May 12, 2020

The Honorable Diana DeGette  
United States House of Representatives  
House Energy and Commerce Committee  
Washington, DC 20515

The Honorable Fred Upton  
United States House of Representatives  
House Energy and Commerce Committee  
2183 Rayburn HOB  
Washington, DC 20515

Dear Representatives DeGette and Upton,

On behalf of the Cancer Support Community (CSC), an international nonprofit organization that provides support, education, and hope to people impacted by cancer, we wish to express our thanks and support of your thoughtful and timely 21st Century Cures 2.0 concept paper. The challenges identified and policy solutions proposed will help to both fill existing gaps that have deprived some patients the opportunity to benefit from scientific advances and ensure that, moving forward, all patients will play an active and integral role in ushering in the new medical innovations intended to benefit them.

We are most encouraged by your recognition of the promise of collecting meaningful Real-Word Data (RWD) and Real-World Evidence (RWE) in research and are eager to work with you to ensure that the intentions behind your stated objectives are fully realized.

Title III – Patient Engagement in Health Care Decision-Making

Increasing Health Literacy to Promote Better Outcomes for Patients:

Title III of the concept paper begins with a sentence that encompasses both the stated challenge and its solution, namely that improving health literacy and access to health information will improve outcomes. It is very difficult, if not impossible, to ask meaningful questions or seek meaningful answers of patients if they do not understand the language or have access to knowledge. Requiring CMS to issue an RFI regarding ways the agency can work with federally subsidized health care program stakeholders to encourage and promote greater health literacy of individuals will provide meaningful insights into filling knowledge gaps and barriers so that all patients have the opportunity to achieve their optimum health. We applaud Title III for not only addressing “what” (i.e. health literacy and access to information) will improve outcomes, but also answering the “why” which is often lost in the health literacy conversation. Without question, patients who understand their diseases or conditions, will be empowered to become part of the decision-making process and take steps to get well.

Title IV – Clinical Trials

Diversity in Clinical Trials:

CSC recognizes the challenge and need of achieving clinical trial diversity called for in Title IV of the concept paper. Clinical trials provide patients with life threatening conditions the opportunity to participate in ground-breaking research that may bring about treatments that improve quality of life,
extend survival, and even prove lifesaving. Nowhere is the benefit of diversity, including across income, sex, age, race and ethnicity, more pronounced than in the field of medical innovation. As so aptly noted in the concept paper, diversity in clinical trials is essential to ensuring medical products are safer and more effective for patients.

CSC supports all four proposed measures to increase diversity in clinical trials: ensuring Medicaid covers routine care costs of clinical trial participation for enrollees with life-threatening conditions; requiring FDA to provide an update on implementation of the items included in the FDASIA Action Plan; requiring HHS to increase awareness and understanding of clinical trials; and requiring HHS to convene a task force on making clinicaltrials.gov more user and patient friendly. These policies will tear down prior barriers that stood in the way of all patients benefiting from medical innovation and elevate the voice of a representative diverse population of patients in the drug development process.

Title V – FDA
Increasing Use of Real-World Data/Evidence:
The discussion of data and optimizing its utilization and analysis in the development of new cures, as presented in Title V of the Cures 2.0 concept paper, is an area of particular focus for CSC and one in which we hope you will consider engaging in additional discussions with us during the transition from concept paper to legislative language.

We appreciate that fostering the development and utilization of this data was a major priority of 21st Century Cures. Because RWD and RWE is typically derived from limited sources such as claims and billing data, and, therefore, offers limited insight and benefit to the development of new cures, we ask that whenever policies in Cures 2.0 require the collection and utilization of RWD and/or RWE, that the breadth, depth, and source of the data required to be collected and utilized be modernized to also encompass patient experience data (PED). With the passage of the 21st Century Cures Act in 2016 and the Food and Drug Administration Reauthorization Act (FDARA) in 2017, Congress heightened the importance of collecting PED that not only includes the physical impacts of a condition, therapy, or clinical investigation/trial but also the psychosocial impacts. Patient experience data can be interpreted as information that captures patients’ experiences, perspectives, needs, and priorities related to (but not limited to): 1) the symptoms of their conditions and its natural history; 2) the impact of the conditions on their functioning and quality of life; 3) their experience with treatments; 4) input on which outcomes are important to them; 5) patient preferences for outcomes and treatments; and 6) the relative importance of any issues as defined by patients. It is important to note the distinction between patient reported outcomes (PRO’s) and patient experience data – with PRO’s being one of four subcategories within Clinical Outcome Assessments (COA’s) – and COA’s representing just one of six sources of PED. While still of value, PRO’s provide limited outcome measures that are based on a report coming directly from the patient about the status of his or her health condition without amendment or interpretation of their response by a clinician or anyone else. CSC recognizes that while RWD, RWE, and PRO’s all offer useful information, they are only pieces of the larger patient experience data puzzle.

In addition, we ask that any requirement made of the Secretary of Health and Human Services (or any other federal agency) to establish a consistent and clear regulatory framework for the recognition and utilization of RWE also include a corresponding requirement for the utilization of patient experience data, and that PED be incorporated into the two goals outlined in this policy.

Finally, CSC believes it is essential for patients to remain at the center of the drug development process beginning with clinical trial design and proceeding through post-market surveillance. Recognizing that patients are the foremost experts in their disease, it is patient experience data that holds the promise of greater insights that can fuel the development of new cures. We strongly support a requirement for the establishment of a task force comprised of patient groups, CMS, FDA and private sector representative.
We urge the task force to be directed to develop a list of recommendations on methods to collect and utilize patient experience data throughout the entirety of the drug development cycle as well as develop recommendations on ways to encourage patients to engage in the generation of real-world data. Once comprised, we would ask the recommendations related to the screening, identification, reporting, consideration, and labeling of PED in clinical trials of a drug or biological product be presented to Congress to ensure that patients remain at the center of the drug development process as intended in 21st Century Cures and to address the challenges and achieve the solutions ultimately presented in the Cures 2.0. As the comprehensive care conversation evolves and becomes more holistic of the patient, we must expand our assessment of patients to include more than disease symptoms, treatment side effects, and physical functioning. It is incumbent upon the FDA, CMS, industry, academic institutions, members of the health care team, patient advocacy organizations, and other stakeholders to consistently and meaningfully seek robust and informative patient feedback and patient experience data at all points along the research and care continuum.

Conclusion
We appreciate the Cures 2.0 concept paper identifying both challenges and policy solutions that will advance medical research and make resulting new cures more accessible to a diverse patient population. We hope our perspective on the issues above are beneficial as you move forward in the legislative process. We would like to continue the discussion and will follow up to schedule a telephone call. As you proceed in the drafting of Cures 2.0 legislation, we encourage you to keep the perspectives of patients at the forefront of your thoughts and hope that you will not hesitate to contact the CSC as we have a large body of data and frontline perspectives from patients that would be beneficial to you.

Sincerely,

Kim M. Czubaruk, Esq.
Senior Director, Policy and Advocacy
Cancer Support Community