May 11, 2022

The Honorable Frank Pallone
U.S. House of Representatives
Washington, DC 20515

The Honorable Cathy McMorris Rodgers
U.S. House of Representatives
Washington, DC 20515

The Honorable Anna Eshoo
U.S. House of Representatives
Washington, DC 20515

The Honorable Brett Guthrie
U.S. House of Representatives
Washington, DC 20515

Dear Chairman Pallone, Ranking Member McMorris Rodgers, Chairwoman Eshoo, and Ranking Member Guthrie,

The Association for Clinical Oncology (ASCO) applauds your bipartisan commitment to reauthorizing the Food and Drug Administration (FDA) user fee programs and expand the Agency's authorities to protect patient safety. We are pleased to support provisions in the *Food and Drug Amendments of 2022* that will have a meaningful impact on cancer care. We urge Congress to pass this legislation before the September 30 deadline.

ASCO represents nearly 45,000 clinical oncologists, researchers, and other oncology professionals who treat and study patients with cancer across the country. ASCO provides the highest-quality resources in education, policy, and clinical care for patients with cancer.

The legislation includes many provisions that ASCO supports, in particular:

- Ensuring clinical trials are representative of diverse populations by requiring drug and medical device manufacturers to submit clinical trial diversity action plans to FDA early in their development process (Title V, Sections 501 and 503).
- Providing FDA with tools to ensure the agency can conduct thorough safety inspections efficiently (Title VII, Subtitle B).
- Improving the FDA's review for safety and efficacy of medical products, including cell and gene therapies, drugs for rare diseases, and novel medical devices (Title VII/Section 707).
- Strengthening program integrity for the Accelerated Approval pathway and preserving patient access to approved treatments by ensuring that drugs show clinical benefit through post-approval studies in a timely manner and streamlining the process for withdrawing approvals for drugs that fail to show a clinical benefit (Title VIII, Section 804).
- Improving the use of real-world evidence and real-world data (Title VIII, Section 805).

Increasing Diversity in Clinical Trials

As cancer care becomes more complex and personalized, the research through which new advances are developed must include diverse representation of participants. ASCO believes that all populations should have an equal opportunity to participate in, be recognized for, and benefit from research across the spectrum, including clinical trials. However, due to a variety of barriers, many patient populations continue to be underrepresented in clinical trials and health care-related research.

Ensuring Patient Access to Life-Saving Therapies

ASCO has long supported the accelerated approval and complementary expedited review programs, which provide patients with the earliest possible access to potentially life-saving therapies, instead of requiring them to wait for confirmation of long-term endpoints, such as survival. Specifically, the Accelerated Approval Program allows for earlier approval of drugs that treat serious conditions, and that fill an unmet medical need based on a surrogate endpoint that demonstrates drug value and an improvement in patient outcomes. The benefit of the program is that life-saving drugs reach patients faster, however many worry that the earlier a treatment is available to patients, the less safety information exists about a particular drug and the harder it may be to complete the confirmatory trials when a patient can receive the drug by prescription.

To mitigate the risks, the FDA uses the same standards to approve treatments via accelerated approval as they would via a traditional pathway; drugs must demonstrate substantial evidence of safety and that the "drug is safe for use under conditions prescribed, recommended or suggested in the proposed labeling" and must meet the standard for effectiveness, which requires substantial evidence based on "adequate and well controlled clinical investigations." Sponsors must also agree to conduct a post-marketing confirmatory study or studies to verify and describe the relationship between the endpoint and the expected clinical benefit following a drugs approval via the accelerated approval pathway. Additionally, as part of its review, the FDA considers the overall risk and benefit of approving, rejecting, or waiting to approve the drug. This evaluation considers the risk for patients who lack access to effective treatments.

The Accelerated Approval Program has been vital for cancer treatment development. Between 2010 to 2020, 85% of accelerated approvals were for oncology indications. While survival is always the end-goal, there are many ways to evaluate benefit of a drug to the patient and their quality of life, including reduction of the size of the tumor, delay in the progression of the disease, and the establishment of complete response rates in hematological diseases. Our understanding of cancer and its treatment is changing every day. Our regulatory systems must adapt in ways that meet a rapidly evolving science, deliver cutting edge treatments to patients, and balance safety with risk.

Expanding Access to and the Use of Real-World Evidence

Advances in health information technologies and a dramatic increase in the adoption of electronic health records (EHRs) have created new opportunities for streamlining clinical evidence through collection of real-world data (RWD). Data collection that captures demographic characteristics including race/ethnicity, sexual orientation, gender identity, socioeconomic status, age, stage of disease, comorbidities, etc. helps reduce health disparities by ensuring all patient populations are included in the development in treatments. Sources of RWD include electronic health records, insurance claims, patient registries, and digital health solutions outside of conventional clinical trials. Additionally, RWD has a largely untapped potential to supplement clinical trial data and improve external validity—and establish real-world effectiveness and toxicity, especially in oncology.

The *21st Century Cures Act* in 2016 tasked the FDA with implementing several provisions, including the Real-World Evidence (RWE) Program and Draft Guidance. This provision requires the FDA to publish draft guidance on how RWE can contribute to the assessment of safety and effectiveness in regulatory submissions and requires the FDA to explore the use of RWE for additional indications of approved drugs and post-approved study requirements. Additionally, the FDA is working with the NCI on data sharing and data aggregation. These provisions will have implications for ASCO's clinical trial, the Targeted Agent and Profiling Utilization Registry (TAPUR), and data registry, CancerLinQ.

ASCO urges the Committees to prioritize these provisions to allow our healthcare continuum to continue building on the use of RWE and RWD as a way to reduce healthcare disparities.

Conclusion

As Congress continues consideration of this must-pass legislation and the additional policy provisions, I urge you and your colleagues to ensure the above remain included throughout the process. These provisions go a long way to strengthening the FDA's ability to increase innovation and ensure patients receive the best possible treatments. We are committed to working with you as the legislation advances. If you have any questions, please contact Kristine Rufener, Associate Director of Congressional Affairs, at Kristine.Rufener@asco.org or 571-483-1547.

Sincerely,

Howard A. Burris III, MD, FASCO
Chair of the Board
Association for Clinical Oncology