RARE DISEASE DIVERSITY COALITION

August 23, 2024

Dr. Robert McKinnon Califf

Commissioner

Food and Drug Administration

10903 New Hampshire Avenue

Silver Spring, MD 20993

RE: FDA Guidance for Industry: Diversity Action Plans to Improve Enrollment of Participants from Underrepresented Populations in Clinical Studies

Commissioner Califf:

The Rare Disease Diversity Coalition (RDDC) is thankful for the opportunity to comment on

the Food and Drug Administration (FDA) industry guidance on Diversity Action Plans to Improve Enrollment of Participants from Underrepresented Populations in Clinical Studies. As an advocacy group dedicated to representing diverse patients with rare diseases, we recognize the pivotal role that inclusive clinical trial enrollment plays in advancing medical research and improving health outcomes. Achieving diversity in clinical trial participant pools is essential for ensuring that treatments are effective and safe for all populations. Inclusive enrollment not only enhances the generalizability of clinical trial results but also addresses health disparities by ensuring that therapies are tested across a broad spectrum of demographic groups.

I. About RDDC

RDDC, a partnership involving rare disease and equity advocates, public health experts, and industry leaders, was launched in 2020 to address the extraordinary challenges faced by rare disease patients of color. RDDC and its partners are committed to be a catalyst for progress for people of color with rare diseases, who are part of two struggles: as rare disease patients, they strive to be included (and not forgotten) in healthcare; and, as people of color, they fight daily for equity—against the reality of historic bias and its lingering disparate social, economic, and health effects. RDDC seeks to identify and advocate for evidenced-based solutions to alleviate the disproportionate burden of rare diseases on these communities.

Our patient communities look to specialists, diagnostics, and emerging, innovative therapies that target specific disease mechanisms for renewed hope that treatment options, and even a cure, might be on the horizon to address the life-limiting and life-threatening conditions they face. People of color in our patient communities often face additional hurdles due to the impact of the social determinants of health, socioeconomic status, cultural barriers, and the lingering impact of historic racism in this country. The genesis of many inequities in our healthcare stem from a precursor disparity in research and availability of new treatments.

II. RDDC's Comments on the Industry Guidance

A. Broadening the Lens: Defining All Underrepresented Populations in Clinical Trials

We recommend the FDA provide a comprehensive definition of underrepresented populations in clinical trials, incorporating a wide range of demographic factors. This expanded definition should include not only racial and ethnic minorities but also consider gender minorities, individuals from rural or geographically isolated areas, and those with varying socioeconomic statuses. Additionally, it should address disparities related to health literacy and access to healthcare resources. By recognizing and defining these diverse groups, the FDA can assist clinical trial sponsors to more accurately identify and address the specific barriers that contribute to underrepresentation in clinical research. For instance, individuals from rural communities may face significant travel and logistical challenges, while racial and gender minorities might

experience systemic biases that deter their participation. Socioeconomic factors can also impose financial constraints, limiting access to trial opportunities.

Addressing the intersectionality of these factors is crucial for developing effective strategies to enhance inclusivity in clinical trials. Any combination of these elements can create compounded barriers that prevent certain groups from participating, thereby skewing research results and limiting the applicability of findings across diverse populations. By embracing a broad and inclusive definition of underrepresented populations, the FDA can ensure that clinical trials more accurately reflect the diverse demographics of patients who will ultimately use the treatments being studied. This approach not only promotes equity but also strengthens the reliability and relevance of clinical research outcomes, leading to better-informed healthcare decisions and more effective treatments for all.

B. Promote Demographic Transparency and Diversity Among Clinical Trial Staff

We recommend that the FDA urge sponsors to include comprehensive demographic information about clinical trial and site staff in their Diversity Action Plans. By urging the disclosure of staff demographics, the FDA can help identify and address disparities in staff composition. This transparency allows for a more informed assessment of whether clinical trial teams reflect the diversity of the patient populations they aim to serve. For example, if a trial site predominantly employs staff from a single demographic group, it may inadvertently create barriers for participants from different backgrounds who may feel less comfortable or understood by the staff.

Encouraging diversity among clinical and site staff is essential for fostering an inclusive research environment. A diverse team is better equipped to understand and address the unique needs of varied patient populations. Moreover, it can enhance recruitment efforts, as participants are more likely to engage with trials when they see individuals who resemble their own cultural, ethnic, or social backgrounds. For instance, a research team with staff from multiple ethnic backgrounds may improve outreach efforts and patient engagement by addressing culturally specific concerns and building trust within diverse communities.

C. Foster Long-Lasting Relationships with Local Communities

We recommend that the FDA encourage sponsors to build and maintain enduring relationships with the communities in which they conduct clinical trials. Developing long-term partnerships

with local organizations, community leaders, and patient advocacy groups is crucial for improving engagement and trust. These relationships can facilitate more effective recruitment strategies and ensure that clinical trials are designed to meet the needs of the community. For example, sponsoring health fairs or informational sessions in collaboration with local organizations can help raise awareness about clinical trials and address community-specific concerns.

Maintaining these relationships beyond the scope of individual trials also helps create a supportive environment for ongoing research. Long-term community engagement fosters mutual trust and collaboration, which can lead to better recruitment, retention, and overall trial success. By integrating feedback from community stakeholders and adapting trial protocols to better fit local needs, sponsors can enhance their research efforts and contribute to more inclusive and effective clinical studies.

D. Utilize Culturally Appropriate and Inclusive Materials

We recommend that sponsors ensure all clinical trial materials, including informed consent forms, educational resources, and promotional content, are culturally appropriate and accessible to individuals of various languages and reading levels. Providing materials that are not only translated but also culturally adapted ensures that participants from diverse backgrounds can fully understand and engage with the trial. For instance, translation should account for cultural nuances and medical literacy, avoiding jargon and using clear, straightforward language.

Inclusive materials help eliminate barriers related to language and literacy, making it easier for participants from various demographic groups to comprehend trial details and make informed decisions. By tailoring content to be culturally sensitive and accessible, sponsors can improve participant comprehension and involvement, which is crucial for the accuracy and reliability of trial data. Ensuring that materials are inclusive and respectful of cultural differences also demonstrates a commitment to equitable research practices, enhancing participant trust and engagement across diverse populations.

Conclusion

Thank you for the opportunity to provide these comments. RDDC looks forward to working with the FDA to help achieve greater diversity in clinical trials.

Sincerely,

Jenifer Waldrop

Executive Director, RDDC