21<sup>st</sup> Century Cures Marches Towards Congress Following Unanimous Approval by the House Energy and Commerce Committee

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### ABSTRACT

**On May 21, 2015 the House Energy and Commerce Committee voted unanimously (51-0) to pass the 21<sup>st</sup> Century Cures Act. This legislation closely mirrors the mission statement of The Cure Alliance: to end human suffering from chronic, debilitating and fatal diseases by safely and quickly moving potential cures from the laboratory to the bedside. As the bill moves forward to the full House and Senate, we need to maximize the opportunities to create a true transformation. In recent years we have witnessed increasing support of the goals and challenges identified by The Cure Alliance and several patient advocate groups.**

**Let's hope as the 21<sup>st</sup> Century Cures Act moves through Congress, it is only strengthened along the way by leaders who understand that with one out of three Americans living with a deadly or debilitating disease for which there are no cures, in the next five years you or someone you love may be diagnosed with an incurable disease.**

**Keywords:** 21 Century Cures, The Cure Alliance, Faster cures, Translational research, Treatments.

Spontaneous cheers erupted in the second-floor room of the Rayburn Office Building on May 21, 2015 when the House Energy and Commerce Committee voted unanimously (51-0) to pass the 21<sup>st</sup> Century Cures Act. Carefully watching the vote was your Cure Alliance leadership, Shelley Ross and Camillo Ricordi.

This legislation so closely mirrors the mission statement of our organization: to end human suffering from chronic, debilitating and fatal diseases by safely and quickly moving potential cures from the laboratory to the bedside. As the bill moves forward to the full House and Senate, we need to maximize the opportunities to create a true transformation.

Perhaps with the exception of Hep-C, we don't cure diseases anymore. Why not? That's something Chairman Fred Upton (R-MI) and Rep. Diana DeGette (D-CO) explored for a year of listening to those in the trenches of research and the patients we currently fail. Their response to what they learned is *the 21<sup>st</sup> Century Cures Act* which addresses many but not all of the barriers to research.

Most importantly, the bill will free up \$13 billion to modernizing the National Institutes of Health and FDA over the next five years. Ten billion dollars will foster innovation among researchers through the (NIH), a welcome relief to a vital government fixture whose budget has been flat for over a decade and whose grants reach only ten per cent of those who need it.

Three billion dollars will help streamline clinical trials and the drug and medical device approval process at the FDA, setting the stage to unravel decades of red tape. No one is arguing that what began with the noble intent of protecting the public health turned into a mission creep of regulations.

The average cost (\$1.1 Billion) and time (more than a decade) it takes to move a new drug from the laboratory to the bedside is not a sustainable business model for anyone. More concerning, drugs are more likely to be developed to treat a disease or its symptoms than to cure it. The drugs developed to cure Hep-C? Who can afford \$94,500 for a 12-week course of treatment?

### **RADICAL CHANGE IS NECESSARY.**

“We have all said too many early good-byes to people we love and treasure. Every single person has a common goal: we want more time with those we love. In this, the greatest country in the world, Americans deserve a system second to none. We can and must do better. The time for 21<sup>st</sup> Century Cures is now,” Rep. Fred Upton said.

“In the last century, American medicine leapt from medicine shows to the mapping of the human genome,” said DeGette adding, “with the *21<sup>st</sup> Century Cures Act*, we seek to support the biomedical community in making a similar leap forward in this next century. With billions in support for our premier research and development institutions and comprehensive reform of our systems, *21<sup>st</sup> Century Cures* will make a real difference in the lives of patients and their families.”

We certainly hope so. Prior to the vote, we met with committee staffers to discuss what might be tweaked in the version that is submitted for a full vote of the House of Representatives. Dr. Charles Brunicardi joined by videoconference.

One key recommendation *The Cure Alliance* offered is to make sure the group that distributes the NIH funds to innovators contains a “disrupter,” like Uber is to the taxi and Netflix is to cable and broadcast television. Henry Ford was a disrupter who once said, “If you ask people what they want, they’d say ‘a faster horse.’”

Dr. Ricordi said, “All scientists believe they are innovators. They have, indeed, been innovating for years. But NIH will need to find the game changers.”

Dr. Ricordi also warned that identifying the best and brightest won’t necessarily fix the systemic problem that young innovative scientists will still be drawn to areas of research where deep funding from pharmaceutical companies exists. That will not necessarily lead to curing diseases unless clear commitments are made in this direction.

*The Cure Alliance* also recommends the final bill address “blocking patents,” often used as a toll booth for scientific discovery, limiting the “freedom to operate” of potentially breakthrough strategies. Dr. Brunicardi spoke from personal experience of his groundbreaking research into pancreatic cancer running into someone’s broadly-reaching patent. Not only are scientists knocked back, but academic institutions are discouraged from certain research paths to avoid patent litigation.

Dr. Brunicardi also told the committee staff a story of a patient who applied to the FDA for “compassionate” use of a treatment for pancreatic cancer that had success in his lab. After nine months back and forth on behalf of his patient, the FDA gave the green light for treatment. Sadly, the confirmation arrived the day the patient died.

In advance of the *21<sup>st</sup> Century Cures Act* the FDA is already at work streamlining some of their standard operating procedures. The proposed forms for compassionate use will now take 45 minutes to



**Figure 1.** Camillo Ricordi congratulates Rep. Diana DeGette (D-CO) and Chairman Fred Upton (R-MI) after the House Energy and Commerce Committee voted unanimously (51-0) to pass the *21<sup>st</sup> Century Cures Act*.

complete instead of 100 hours. Clearly, the time for change is now.

The *21<sup>st</sup> Century Cures Act, H.R. 6*, will be offered for a full vote of the House of Representatives later this year. The Senate is preparing their version, *Innovation for Healthier Americans*, led by Sen. Lamar Alexander (R-TN) and Sen. Richard Burr (R-NC).

In their report released last January, they wrote: “Together, we can take important steps to ensure that America remains the world’s leading global innovator in medicine, and in the process also ensure that our nation’s patients have access to the most cutting-edge medical products in as timely a manner as possible.”

In recent years we have witnessed increasing support of the goals and challenges identified by The Cure Alliance and several patient advocate groups<sup>1-7</sup>.

Let’s hope as the *21<sup>st</sup> Century Cures Act* moves through Congress, it is only strengthened along the way by leaders who understand that with one out of three Americans living with a deadly or debilitating disease for which there are no cures<sup>8</sup>, in the next five years you or someone you love may be diagnosed with an incurable disease<sup>9</sup>.

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