NATIONAL HEALTH COUNCIL

August 2, 2024

The Honorable Diana DeGette
United States House of Representatives
Washington, DC 20515

The Honorable Larry Bucshon, M.D.
United States House of Representatives
Washington, DC 20515

Dear Representatives DeGette and Bucshon:

The National Health Council (NHC) is pleased to respond to your request for information (RFI) on the future of 21st Century Cures 2.0 legislation.

Created by and for patient organizations more than 100 years ago, the NHC brings diverse organizations together to forge consensus and drive patient-centered health policy. We promote increased access to affordable, high-value, equitable, and sustainable health care. Made up of 170 national health-related organizations and businesses, the NHC's core membership includes the nation's leading patient organizations. Other members include health-related associations and nonprofit organizations including the provider, research, and family caregiver communities; and businesses and organizations representing biopharmaceuticals, devices, diagnostics, generics, and payers.

The NHC was an engaged partner during the crafting of the 21st Century Cures Act and appreciated its passage and its lasting impact on bringing new treatments and therapies to patients. We also share your goal of "shaping a more dynamic, patient-centered heath care system, driving research, and improving treatment options" through Cures 2.0. This initiative could build on the tremendous successes of the 21st Century Cures Act, help us prepare for future pandemics and other health disruptions, and lead to the development of and access to new drugs, devices, and other interventions. We are grateful for your continued leadership on these crucial issues.

Regarding your first question about whether the policies included in Cures 2.0 that have advanced through legislation or executive action meet the needs that the original Cures 2.0 bill aimed to address, the NHC offers the following. The NHC supports the important advancements noted in the RFI that have been made on issues such as:

- Establishing the Advanced Research Projects Agency for Health (ARPA-H)
- Increased guidance from the from the FDA on critical issues such as:
    - Cell and gene therapy
    - Expedited drug approval processes
    - integration of real-world evidence in regulatory decision-making
    - Coverage for breakthrough medical devices
    - Establishing strategies for testing and response mechanisms for future public health emergencies
    - Addressing long Covid

However, there is still a need to codify these guidances and, in the case of ARPA-H, expand on progress that has been made. We urge you to continue to address these important issues in Cures 2.0 and consider codifying progress that has been made through regulation or executive order.

The RFI focuses on which aspects of the 2022-introduced bill are still relevant and what additions or changes might need to be made. The specific comments below are focused on those questions and reflect much of our previous input into earlier iterations of Cures 2.0. At a high level, the NHC believes the issues addressed in Cures 2.0 are still relevant and needed.

Title I: Public Health

As the last few years have taught us, our public health system has been desperately underfunded for decades and would benefit from increased investment and improvement. The proposals in Title I take significant steps to increase our ability to respond to the next pandemic and address the ongoing impact of COVID-19.

The NHC supports the survey on sources of coverage and learning collaborative on long-COVID proposed is Section 101. These first steps to addressing what will be a long-term challenge in understanding and treating long-COVID are necessary. We appreciate the attention to the issue in this bill. In the learning collaborative section, we recommend patients themselves be required to be a part of the collaborative along with groups representing patients.

We appreciate that Section 103 identifies the importance of a plan for addressing the needs of patients with rare diseases in public health emergencies. COVID-19 was especially difficult for people with chronic diseases and disabilities. Many underlying conditions exacerbated COVID-19 infections, leading to serious disease and death, and many people struggled to manage their chronic conditions through continued care while putting themselves at risk of contracting the virus. While we support this provision, we recommend that this plan be broadened to include preparedness for all people with chronic diseases and disabilities.

Section 105 included provisions from the Pioneering Antimicrobial Subscriptions to End Up surging Resistance (PASTEUR) Act. We believe addressing growing antimicrobial resistance is an important inclusion. People with chronic conditions and disabilities are more likely to suffer the effects of antimicrobial resistance as they are more frequently in hospitals and more likely to have severe consequences from resistance. Therefore, we appreciate the focus on this issue.

One area we believe is missing from the current legislation is emphasis on disease prevention. While this section is understandably focused on pandemic preparedness, one of the greatest reasons the United States saw a disproportionate impact of the

pandemic compared to other nations is our high prevalence of chronic disease and behavioral health that led to worse COVID-19 outcomes. We recommend adding a section to this bill that calls for greater investment in – and coordination of - federal programs focused on chronic disease and behavioral health prevention activities.

Title II: Patients and Caregivers

The NHC greatly appreciates this Title's focus on building on the 21st Century Cures Act's, the FDA Safety and Innovation Act's, and the FDA Reauthorization Act's priorities on elevating the voice of the patient in medical product development and regulation. Similarly, the NHC is pleased to see your recognition of the role of caregivers in helping their friends and family members manage their chronic conditions.

Section 201, which includes training for caregivers to help them be a part of the care team, is critical. Although progress on this topic has been made through regulations, it is important to codify this caregiver support. Too often, caregivers must "learn on the job" and are not given the training they need to meet the needs of their loved ones. Very often, they are asked to provide medical tasks that may be beyond their abilities or comfort. Such training activities must be made available, but guardrails need to be put in place to assess caregiver burden and help the caregiver and care recipient feel comfortable with what the care team is asking of them.

Health literacy is another vital issue. The NHC particularly appreciates Section 202's inclusion of increasing health literacy around the specifics of insurance coverage. One addition that would be helpful is a review of how the health care system can better communicate important health information in a way that is approachable for patients and improve how information is delivered in a helpful way. Too often, the onus is on the patient to learn about the health system instead of getting health information that is usable and relevant to them.

The proposals in Section 203 are a good first step to increasing diversity in clinical trials. The legislative requirement for diversity action plans in clinical trials and the recent FDA draft guidance on this topic mark important progress in diversifying clinical trials. However, Cures 2.0 includes additional actions to help advance diversity in clinical trials. The NHC particularly appreciates the efforts to make clinicaltrials.gov more user-friendly and the bill's requirement that patient advocates serve on the proposed task force. We also recommend inclusion of an array of providers in the task force because they are a primary connector to clinicaltrials.gov. In addition, the NHC believes further steps are needed to improve the diversity of clinical trials. Specific ideas include having FDA provide guidance on engaging people of color in decentralized trials, steps to reduce burden of participation, and engaging with community partners like community health centers and other providers in marginalized communities to engage diverse communities in trials.

Section 204 of Cures 2.0 that requires submission of standardized patient experience data will help create consistency in how FDA reflects patient input. The NHC has a long history of helping provide guidance on collecting patient experience data (PED). A standardized format for collecting PED data would provide clarity for sponsors on what would be submitted and be more understandable for the general public. There needs to be more significant guidance on what is collected in the standardized data set to assure it is comprehensive and effective for all patients. When regulations are issued, the FDA needs to clarify what "standardized" will mean. The NHC requests comprehensiveness and comparability, but there cannot be one core set of data that will fit all situations.

Finally, the NHC applauds the proposal in Section 205 to allow for Medicare coverage for the cost of participating in Patient-Centered Outcomes Research Institute (PCORI)-funded clinical trials in alignment with clinical trials that are funded in other ways.

Title III: Food and Drug Administration

In Section 304, you propose increasing the use of real-world evidence. The NHC appreciates this focus on real-world evidence and encourages you to include provisions to help patients and their representatives actively participate in developing quality real-world evidence. The NHC has developed resources to do just that as part of our real-world evidence classroom. One way to achieve this goal is to have a seat for patient organizations on the task force proposed in the current legislation. We feel it is important that patients be represented at this table to provide their unique perspective as it relates to the development, dissemination, and use of real-world evidence and real-world data.

The NHC supports the intent of the improved FDA-CMS communications proposed in Section 305. The FDA providing CMS earlier information could speed up access to newly approved therapies by supporting quicker coverage decisions. The NHC also supports the language in Section 305(b) that confirms that the FDA and CMS would retain their independence in making decisions about approval and coverage, respectively.

The NHC understands the importance of getting treatments to patients as quickly as possible. The accelerated approval process has been an important tool in speeding access for patients. However, we must be diligent in balancing timely approvals with the important patient safeguards that the FDA oversees. If accelerated approval processes are made easier to access through proposals such as the one in Section 309, the NHC requests there be a thorough review about how that process will be overseen to encourage earlier approvals while appropriately encouraging timely post-market studies that better help us understand the efficacy of products that are approved through this pathway.

Title IV: Centers for Medicare and Medicaid Services (CMS)

The first few Sections of this Title address access to innovative health technologies and telehealth. The last few years have shown increased access to telehealth is welcomed by patients and increases access to care for many who could not access it before. Lessons were learned from the increased flexibility provided during the current pandemic and those flexibilities that have worked should be continued. Section 402 and Section 403 do that, and the NHC supports those efforts. There are a number of legislative proposals attempting to address real barriers to accessing telehealth, and the NHC welcomes continued conversation on the topic and hopes to see meaningful legislation passed this year.

The NHC has supported efforts to create a pathway to accelerate the coverage of new and innovative devices. Section 404 of the bill includes a Medicare Coverage of Innovative Technology (MCIT) pathway that the NHC has supported. Since the introduction of the legislation, CMS has issued proposed guidance on a Transitional Coverage for Emerging Technologies (TCET), which the NHC also supports. However, the TCET guidance has not been finalized. In addition, the NHC has endorsed legislation, H.R. 1691 - Ensuring Patient Access to Critical Breakthrough Products Act of 2023 and encourages the alignment of the approach that legislation takes into Cures 2.0 if that bill is not passed this year. Patients will benefit from breakthrough devices if a pathway exists to promote faster coverage and access to devices that can support their health and independence.

Access to genetic testing is critical for so many people with chronic diseases and disabilities, especially pediatric patients with rare diseases and their parents who rely on it to receive diagnoses and make treatment decisions. The NHC appreciates the approach that has been developed in Section 407and supports continued work to increase access to genetic testing.

Additional Issue – Step Therapy

While the United States has led the way in fostering the development of innovative treatments, many in the nation are unable to access these new treatments due to utilization management (UM) policies such as prior authorization and step therapy. The NHC knows that UM protocols should be grounded in sound clinical decision-making. However, the development of such protocols is typically done without much or any patient input, and the rationale for such decisions is not always public or accessible to patients. This often results in UM serving as one of the most persistent barriers to care that patients face. A survey of the NHC membership identified addressing concerns about the overuse of UM as their top policy concern for the coming year.

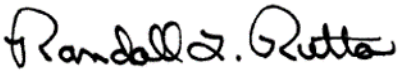
Part of the solution is passing the bipartisan Safe Step Act (S. 632/H.R. 2630), which would ensure that employer health plans offer an expedient and medically reasonable

step therapy exceptions process. Step therapy is a complex form of UM that requires patients to try and fail on certain treatments before the plan covers other treatments, including those initially selected by the patient and their provider. When inappropriately used, step therapy is particularly egregious, as it can delay needed care for extended periods and lead to negative health outcomes for patients.

Conclusion

The NHC appreciates the opportunity to provide additional input on these critical issues. Please do not hesitate to contact Eric Gascho, Senior Vice President of Policy and Government Affairs if you or your staff would like to discuss these issues in greater detail. He is reachable by phone at 202-973-0545 or via e-mail at egascho@nhcouncil.org.

Sincerely,



Randall L. Rutta
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