August 2, 2024

The Honorable Diana DeGette
U.S. House of Representatives
Washington, DC 20515

The Honorable Larry Bucshon
U.S. House of Representatives
Washington, DC 20515

Dear Representatives DeGette and Bucshon:

On behalf of Research!America, thank you for the opportunity to provide input to support the development of "Cures 2.1," an initiative building on the landmark, bipartisan, 21st Century Cures Act and Cures 2.0 Act.

Both the 21st Century Cures Act and the Cures 2.0 legislation have addressed urgent needs by challenging the status quo and advocating for better outcomes for patients. It is a privilege to support your efforts in initiating changes that can more rapidly mitigate health threats for more patients.

We applaud your bipartisan approach to "Cures 2.1," the same formula that led to beneficial and actionable proposals for inclusion in 21st Century Cures and Cures 2.0. As we consulted with our alliance members – spanning patient advocacy, academia, independent research institutes, industry, and philanthropy – about the questions posed in your request for input, we sought examples of Cures provisions, such as NIH and FDA funding, that have previously garnered widespread support across our alliance, as well as prominent examples of existing bipartisan legislation and policy themes that could lend themselves to bipartisan action.

We hope the comments below prove useful to you and your respective staff members:

National Institutes of Health (NIH) Resources: We deeply appreciated the inclusion of NIH funding provisions in the 21st Century Cures Act. Drawing from lessons learned since Cures funding was first allocated as part of the FY17 appropriations process and considering the groundbreaking progress achieved since the Act was signed into law, we believe Cures 2.1 can leverage a new NIH funding stream that spurs unprecedented medical and public health progress.

There is enormous opportunity to drive progress through a funding stream that leverages the breadth of research NIH supports to clear hurdles and identify new strategies against threats like cancer, which will take more than 600,000 lives in the U.S. this year, rare diseases, which affect 1 in 10 people in the U.S., and diabetes, which contributes to the deaths of nearly 400,000 people and generates more than $400 million in costs in the U.S. each year. Our nation and the global community are making progress, but we are not making it quickly enough. We have all experienced the suffering or death of a loved one or confronted an illness that can be, but has not yet been, overcome. With determined investment, our nation can shatter the boundaries of science against diseases that needlessly rob people of time, independence, longevity, and hope. Importantly, a bold investment in the health and wellbeing of the American people would also

strengthen our global leadership in science & technology, delivering U.S. business and job growth, bolstering global economic competitiveness, and advancing our national security interests.

Public opinion strongly aligns with greater investment in NIH. Our most recent survey, conducted in the first quarter of 2024, found that a majority of Americans across the political spectrum (75% of Democrats, 54% of Republicans, and 52% of Independents) believe Congress should invest more taxpayer dollars to advance U.S. science and technology. Strikingly, 73% of Democrats, 60% of Republicans, and 56% of Independents would be willing to pay $1 per week more in taxes if they were certain all of the money would be spent on additional medical and health research. Further, across the political spectrum, a majority of Americans (63% of Democrats, 59% of Republicans, and 55% of Independents) believe it is very important that the U.S. is a global leader in research to improve health.

To maximize its beneficial impact, additional resources for NIH should be accorded a top priority for Cures 2.1.

Food and Drug Administration (FDA) Resources: The 21st Century Cures Act provided additional funding for the FDA in recognition of the additional responsibilities the agency was directed to fulfill under the legislation. We perceive supplemental funding as fundamentally important to realizing FDA-related objectives for accelerated progress.

Agency for Healthcare Research and Quality (AHRQ) Resources: Our nation undervalues health services research at a tragic cost to patients and a staggering cost to our economy. The implementation of just one AHRQ-funded study on reducing hospital acquired conditions prevented an estimated 20,500 hospital deaths and saved $7.7 billion in health care costs from 2014 to 2017. Imagine if AHRQ had the support of Congress, including the resources, to combat the estimated $1 trillion annually spent on suboptimal care and its consequences? It is not just time to elevate the priority of research-driven medical progress, but research-driven health care progress. The health, wellbeing, and very lives of Americans, and fiscal accountability to taxpayers, hang in the balance. We ask that you consider providing new funding for AHRQ in Cures 2.1 and would welcome the opportunity to help further develop this idea.

Pending Legislation

1. Biosecurity: The Cures 2.0 Act laid down a crucial marker for our nation by introducing important and timely policy proposals in response to the COVID-19 pandemic. That same resolve to spur timely, biosecurity-essential action is needed now to secure reauthorization of the Pandemic and All Hazards Preparedness Act (PAHPA). We urge you, as part of your efforts to advance the objective of the Cures movement, to champion reauthorization of PAHPA as soon as possible this year.

2. Antimicrobial Resistance (AMR): The threat AMR poses in the U.S. and around the globe is staggering. According to the CDC, more than 2.8 million antimicrobial-resistant infections occur each year in the U.S. and more than 35,000 people die as a result.

Globally in 2019, AMR took the lives of 1.2 million people and was associated with the deaths of 5 million people. We firmly believe Congress would be acting in the best interests of the American people by passing bipartisan, bicameral legislation – the PASTEUR Act and the SUPER BUGS Act – as soon as possible this year. Together these bills will advance research & development (R&D), surveillance, and international partnerships to strengthen the U.S. and global response to AMR.

3. Clinical Trial Participation: We applaud the emphasis previous Cures legislation has placed on promoting clinical trials participation and diversity. Challenges in both arenas impede and narrow the scope of medical progress. We urge your consideration of HR 8412 (the Clinical Trials Modernization Act) and HR 7418. Both bills are intended to advance clinical trials participation and diversity by addressing financial hurdles that disincentivize and create disparities in the ability of patients and healthy volunteers to join trials.

4. Bolstering Progress Against Rare Pediatric Diseases: The bipartisan Creating Hope Reauthorization Act of 2024 (H.R.7384) would reauthorize the Rare Pediatric Disease Priority Review Voucher program, which is set to expire on September 30, 2024. The National Organization for Rare Disorders has put together a primer documenting the important role the voucher has played in accelerating progress against these diseases. We hope you will help champion passage of this important bill as a time-sensitive legislative priority.

Policy Themes

We hope the following themes, which arose prominently during discussions with alliance members about the Cures 2.1 effort, prove useful as you consider next steps. Inclusion of a theme in these comments does not constitute an endorsement of, or opposition to, any pending or previous legislation.

Regulatory Harmonization between the FDA and the Centers for Medicare and Medicaid Services (CMS)

There are important opportunities to strengthen regulatory harmonization between the FDA and CMS, which can create time and cost-saving efficiencies in the discovery, development, and delivery pipeline. In January, the Bipartisan Policy Center issued a report and recommendations centered on FDA/CMS harmonization.

The faster affordable, safe, and effective medical interventions reach patients, the more individuals can benefit, thereby maximizing the overall societal impact of U.S. research and development efforts.

When pain, disability, and even death hang in the balance, it is strongly in the public interest to eliminate any unjustifiable delays or discordance in FDA and CMS regulatory activities. The Department of Health and Human Services (HHS) should actively seek out, acknowledge, and

promptly address any evidence of weaknesses in communication or coordination; poorly delineated roles and responsibilities; incongruent perspectives or priorities; or any other challenge that squanders time, increases costs, creates false hope for patients, or selectively disadvantage certain patients.

When there are significant disconnects between FDA and CMS processes and decision-making, patients pay an unacceptable price. Two examples that alliance members have raised with us:

- Decisions by federal and private insurers to deny coverage for products because of the regulatory pathway FDA has used to evaluate those products.
- Timeframes for CMS coverage that surpass any time saved through expedited FDA review of drugs or devices.

Regardless of how these disconnects are resolved, they should be resolved. Whether federal actions are creating false hope or accelerating product availability and then denying access to those who are the most economically vulnerable, patients shouldn't be treated as an afterthought in FDA and CMS processes and decision-making.

Accelerating Progress Against Neglected Tropical Diseases (NTDs)

NTDs encompass a range of over 20 chronic, disabling, disfiguring, and deadly conditions resulting from parasitic, viral, bacterial, and fungal infections. Historically predominant in developing nations, the presence of NTDs in the U.S. is rising, but a lack of regular surveillance limits our ability to respond to and eliminate these threats. Recent studies suggest that up to 12 million Americans live with at least one NTD. The Gulf Coast states of Texas, Louisiana, Mississippi, Alabama, and Florida are particularly vulnerable to NTDs due to their climate, where insects that transmit these diseases thrive, and high rates of poverty. NTDs are intimately linked to poverty, spreading readily in areas that lack clean water and proper sanitation. Recent estimates posit that 3–4 million Gulf Coast residents are affected by at least 1 NTD.

Earlier this year, as part of Research!America's commitment to advocate for research that benefits people in the U.S. and across the globe, we surveyed Research!America alliance members about existing and potential incentives to encourage more private sector R&D in the area of NTDs. We spoke with individuals from industry, academia, and the global health community. FDA's Tropical Disease Priority Review Voucher Program (TD-PRV) and FDA pilot programs like Project ORBIS featured prominently in these interviews. We believe both of these areas of opportunity merit consideration as Cures 2.1 legislation is developed.

a. TD-PRV: Respondents raised concerns about the process for determining whether products are eligible for a voucher – they described an opaque process with unpredictable timelines that are inexplicably long.

   Similar concerns were raised about the process for adding diseases to the list of TD-PRV-eligible diseases and conditions. Respondents told us the review board process is also opaque, infrequent, and inexplicably drawn out. Of particular concern: FDA appears to

have the authority to deem all diseases on the World Health Organization's list of NTDs eligible for the TD-PRV program, but the agency has not chosen to do so.

Potential TD-PRV reforms shared with us include directing FDA to:

- Harmonize the TD-PRV Program with the WHO's list of NTDs.
- Establish clear and expedited TD-PRV pathways that ensure timely, transparent decision-making on TD-PRV eligibility and inclusion of new diseases in the program.
- Improve efficiency and transparency in TD-PRV processing by implementing clear guidelines and milestones like those in the Prescription Drug User Fee Act (PDUFA) process.
- Enhance public accessibility of TD-PRV data through the FDA Data Dashboard.
- Address barriers to TD-PRV eligibility by amending current law to align TD-PRV eligibility criteria with the Orphan Drug Act's 'no reasonable expectation of cost recovery in the United States' criterion.

b. Create a "Project ORBIS" for NTDs: The FDA Oncology Center of Excellence, authorized under the 21st Century CURES Act in 2016 and launched one year later, works on multiple fronts to spur the development and expedited regulatory review of medical products for oncologic and hematologic malignancies. One of the Centers' many accomplishments is the establishment of Project ORBIS, which works to harmonize regulatory review of these products internationally. The survey respondents emphasized the value of regulatory harmonization in NTDs. Coordinating and to the extent possible standardizing regulatory policies across nations would facilitate market access globally, serving as a robust incentive for the development of products for NTDs that increasingly impact populations in the U.S. and pose a staggering burden globally.

Bipartisan, Bicameral Review of Inflation Reduction Act (IRA) Price Negotiation Provisions

Several members believe Cures 2.1 should set in motion a bipartisan, bicameral effort to assess the current and projected impact of the Inflation Reduction Act (IRA), inclusive of such variables as patient equity, indirect fiscal impact, and scientific discovery.

Previous assessments have focused on important variables like the impact on Medicare beneficiary cost-sharing and the direct fiscal impact of the law, but concerns have been raised about unintended inequities across patient populations, unaccounted-for effects on the direction and pace of scientific discovery and its translation, the indirect fiscal impact of disincentivizing small molecule drugs, and changes in the volume and focus of R&D that may directly contradict previous congressional actions intended to address unmet medical needs.

Examples of questions that alliance members have raised:

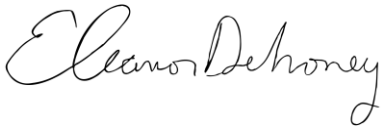
- What are the impacts on patients, scientific progress, and Medicare costs of applying different negotiation timelines to small molecule drugs than those applied to biologics?

- Are Orphan Drug Act incentives contravened by IRA's treatment of orphan drugs?
- How are changes in the volume and flow of R&D investment weighed against cost and fiscal considerations in evaluating the net impact of the law?

A dispassionate evaluation of the IRA's impact that takes additional variables into account should be structured to forge common ground, advance the best interests of Medicare beneficiaries and all healthcare consumers, and provide useful information to policymakers, researchers, and members of the public regardless of their previous support for or opposition to the price negotiation provisions of the law.

Again, Representatives DeGette and Bucshon, we hope these comments, which are representative rather than exhaustive, prove useful as you determine next steps on Cures 2.1. It would be a privilege to continue engaging with you and your respective staff members as this important initiative moves forward. We are deeply grateful for your determination to push past unjustifiable impediments to, and seize new opportunities for, enduringly beneficial scientific, medical, and public health progress.

Sincerely,



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