American Association for Cancer Research
FINDING CURES TOGETHER

1401 H Street, NW | Suite 740
Washington, DC 20005
202-898-6499 | 202-898-0966 Fax
www.AACR.org | @AACR | Facebook.com/AACR.org

February 5, 2019

Dockets Management Staff (HFA-305)
Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

RE: Docket No. FDA-2018-N-4000 Framework for a Real-World Evidence Program

To Whom It May Concern:

On behalf of the American Association for Cancer Research (AACR), the oldest and largest scientific organization in the world dedicated to the prevention and cure of cancer through research, education, communication, and collaboration, we sincerely thank the U.S. Food and Drug Administration (FDA) for the opportunity to provide comments in response to the Framework for FDA's Real-World Evidence Program. Our membership includes more than 40,000 basic, translational, and clinical researchers; population scientists; other health care professionals; and patient advocates residing in 120 countries.

The AACR commends the FDA's proven record of using real-world evidence (RWE). The Center for Devices and Radiologic Health (CDRH) regularly collects real-world data (RWD) from such sources as electronic health records and patient registries, using them to support over 50 regulatory decisions to date. When completed, CDRH's National Evaluation System for Health Technologies will generate better evidence for medical device evaluation and regulatory decision-making. For over a decade, the Sentinel Program, run through the Center for Drug Evaluation and Research, has made use of RWE to monitor safety and potential adverse effects of approved medical products. This expertise is critical as the FDA explores new ways to use RWE. Additionally, the recent hire of Amy P. Abernethy, MD, PhD, as principal deputy commissioner shows that the Agency is taking the implementation of the RWE framework seriously.

The AACR has long recognized the potential of RWE for advancing cancer research. In 2015, AACR Project Genomics Evidence Neoplasia Information Exchange (GENIE) was created to fulfill an unmet need in oncology by providing the statistical power necessary to improve clinical decision-making, particularly in rare cancers and rare variants in common cancers. This registry contains genomic data obtained during the course of routine practice at 19 national and international institutions. As a result, the registry is derived from data from a variety of cancer types, including rare cancers, and is enriched in examples of late-stage disease, approximating a "real-world" dataset. As of January 2019, the Project GENIE database contains almost 60,000 de-identified genomic records representing over 80 major cancer types.

The AACR was pleased to see that the Framework for FDA's Real-World Evidence Program recognizes the importance of data standards to the success of RWE. We also believe that it is necessary to consider metadata (where, how, and when data were collected as well as how individual data elements are defined by their collectors) and data uniqueness when determining data quality. Because RWD come from a variety of sources, the availability of metadata will support higher-quality RWE as well as help prevent the duplication of patient data within datasets. Project GENIE is working to adopt or use current standards where they exist and make the most sense, creating new definitions only where necessary. However, the intent is to make our definitions and data model open source, which we hope will promote their adoption as "new standards," and position Project GENIE to serve as an additional RWD source that could be used to compare to other initiatives.

American Association for Cancer Research
FINDING CURES TOGETHER

1401 H Street, NW | Suite 740
Washington, DC 20005
202-898-6499 | 202-898-0966 Fax
www.AACR.org | @AACR | Facebook.com/AACR.org

Recognizing the regulatory challenges associated with the utilization of RWE, the AACR has hosted events to promote discussion and disseminate knowledge, including Regulatory Science and Policy Sessions on RWE at our Annual Meetings in 2017 and 2018. Additionally, together with the FDA, we will host a full-day workshop on July 19, 2019, to explore both what is practical and possible for RWE in oncology. Using pre- and post-market use cases and examples from large genomic databases, the FDA-AACR Real-world Evidence Workshop will examine the regulatory and drug discovery implications of using RWE.

In conclusion, the AACR applauds the FDA's deliberate efforts to advance the use of RWE to support regulatory decision-making. As the FDA works to implement the Framework for RWE, the AACR stands ready to engage as stakeholder and partner. We look forward to continued collaboration with the FDA to advance RWE in support of the approval and understanding of safe and effective cancer drugs, biologics, and devices. If you have further questions, please contact Sarah Martin, MS, PhD, Associate Director, Regulatory Science and Policy, at sarah.martin@aacr.org.

Sincerely,

Kenneth C. Anderson, MD
Chair, Regulatory Science and Policy Subcommittee
American Association for Cancer Research

Margaret Foti, PhD, MD (hc)
Chief Executive Officer
American Association for Cancer Research

George D. Demetri, MD
Chair, Science Policy and Government Affairs Committee
American Association for Cancer Research