

H.R.6000 – Cosponsor Cures 2.0 Act

MPS diseases are rare genetic diseases that affect both children and adults. They cause progressive damage to cells in the body, resulting in severe disability and early death. There are few treatments and no cures. But there is hope.

Background:

The 21st Century Cures Act (Cures Act) was signed into law (P.L. 114-255) on December 13, 2016. The law authorized \$6.3 billion in funding, primarily to the National Institutes of Health (NIH) and the Food and Drug Administration (FDA). The Cures Act was designed to encourage development of medical products and devices, and to streamline the process of getting them into the market.

The Cures Act consists of five main sections:

1. Division A: Research and drug development
2. Division B: Behavioral health
3. Division C: Healthcare access and quality improvement
4. Qualified Small Employer Health Reimbursement Arrangement (QSEHR)
5. Electronic health records information blocking

Under Division A, the Cures Act supports several areas that are particularly relevant for the rare disease community. These include streamlining the FDA drug approval process, support for targeted drugs for rare disease, and additional funding for medical research advances in the rare disease space at the NIH.

Cures 2.0:

Introduced by U.S. Reps. Diana DeGette (D-CO) and Fred Upton (R-MI), the Cures 2.0 Act is designed to revolutionize how the U.S. provides care to patients are several provisions aimed at speeding up the delivery of groundbreaking, new – and potentially lifesaving – cures, treatments and innovations to those who need them most. The legislation would also create an entirely new agency aimed at ending some of the world’s most difficult diseases. The Advanced Research Projects Agency for Health, or ARPA-H, would be housed within the National Institutes of Health and tasked with finding new cures and treatments to a slate of illnesses that affect tens of millions of Americans across the country. In addition to establishing a new advanced research agency for health, the legislation would also authorize the full \$6.5 billion the administration has requested to run the agency for the first three years.

The Cures Act 2.0 will:

- Transform how Medicare covers innovative new treatments and technologies to make those new discoveries available to patients sooner.
- Increase access to telehealth services for Medicare and Medicaid patients, including those covered under the Children’s Health Insurance Program, known as CHIP, to ensure more Americans are getting the help they need, when they need it.
- Provide training and educational programs for at-home caregivers – including family members with no prior health care experience to help them better care for loved ones when they are home.
- Require more diversity in clinical trials to ensure any new drugs and treatments approved for use in the U.S. are both safe and effective for a greater – and more representative – portion of the population.
- Provide patients more information about the illness they face and the treatment options available to them to make them a more integral part of the decision-making process.