

May 26, 2023

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

**Re: Clinical Trial Considerations to Support Accelerated Approval
of Oncology Therapeutics; Draft Guidance for Industry;
Availability (Docket No. FDA-2023-D-0110)**

To the Food and Drug Administration:

The RWE Alliance appreciates the opportunity to comment on the draft guidance entitled “Clinical Trial Considerations to Support Accelerated Approval of Oncology Therapeutics” (“Draft Guidance”).¹ We are a coalition of real-world data (“RWD”) and analytics organizations with a common interest in harnessing the power of real-world evidence (“RWE”) to inform regulatory decision-making to improve patients’ lives. Our members have deep knowledge and experience working with healthcare data across disease areas and patient populations, and we aim to bring these collective insights to bear in support of RWE policies.²

The RWE Alliance envisions a future in which data generated in everyday clinical practice and everyday life through electronic health records (“EHRs”), administrative claims and billing data, product and disease registries, personal devices, wearables, and health applications will be used to generate evidence that complements clinical trial data to inform regulatory decisions. To achieve this goal, the RWE Alliance advocates for policies that will (1) advance FDA’s RWE Framework, (2) encourage the use of RWE to better understand treatment effects in underrepresented populations, (3) enhance opportunities for RWE organizations to consult with FDA, and (4) increase communication on the generation and use of RWE.³

We appreciate the Draft Guidance’s recommendations to sponsors of anti-cancer drugs or biological products on considerations for designing trials intended to support

¹ 88 Fed. Reg. 18148 (Mar. 27, 2023).

² For information about our members, please see our website, <https://rwealliance.org/who-we-are/>.

³ Additional information about what we believe is available on our website, <https://rwealliance.org/what-we-believe/>.

accelerated approval. We recommend that the final guidance highlight the potential uses and benefits of RWD/E as a source of clinical evidence when designing trials intended to support accelerated approval for oncology drug or biological products. FDA's accelerated approval pathway is critical for development of oncology therapies to meet significant unmet medical need. There are opportunities to leverage RWD/E in generating evidence to support an accelerated approval as well as confirmatory evidence to verify a product's clinical benefit.⁴ We believe such uses include, for example, RWD-based external controls,⁵ pragmatic study designs that fill in critical variables using RWD, fully observational real-world studies to determine standard of care treatments and outcomes, innovation extension studies, and post-approval monitoring studies using RWD. RWD also can be used to improve long-term outcome measurements (e.g., allowing follow-up data collection in registries) and help characterize subpopulations. In appropriate cases, RWD may be used to generate relevant evidence faster and more efficiently than might be possible through a traditional clinical trial, which ultimately benefits patients. Finally, we recommend that the final guidance reference guidances⁶ from FDA's RWE Program that describe relevant considerations when using and designing trials using RWD/E.

The RWE Alliance appreciates the Agency's commitment to advancing the use of RWD and RWE in regulatory decision-making. Thank you for considering these comments, and please let us know if you have any questions. We welcome the opportunity to discuss further.

Best regards,

The RWE Alliance

⁴ For example, FDA approved Vioice for the treatment of adult and pediatric patients 2 years of age and older with severe manifestations of PIK3CA-Related Overgrowth Spectrum (PROS) who require systemic therapy through its accelerated approval pathway. FDA based its approval on EPIK-P1, a retrospective chart review study.

⁵ For example, FDA approved Blincyto for the treatment of Philadelphia chromosome -negative relapsed or refractory B-cell precursor acute lymphoblastic leukemia through its accelerated approvals pathway. FDA considered as part of its review an analysis of patient-level data from 694 historical controls to better understand efficacy results in the context of the heterogeneity of the patient population with regard to prognostic factors.

⁶ The final guidance should consider referencing FDA's draft guidance "Considerations for the Design and Conduct of Externally Controlled Trials for Drug and Biological Products" in addition to other relevant guidances.