

June 10, 2014

The Honorable Fred Upton
2183 Rayburn House Office Building
Washington, DC 20515

The Honorable Diana DeGette
2368 Rayburn House Office Building
Washington, DC 20515

Submitted electronically to cures@mail.house.gov

RE: Stakeholder Groups' Response to 2nd White Paper — 21st Century Cures: An Update on the President's Council of Advisors on Science and Technology (PCAST) 2012 Report on Propelling Innovation

Dear Chairman Upton and Representative DeGette:

The undersigned organizations represent health care providers, hospitals, pharmacists, clinical laboratory scientists and medical microbiologists, public health experts, patients and advocates who share a deep concern about the growing threat of antibiotic resistance and the lack of new antibiotics to treat serious or life-threatening infections. Without swift congressional action, we fear that antibiotic research and development (R&D) will continue to struggle, and that patients will continue dying from infections that are resistant to current antibiotics. No one is safe from these infections, but certain populations are at heightened risk, including individuals with weakened immune systems (e.g. chemotherapy patients, transplant patients, the elderly, premature infants, patients with a primary immunodeficiency disease and patients with HIV), soldiers with deep combat wounds, and patients who have had recent surgeries.

We write to thank you for launching the 21st Century Cures Initiative and to offer comments on the initiative's second white paper, *"21st Century Cures: An Update on the President's Council of Advisors on Science and Technology (PCAST) 2012 Report on Propelling Innovation."* The 2012 PCAST report recommends the establishment of a new Food and Drug Administration (FDA) pathway in which sponsors could seek approval of a new drug for use in a limited population of patients with a serious disease and unmet medical need. The PCAST report specifically recommends new antibiotics to treat patients with drug resistant infections as one area for which this new pathway would be appropriate.

We urge the Committee to act promptly on the Antibiotic Development to Advance Patient Treatment (ADAPT) Act, which would carry out the PCAST recommendation to establish a new limited population approval pathway for antibiotics to treat serious or life-threatening infections where an unmet medical need exists. This would allow FDA to consider the risk benefit calculation for patients with few or no other options. Under this bill, the FDA could approve ADAPT drugs based upon smaller clinical trials. It is often not feasible for these drugs to be developed using traditional, large clinical trials due to the limited numbers of patients in whom these infections currently occur.

Importantly, ADAPT drugs must still meet FDA standards of evidence for safety and effectiveness for the limited indicated population. ADAPT's provisions aimed at guiding appropriate use of these drugs include a statement on the label that the "drug is indicated for use in a limited and specific population", but could be strengthened by including a prominent visual element, such as a logo, to make it simple for

the health care community to quickly recognize that these drugs are approved for a limited population and must be used prudently. We believe this issue can be easily resolved as the bill advances.

Quick movement on the ADAPT Act is a logical first step for the 21st Century Cures Initiative. This bill already has over two dozen bipartisan cosponsors on the Committee. The limited population approval approach is supported by a wide array of stakeholders, including medical societies, public health organizations and the pharmaceutical industry. Congress, the FDA, the [Centers for Disease Control and Prevention \(CDC\)](#), the [Director of the National Institute for Allergy and Infectious Disease](#), and the [Trans-Atlantic Task Force on Antimicrobial Resistance \(TATFAR\)](#) have all underscored the urgent need for new antibiotics. With more and more patients dying from multi-drug resistant infections every day, we see no reason for the Committee to delay consideration of this thoughtful, widely supported proposal.

As medical, healthcare, public health and patient organizations dedicated to patient care and safety, as well as public health in general, we urge you to move the ADAPT Act now – without waiting for other legislative initiatives that will be in a part of the 21st Century Cures Initiative – given the public health interests at stake. We look forward to working with you toward the establishment of a limited population approval pathway to speed patient access to new life-saving antibacterial drugs.

Sincerely,

AIDS Action Baltimore, Inc.
Alliance for Aging Research
American College of Rheumatology
American Gastroenterological Association
American Society for Microbiology
American Thoracic Society
Association for Professionals in Infection Control and Epidemiology
Cempra Inc.
Harm Reduction Coalition
HIV Medicine Association
Immune Deficiency Foundation
Infectious Diseases Society of America
National Association of County and City Health Officials
National Association of Pediatric Nurse Practitioners
National Foundation for Infectious Diseases
Pediatric Infectious Diseases Society
Society for Healthcare Epidemiology of America
Society of Critical Care Medicine
Society of Infectious Diseases Pharmacists
The Pew Charitable Trusts
Trust for America's Health
UPMC Center for Health Security