NATIONAL HEALTH COUNCIL

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August 25, 2021

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

The Honorable Fred Upton
U.S. House of Representatives
Washington, DC 20515

Dear Representatives DeGette and Upton:

The National Health Council (NHC) is pleased to respond to the discussion draft of 21st Century Cures 2.0 legislation. Created by and for patient organizations 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, sustainable health care. Made up of more than 140 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 representing biopharmaceutical, device, diagnostic, generic drug, and payer organizations.

The NHC greatly appreciated your leadership and partnership on the 21st Century Cures Act and its lasting impact on bringing new treatments and therapies to fruition and shares your desire to "improve how those new treatments and therapies are delivered to patients" through Cures 2.0. This important proposal will 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.

The following is a title-by-title response to the discussion draft.

Title I: Public Health

As the last year has taught us, our public health system has been desperately underfunded for decades and would benefit greatly 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 the current one.

The NHC supports the survey on sources of coverage and learning collaborative on long-COVID proposed in Section 101. These are necessary first steps to addressing what will be a long-term challenge in understanding and treating long-COVID, and we appreciate the attention to the issue in this bill. In the learning collaborative section, we recommend it be required that patients be engaged as a part of the collaborative along with groups representing patients. It has been demonstrated (i.e., the work of the Patient-Centered Outcomes Research Institute) that patient engagement improved the research itself and the usability of the research findings. We also recommend that work on long-COVID not be limited to those diagnosed with COVID or post-COVID syndrome but also include efforts to address other post-viral illnesses and un/under diagnosed illnesses like myalgic encephalomyelitis/chronic fatigue syndrome.

We appreciate that Section 103 identifies the importance of a plan for addressing the needs of patients with rare diseases during public health emergencies. COVID-19 was especially difficult for people with chronic diseases and disabilities. Many underlying conditions exacerbated COVID-19 infection, leading to series disease and death, and many people struggled to continue ongoing care to manage their chronic condition 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, not just those with rare conditions.

We also appreciate that Section 104 identifies the importance of educating all Americans on the importance of vaccines. During a time when we see an increase in the amount of misinformation, it is critical that we continue to add more resources for vaccine awareness campaigns. We have seen drastic decreases in childhood immunization rates this past year and delays in accessing care. This campaign work will be integral to preventing future outbreaks of preventable diseases.

In Section 105, you include 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 diseases 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.

Additional Issue – Chronic Disease Prevention

One area we believe is missing from the discussion draft is some emphasis on chronic disease prevention. While this section is understandably focused on pandemic preparedness, one of the greatest reasons the United States saw a disproportionate impact due to the pandemic compared to other nations is our high prevalence of chronic disease that led to worse outcomes caused by COVID-19. We recommend adding a section to this bill that calls for greater investment in – and coordination of – federal programs focused on chronic-disease prevention activities.

Title II Patients and Caregivers

We greatly appreciate 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 focus on elevating the voice of the patient in medical product development and regulation. Similarly, we are pleased to see your recognition of the role of caregivers in helping their family members and friends manage their chronic conditions.

Section 201, which includes training for caregivers to help them be a part of the care team, is critical. Too often, caregivers must "learn on the job" and are not given the training they need to meet the needs of their loved ones. However, we must be careful that we are asking appropriate

tasks of caregivers. Very often, they are asked to provide medical care that may be beyond their abilities or comfort level. Such training activities must be made available, but we also must put guardrails 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 an understandable and helpful way. Too often, the onus is on the patient to learn about the health system instead of receiving health information that is usable and relevant to them. Patients should be engaged to help to co-develop materials to ensure these goals are achieved.

The proposals in Section 203 are a good first step to increasing diversity in clinical trials. We particularly appreciate 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 and other marginalized populations 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.

The NHC has a long history of helping provide guidance on collecting patient experience data (PED). A standardized format for collecting PED would provide clarity for sponsors and others on what should be submitted and be more understandable for the public. However, we believe there needs to be more significant guidance on the development of standardized methods and approaches to assure comprehensive, sound data are captured and used optimally. When regulations are issued, FDA needs to clarify what "standardized" will mean and how the standards will be derived. This work should align the FDA, Patient-Centered Outcomes Research Institute (PCORI), and the Centers for Medicare and Medicaid Services' (CMS) and National Quality Forum's (NQF) quality measurement sets and systems, around the same standard set of impacts patients care about.

But as we strive for comprehensiveness and comparability, there cannot be one core set of data elements that will fit all situations. Standardized sets and the tools to support their creation are nascent. For this reason, the NHC has a current initiative to articulate methods for developing patient-centered core impact sets (PC-CIS). For a specific disease or population, the core impact set includes those impacts that a disease and treatment have on a patient's and family's life that patients and families report as are most important to them based on their experiences (PED). The core set is the starting point for selecting measures and endpoints for trials and other uses, but, these standardized core impact sets do not yet exist.

We strongly recommend a systematic process for their development and avoidance of haphazard, uncoordinated efforts. The Cures 2.0 legislation is an opportunity to spearhead an effort to create the standardized methods and mechanisms for standardized PED collection in order to derive the patient-centered core impact sets needed. This could be done by information and support from FDA through guidance.

Finally, we applaud the proposal in Section 205 to allow for Medicare coverage for the cost of participating in 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. We appreciate this focus on real-world evidence and encourage you to build in provisions to help patients and their representatives actively participate in developing and contributing to high-quality real-world evidence. Patient groups, through the registries and survey data they collect, are important participants in this discussion. 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 at least one seat reserved for patient organizations on the task force proposed in the discussion draft. We believe it is important 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 improved FDA-CMS communications proposed in Section 305 will significantly help get innovative therapies to patients as quickly as possible. Coverage decisions must be connected as early as possible to review and approve new therapies. However, we encourage clarifying language that better delineates the unique role that the FDA plays in approving products and that CMS plays in ensuring appropriate coverage and reimbursement of them.

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 patient access. 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, we ask that there be a thorough review about how that process will be overseen to encourage faster approvals while appropriately encouraging timely post-market studies that better help us understand the efficacy and long-term safety 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 year has shown increased access to telehealth is welcomed by patients and enhances access to care for many who could not access it before. We need to take the lessons from the increased flexibility provided during the current pandemic and continue those flexibilities that have worked. Section 402 and Section 403 do that, and we support those efforts. We know there are many legislative proposals attempting to address real barriers to accessing telehealth. We welcome continued conversation on the topic and hope to see meaningful legislation passed this year.

We have supported CMS efforts to create a Medicare Coverage of Innovative Technology (MCIT) pathway to accelerate the coverage of new, innovative breakthrough devices for Medicare beneficiaries. Patients will benefit from breakthrough devices if the pathway exists to promote faster coverage and access to devices that can support their health and independence. We support Section 404's codification of CMS' proposed pathway. One recommended addition is to incorporate a mechanism for CMS to consider the patient perspective in determining which technologies would be eligible for this pathway so that technologies that are most valued by patients are prioritized for coverage. We also urge CMS to include complex rehabilitation technology, assistive technology, and devices, as well as technology that increases the accessibility and effectiveness of telehealth in these efforts to advance coverage for innovative technologies.

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. We appreciate the approach that has been developed in Section 407, particularly the broad eligibility of DNA clinical sequencing services to allow the most appropriate tests to be ordered and covered as decided by the clinician. We support continued work to increase access to genetic testing.

In addition to the provisions referenced above, the NHC recommends additional provisions that would direct CMS to better incorporate the patient perspective in their coverage and reimbursement decisions.

Title V: Research

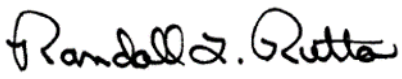
As mentioned above, our comments on the Advanced Research Projects Agency for Health (ARPA-H) are included under a different cover in direct response to the request for information (RFI). We appreciate the effort to create an authorizing structure for this important new endeavor.

Finally, in section 502, there is a significant new investment in research in Federally backed venues such as academic institutions and other public labs. While we appreciate the inclusion of nonprofit Federally funded researchers as eligible recipients of these funds, we know there is significant non-Federally funded research by nonprofits, particularly in patient group-supported laboratories. Therefore, we recommend a GAO study that quantifies the impact of nonprofit-funded research that has been stopped due to the pandemic and includes recommendations for relief.

Conclusion

We appreciate the opportunity to provide additional input on these critical issues. In addition, we have responded to your Request for Information (RFI) on creating the Advanced Research Projects Agency for Health (ARPA-H) under a separate cover. Please do not hesitate to contact Eric Gascho, 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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