REAL-WORLD EVIDENCE ALLIANCE

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

Re: Advancing Rare Disease Therapies Through a Food and Drug Administration Rare Disease Innovation Hub; Public Meeting; Request for Comments (Docket No. FDA-2024-N-3528)

To the Food and Drug Administration:

The RWE Alliance appreciates the opportunity to comment on the public meeting "Advancing Rare Disease Therapies Through an FDA Rare Disease Innovation Hub" ("Public Meeting") hosted by the FDA Rare Disease Innovation Hub and the Reagan-Udall Foundation.[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 the lives of patients. 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 from electronic health records, administrative claims and billing records, product and disease registries, personal devices, wearables, and health applications will be used to generate evidence to support regulatory decision making related to medical product safety and effectiveness. To achieve these goals, 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, (4) increase communication on the generation and use of RWE, and (5) recognize the unique aspects of and opportunities for RWD/E.[3]

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[1] 89 Fed. Reg. 65631 (August 12, 2024).

[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/.

We agree with FDA about the need for a comprehensive rare disease strategic agenda that leverages innovative trial designs and focuses on developing therapies for all types of conditions, including those affecting small populations and those with a variable or less established natural history. We believe that real-world data sources will play a vital role to help push FDA's rare disease agenda forward. RWD is essential for developing therapies for rare diseases because of its role in establishing disease natural history. RWD also can unlock additional insights on the safety and effectiveness of medical products in real-world settings and in the context of small patient populations that require innovative study designs to generate robust evidence. We urge the FDA Rare Disease Innovation Hub to center real-world data sources as essential tools to help bring safe and effective therapies to patients with rare conditions.

We offer the following specific comments in response to FDA's question: "What specific rare disease-related scientific, regulatory, or policy issues should be prioritized for consideration by the Rare Disease Innovation Hub?" We recommend that FDA continue developing the scientific methods to support a range of innovative or adaptive trial designs for rare disease drug development that can potentially leverage RWD. For instance, clinical trials using external control arms can offer an important alternative for generating effectiveness evidence for serious and rare conditions that cannot be studied using a randomized design. FDA should consider a range of scenarios in which RWD can replace or augment the control arm in both early- and late-stage trials. RWD also can play an important role in measuring outcomes and other data elements needed to evaluate therapies for rare diseases. We suggest that FDA publish case studies or details on these innovative or adaptive trial designs, including statistical methods, to advance the science of evidence generation.

The RWE Alliance appreciates the Agency's commitment to advancing the use of RWD and RWE to develop new treatments for patients with rare diseases. 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