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January 27, 2023

Division of Dockets Management
Office of Science and Technology Policy
Eisenhower Executive Office Building
1650 Pennsylvania Avenue
Washington, D.C. 20504

RE: Docket No. 2022-23110: Request for Information; Clinical Research Infrastructure and Emergency Clinical Trials

To Whom It May Concern:

The Pharmaceutical Research and Manufacturers of America ("PhRMA") is pleased to submit these comments to the Office of Science and Technology Policy (OSTP) in response to the Notice of Request for Information (RFI) on clinical research infrastructure and emergency clinical trials.[1] PhRMA recognizes OSTP's commitment to advancing an infrastructure that can support clinical trials to address outbreaks of disease and other emergencies, the expansion of clinical research into underserved communities, and increase diversity among both trial participants and clinical trial investigators. PhRMA and its member companies believe that creating a sustainable network of sites in underserved communities will help ensure ongoing access to clinical trials for those who want to participate in both emergency and non-emergency situations, ultimately helping to enhance diversity in clinical research and advance health equity.

PhRMA is a voluntary, nonprofit association that represents the country's leading biopharmaceutical research and biotechnology companies, which are devoted to discovering and developing medicines that enable patients to live longer, healthier, and more productive lives. Since 2000, PhRMA member companies have invested more than $1.1 trillion in the search for new treatments and cures, including $102.3 billion in 2021 alone.

I. GENERAL COMMENTS

We appreciate OSTP's solicitation of feedback on "how to ensure that trial sites in underserved areas are included [in an emergency clinical trial infrastructure] and how to increase diversity both among study participants and among the investigators." PhRMA and its member companies are committed to enhancing diverse participation in clinical trials, including identifying and addressing potential barriers to enrollment, retention, and a positive patient experience.[2] PhRMA applauds the OSTP for convening the public meeting "Preparing U.S.

[1] 87 FR 64821; Notice of Request for Information (RFI) on Clinical Research Infrastructure and Emergency Clinical Trials (October 2022). Available at https://www.govinfo.gov/content/pkg/FR-2022-10-26/pdf/2022-23110.pdf

[2] See comments filed by PhRMA on Aug. 7, 2021, in response to Draft Guidance for Industry - Enhancing the Diversity of Clinical Trial Populations — Eligibility Criteria, Enrollment Practices, and Trial Designs; See comments filed on Jun 29, 2020 in response to Request for Comments – Office of Minority Health and Health Equity Strategic

Clinical Trials Infrastructure for Emergencies: A White House Virtual Roundtable Discussion"[3] on January 12, 2023, and seeking stakeholders' input.

PhRMA shares OSTP's recognition of the importance of having an infrastructure that supports emergency clinical trials and agrees with OSTP that one goal of such an infrastructure should be to "support the expansion of clinical research into underserved communities, and increase diversity among both trial participants and clinical trial investigators."[4] PhRMA believes an important part of any such infrastructure is the need to support participation and access of diverse populations. PhRMA understands that addressing potential barriers to clinical trial enrollment is a key consideration.

As we look at the development of new medicines during both emergency and non-emergency situations, it is essential to take meaningful action to help ensure that underserved and underrepresented communities, who have historically faced barriers to participating in the development of health care advances, are given the opportunity to be included every step of the way. As an emergency infrastructure is contemplated, it will be critical to think through a robust education and outreach effort to address potential misperceptions that "emergency" suggests any jeopardizing of a focus on safety and efficacy.

In addition to the general comments above, PhRMA provides specific comments in response to the RFI below.

II. SPECIFIC COMMENTS

A. Effective Ways to Increase Diversity

1. Expanding Clinical Research Sites in Underserved Areas

Enhancing diversity in clinical trials depends on identifying and reducing barriers to clinical trial access and participation. To this end, there is a need to work with patients, health care providers, and clinical trial investigators to understand barriers and identify approaches to address these barriers and enhance access to clinical trials for diverse patient populations by:

- Taking into account the needs of diverse populations in clinical trial design.
- Adopting enrollment and retention practices that enhance inclusiveness and make trial participation less burdensome for participants.
- Broadening eligibility criteria to increase diversity in enrollment when scientifically and clinically appropriate.

Priorities; See comments filed on Sep. 9, 2020 in response to Request for Comments - Office of Women's Health Strategic Priorities.; See comments filed on June 13, 2022 in response to Draft Guidance for Industry- Diversity Plans to Improve Enrollment for Participants From Underrepresented Racial and Ethnic Populations in Clinical Trials.

[3] https://www.whitehouse.gov/ostp/news-updates/2023/01/06/preparing-u-s-clinical-trials-infrastructure-for-emergencies-a-white-house-virtual-roundtable-discussion/

[4] *See* 87 Fed. Reg. at 64821.

PhRMA believes that building trust in underserved communities and acknowledging past wrongs is an important first step in enhancing clinical trial diversity.[5] Some patients may not trust medical research due to the historic record of mistreatment,[6] Today, patients and research participants' rights are protected by law and ethics committees, including institutional review boards that oversee clinical trials.[7]

PhRMA also believes in the importance of enhancing education about the role of clinical trials throughout the medical community and throughout the range of potential study participants and trusted thought leaders to enhance awareness of and diversity among clinical investigators, clinical trial support staff, and others that can help broaden representation and participation in the clinical trial process. The clinical trial process including the recruitment and retention of patients is complex and multifactorial. The lack of participation by historically understudied populations often is due to lack of clinical trial awareness at hospitals and clinics that treat diverse populations. To address this gap, PhRMA believes that it is important to conduct outreach to the medical professionals in underserved communities and support trial sites with comprehensive education on medical product development. There is a need to support the recruitment and retention of clinical trial personnel with diverse backgrounds, including racial and ethnic backgrounds, and support the collaboration of trusted messengers to educate underserved communities on clinical trials.

Another effective way to increase diversity in study participants is to ensure adequate community outreach by improving clinical trial awareness, community health education and individual health literacy. Educational efforts are a key component of reaching underrepresented populations. Outreach efforts should be aimed at increasing access and reducing barriers for underrepresented and diverse populations to participate in clinical trials. This can be done by partnering with health and community advocacy groups to reach underrepresented populations, to increase clinical trial awareness, and provide access to potential opportunities for participation.

2. *Use of Decentralized Clinical Trials and Technological Innovations Such as Digital Health Technologies*

The conduct of clinical trials may result in recruitment challenges and enrollment barriers that may occur as a result of factors such as site location, planned visit schedules, as well as travel

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[5] PhRMA members voluntarily adopted the Clinical Trial Diversity Principles, which aims to increase the participation of underrepresented populations to clinical trials. Principles on Conduct of Clinical Trials & Communication of Clinical Trial Results. Available at https://phrma.org/-/media/Project/PhRMA/PhRMA-Org/PhRMA-Org/PDF/P-R/PhRMAPrinciples-of-Clinical-Trials-FINAL.pdf.

[6] The U.S. Public Health Service Syphilis Study at Tuskegee. Available at https://www.cdc.gov/tuskegee/timeline.htm.

[7] Institutional Review Boards (IRBs) and Protection of Human Subjects in Clinical Trials. Available at https://www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/institutional-review-boards-irbs-and-protection-human-subjects-clinical-trials

and financial implications. There is a potential for digital health technologies (DHTs) to provide scientific and practical advantages in supporting the assessment of patients by generating information outside of the traditional clinic visit though it must be recognized that significant variability in access to broadband and digital technologies. DHT tools, which encompass a range of solutions that include digital apps, in-home testing, remote monitoring and diagnostics, and other technologies, may help improve diverse participation in clinical trials when coupled with other efforts and resourced appropriately. The use of DHTs can support and enable the conduct of decentralized clinical trials (DCTs), the clinical investigations in which some or all trial-related procedures and data acquisition take place at locations remote from the investigator. DCTs can help improve access for patients by reducing the in-person clinical trial site visits and helping reach patients who may not otherwise be able to easily access a clinical trial.

PhRMA supports the development and use of additional technology tools to support a health emergency clinical research infrastructure. In the specific context of an emergency or large-scale disease outbreak, the use of DHTs and DCTs can provide increased access for patients to clinical trial networks. Throughout the COVID-19 pandemic, DCT and DHT tools were helpful in conducting clinical trials and reaching underserved communities. The Prescription Drug User Fee Act VII and the Food and Drug Omnibus Reform Act[8] will build upon these lessons learned during the COVID-19 pandemic and advance the use of digital technologies, decentralized clinical trials, and other novel clinical trial designs to help increase clinical trial access for patients and enhance clinical trial diversity and enrollment.[9]

B. "Warm Base" Research

PhRMA believes a community-based infrastructure that supports underserved communities is important not just for emergency clinical research, but for overall equitable access to clinical trials. Creating an infrastructure that includes a network of clinical trials sites connected through and supported by robust communication, community relations, ongoing site training and mentoring, sustainable support and standardized platforms and metrics. These are important components of a community-based infrastructure to support clinical trials. Having a network of sites in a state of readiness to undertake additional or future clinical research, i.e., a "warm base," can help facilitate clinical trials more efficiently during an emergency.

Over the past two years, PhRMA has solicited feedback from thousands of stakeholders – patients, providers, clinical trial experts and racial justice experts - to thoroughly understand the systemic challenges to enhancing clinical trial diversity and help build towards actionable advancements.[10]

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[8] *See, e.g.,* Pub. L. No. 117-328, §§ 3605-3603 (directing FDA to "convene a public meeting to discuss the recommendations provided by [FDA] during the COVID-19 emergency period to mitigate disruption of clinical trials" and issue or revise draft guidance on "recommendations to clarify and advance the use of" DCTs)

[9] For more info, see https://www.fda.gov/media/151712/download

[10] The initiative follows more than two years of PhRMA-led stakeholder engagement to assess barriers to clinical trial participation and identify tangible actions and goals that can make a difference. PhRMA Joins Top Academic Leaders to Announce New Community-Based Initiative to Enhance Clinical Trial Diversity. For more info,

III. CONCLUSION

PhRMA and its member companies support efforts to enable emergency clinical research and build capacity to conduct coordinated and large-scale clinical trials across a range of institutions and sites to address outbreaks of disease and other emergencies. PhRMA appreciates the opportunity to provide comments on this RFI and welcomes additional questions regarding this topic.

Respectfully submitted,

/s/

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/s/

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https://phrma.org/resource-center/Topics/Access-to-Medicines/PhRMA-Joins-Top-Academic-Leaders-to-Announce-New-Community-Based-Initiative-to-Enhance-Clinical-Trial-Diversity