October 11, 2017

Mark B. McClellan, MD, PhD
Director
Duke University Margolis Center for Health Policy
1201 Pennsylvania Avenue 5th Floor
Washington, DC 20004

Dear Dr. McClellan

On behalf of the Cancer Support Community (CSC), an international nonprofit organization that provides support, education, and hope to cancer patients, survivors, and their loved ones, we appreciate the opportunity to respond to the request for comments regarding the “Framework for Regulatory Use of Real-World Evidence.”

As the largest direct provider of social and emotional support services for people impacted by cancer, and the largest nonprofit employer of psychosocial oncology professionals in the United States, CSC has a unique understanding of the cancer patient experience. Each year, CSC serves more than one million people affected by cancer through its network of over 40 licensed affiliates, more than 120 satellite locations, and a dynamic online community of individuals receiving social support services. Overall, we deliver more than $40 million in free, personalized services each year to individuals and families affected by cancer nationwide and internationally.

Additionally, CSC is home to the Research and Training Institute—the only entity of its kind focused solely on the experiences of cancer patients and their loved ones. The Research and Training Institute has contributed to the evidence base regarding the cancer patient experience through its Cancer Experience Registry, various publications and peer-reviewed studies on distress screening, and the psychosocial impact of cancer and cancer survivorship. This combination of direct services and research uniquely positions CSC to provide valuable patient and evidence-informed feedback to the Duke Margolis Center for Health Policy.

We appreciate your outreach to the community and respectfully submit these comments regarding the “Framework for Regulatory Use of Real-World Evidence.” We are grateful that you’ve contributed this important document to the discussion regarding both real-world data (RWD) and real-world evidence (RWE). This briefing paper is a helpful primer that clearly outlines the definitions of both RWD and RWE and summarizes potential regulatory, clinical, and data collection considerations. We believe that RWD and RWE pave the way for researchers, clinicians, policymakers, and regulators to more comprehensively assess the impact of treatments, health system innovations, and policies on patients and their loved ones.

From the patient advocacy perspective, we believe that there is room for improvement in the current use of RWD and RWE as outlined in this briefing paper. While gathering data from multiple sources beyond randomized clinical trials in an excellent first step towards a more holistic assessment of patient information, it is only the first phase in a truly patient-centered process. Patient-reported outcomes (PROs) are defined as “any report of the status of patient’s health condition that comes directly from the

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patient, without interpretation of the patient’s response by a clinician or anyone else” (National Quality Forum, n.d.). In other words, PROs include data regarding how patients are feeling, their symptoms, and any effects of a treatment (Weldring & Smith, 2013). However, PROs do not always measure what is important to the patient as defined by the patient. Rather, they are often a reflection of information that is important for the patient’s health care team or the health care system to gather. Questions posed by members of the health care team and information that is simply reported by the patient do not necessarily capture the full range of factors that the patient considers to be important throughout their service journey.

As noted in the document, certain symptoms are rarely captured in clinical records and would therefore not likely be included in RWE unless patient-generated data on such symptoms could be adequately incorporated. Ushering in an era of RWD and RWE means that we have an opportunity to elicit “patient directed outcomes” or “patient experience outcomes” based on what is defined as important by the patient themselves. These not only include information regarding side effects, symptom management, or functional status but also social and emotional status as well as logistical concerns such as out-of-pocket costs, ability to get to and from treatment, impact on work and family life, or concerns about appearance, among others. RWD must be inclusive not only of signs and symptoms of disease, but of the full range of factors that contribute to the quality of life of patients and their loved ones. Collecting data based on systems as they exist today, and processes as we have always known them, is simply not good enough in an era of precision medicine and patient-centered care. As noted in the briefing paper, it may be necessary to request new fields in electronic health records (EHR). We also support ways to validate emerging tools through which patients can actively monitor and offer corrections to their EHR. Finally, these changes to patient experience data should apply to all patients and diseases including those that are low-severity or low-prevalence.

In conclusion, CSC thanks the Duke Margolis Center for Health Policy for the opportunity to provide feedback regarding the “Framework for Regulatory Use of Real-World Evidence.” We believe that RWD and RWE will not only empower patients to be more involved in their treatment, the health care system, and in policymaking, but will also create a more responsive and dynamic regulatory process. However, as is noted in the briefing paper, there must be parameters in place to ensure adherence to scientific rigor. We believe that patients’ values, beliefs, and preferences can be built into rigorous study designs and clinical practice, and should play a significant role in the development, use, and analysis of RWD and RWE. As one of the largest direct practice and oncology patient advocacy organizations, CSC stands ready to help recruit patient advocates and offer feedback or information to help inform and shape this discussion. Please feel free to contact me at 202.650.5382 or linda@cancersupportcommunity.org if you have questions or if we can serve as a resource to your work.

Sincerely,

Linda House, RN, BSN, MSM
President

References

Weldring, T. & Smith, S. M. S. (2013). Patient-reported outcomes (PROs) and Patient-Reported Outcomes Measures (PROMs). Health Services Insights, 6, 61-68.