NATIONAL HEALTH COUNCIL
Celebrating 100 Years

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February 28, 2020

The Honorable Stephen M. Hahn, MD
Commissioner
Food and Drug Administration
Dockets Management Staff (HFA-305)
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

RE: Office of Minority Health and Health Equity Strategic Priorities; Establishment of a Public Docket; Request for Comments

Dear Commissioner Hahn:

The National Health Council (NHC) appreciates the opportunity to provide comments as the Food and Drug Administration's (FDA's) establishes its Office of Minority Health and Health Equity (OMHHE) Strategic Priorities public docket. The OMHHE outreach, communication, research, and collaboration programs have an important role in reducing health disparities that disproportionately affect racial and ethnic minority, underrepresented, and underserved populations. We agree that to achieve OMHHE's goal of health equity our health care system must identify and address the multiple, complex factors driving health disparities.

Created by and for patient organizations 100 years ago, the NHC brings diverse organizations together to forge consensus and drive patient-centered health policy. We promote increased access to affordable, high-value, sustainable health care. Made up of more than 140 national health- related organizations and businesses, the NHC's core membership includes the nation's leading patient organizations. Other members include health-related associations and nonprofit organizations including the provider, research, and family caregiver communities; and businesses representing biopharmaceutical, device, diagnostic, generic, and payer organizations.

Overarching Comments

FDA should work across the agency to address disparities of all underrepresented, underserved, and/or disadvantaged populations.

While we wholeheartedly support FDA's focus on addressing racial and ethnic disparities, we believe that the FDA should also work thought the agency to identify the unique needs of and address disparities faced by all underrepresented, underserved, and/or disadvantaged populations, including those with disabilities and complex health needs. While the OMHHE can serve an important role, it is important that its focus be incorporated throughout the culture and operations of the agency. An emphasis on bringing issues affecting underrepresented populations to the forefront of the FDA and ensuring that there is sufficient expertise on the topic, should exist in all aspects of the FDA, ranging from the Commissioner's office to each review division. This could be a comparable process to the FDA's current approach to rare disease, where Rare Disease Program staff are embedded in each review division.

As a part of these efforts, the FDA should consider a broader focus on underserved populations. For example, the agency could consider a definition that is consistent with the one used by the Centers for Medicare & Medicaid Services' (CMS') Office of Minority Health which "offers a comprehensive source of information on eliminating health disparities and improving the health of all minority populations, like racial and ethnic minorities, people with disabilities, members of the lesbian, gay, bisexual, and transgender community, and rural populations."[1]

The NHC particularly asks FDA to include complex populations in its emphasis on underserved and underrepresented populations. As FDA noted in its draft guidance on Enhancing the Diversity of Clinical Trial Populations, "failure to include complex participants in a development program may lead to a failure to discover important safety information about use of the investigational drug in patients who will take the drug after approval." For many individuals, the ability to take part in the drug-development process is an empowering step that aligns with their health care goals and could allow earlier access to treatments to significantly improve health outcomes. A focus on this population, not only in clinical trials, can help ensure that the FDA considers how medical products work in a real-world setting.

Product Development Efforts

FDA should encourage industry engagement with patients from underserved populations throughout the product lifecycle.

The NHC has previously commented on the FDA's patient-focused drug development (PFDD) discussion documents related to Guidance 2: "Methods to Identify What is Important to Patients" and Guidance 3: "Selecting, Developing or Modifying Fit-for-

[1] https://www.cms.gov/About-CMS/Agency-Information/OMH

Purpose Clinical Outcomes Assessments (COAs)." As we noted in those comments, patient engagement in product development occurs at two levels:

- Patients engaged as partners informing the drug-development process; and
- Patients participating to provide data on the patient experience to inform development and studies.

We have previously expressed the concern that historically the primary role of patients has been limited to their role as a study subject and have supported FDA's recent efforts to enhance patient engagement throughout the product development process. We urge FDA to connect overall patient engagement efforts with those efforts specifically designed to engage patients from underserved populations to ensure continued engagement and bi-directional communications between FDA and patients, including those with disabilities and complex health care needs, and to understand real-world treatment experiences of underserved populations.

The NHC believes strongly that the scope and breadth of patient involvement in developing and communicating experiences with treatment are fundamental to any efforts to increasing diversity in medical product development. In practical terms, this means that those engaged - patients, caregivers, advocates, and advocacy organizations - should be *representative* of the target patient population. Patient engagement strategies that fail to focus on securing perspectives of populations representative of a specific disease state, will likely fall short of delivering the diversity essential to OMHHE's efforts.

In 2017, the NHC convened a half-day Roundtable with key stakeholders, including representatives from patient groups, life science companies, value-assessment framework developers, payers, research organizations, and the FDA. We focused first on building a consensus understanding of what "representativeness" means when applied within the context of product development, regulatory decision-making, and value assessments[2]. Stakeholders agreed that representativeness means that a sufficient number and types of people are included in the engagement activity to ensure that those engaged can speak on behalf of the target population. It refers to "who" and "how many" individuals to include in an interaction (e.g., discussions intended to inform strategies on securing a diverse set of clinical-trial participants) in order to, as closely as possible, engage with individuals that represent the broader, target patient population.

The NHC urges OMHHE to work across the FDA toward actionable guidance provisions that reduce the burden of clinical-trial participation as a step toward more diverse enrollment.

Clinical-trial populations that reflect the diversity of the patient population for a specific disease is an ideal that is rarely attained due to challenges associated with study

[2] National Health Council. Tackling Representativeness: A Roadmap and Rubric, (https://www.nationalhealthcouncil.org/wp-content/uploads/2019/12/Representativen…)

participation. FDA has previously recognized that the burden associated with study participation can severely hamper diversity in clinical trials. These burdens include:

- Requiring participants to make frequent visits to clinical-trial sites may overly burden elderly, children, disabled, and cognitively impaired individuals requiring transportation or caregiver assistance, as well as participants in rural areas located far from research facilities;
- Financial costs of travel and lost work income;
- Time commitments interfering with work and family responsibilities; and
- A general mistrust and/or misunderstanding of clinical research among some sub/populations.

The NHC urges OMHHE to work across agency functions to address impediments that deter diverse clinical-trial populations and encourage sponsors to address these issues with patient engagement as early in clinical development planning as possible. A meaningful dialogue with the patient community can inform sponsors on the best ways to facilitate enrollment without impeding study validity (e.g., reduced frequency of study visits and use of technology to collect data on safety and efficacy). Perceptions of burden may vary significantly across disease states (e.g., added burden associated with mobility challenges is different from burden related to lost income), and patient engagement is crucial to determining strategies that are most effective and valuable to underserved patients.

The OMHHE could further enhance its value to the stakeholder community by working to operationalize elements of FDA's general guidance on reducing clinical-trial participant burden, including:

- Encouraging clinical-trial sites in geographic locations with a higher concentration of racial and ethnically diverse patients;
- Working with sponsors to incorporate diversity considerations when selecting health care providers to assist with clinical-trial recruitment; and
- Facilitating bi-directional public outreach and education.

We further urge the FDA to recognize that efforts to promote diverse clinical-trial enrollment should begin early in development planning. OMHHE could fill vital information gaps on the prevelance of disease across various populations to help inform target trial participants. As part of this activity, it would also be helpful to provide guidance to industry on how to navigate the complexity of enrolling appropriately diverse trials for U.S. approval while considering global prevelance factors that may vary in other countries in which the sponsor is seeking regulatory approval.

Once target populations are established, engaging patients and community leaders as partners early can ensure that study sites are both geographically and culturally accessible to diverse groups of patients. For example, if the majority of study sites are located in suburbs only accessible by car, lower-income individuals who depend primarily on public transportation will be unlikely to participate. Additionally, evidence suggests that patients are more likely to seek physicians with the same race, indicating

that not only geographic diversity, but also diversity of clinical staff is important[3]. Information on differences in how patients experience a particular condition, disease trajectory, and even what outcomes are particularly important from the patient perspective could improve study efficiency.

This proactive approach to patient engagement, with an eye toward ensuring that activities include a patient population representative of the disease state, would enable greater understanding on study participation barriers. Similarly, any granularity with respect to racial, ethnic, and other minorities is dependent on ensuring that engagement captures a representative population. For example, if cognitive impairment is a known symptom or common comorbidity in a target disease, clinical-trial protocols should include mechanisms that address the need for caregiver participation and minimizing caregiver burden. These mechanisms may be different for racial minorities than they might be for disabled populations or individuals in rural areas. Ideally, engagement efforts throughout a product lifecycle should be sufficiently flexible to accommodate divergent needs associated with geographic factors and differences in age, race, cognitive function, socioeconomic status, and language proficiency. Finally, continuing this engagement beyond the FDA-approval process to maintain a bi-directional mechanism for communicating patient experience would facilitate more efficient identification of any disparities in patient response to treatment.

Education and Outreach

OMHHE initiatives should improve information available to clinicians and patients on diverse experiences.

The NHC encourages OMHHE to devise outreach and engagement initiatives that will likely translate into improved patient outcomes. For individuals with chronic diseases and disabilities, information on the benefits and risks of incorporating a new treatment into disease management is rarely available when a product is introduced to market. For the most part, negative effects from medications are monitored on a case-by-case basis with clinicians adjusting dosage and treatment type according to how an individual reacts to prescribed treatments. OMHHE can play a role in identifying how the benefit-risk profile may vary in subpopulations and ensuring that the clinician community is aware of these subpopulation-specific factors, how they might influence outcomes, and what actions the patient and clinician should take.

FDA should reach out to patient organizations and provide guidance to industry to bridge the knowledge gap about medical products' performance in underserved populations.

The NHC has actively engaged with the FDA and other stakeholders over the past several years to ensure the patient voice is meaningfully represented in product development. The NHC has worked to build consensus across a range of issues to

[3] Saha S, Taggart SH, Komaromy M, Bindman AB. Do patients choose physicians of their own race?. *Health Aff (Millwood)*. 2000;19(4):76–83. doi:10.1377/hlthaff.19.4.76

advance the dialogue on patient engagement, from identifying key priority areas and topics for guidance development to providing feedback and suggestions on a common glossary of terms.

We remain committed to ensuring that a range of patient voices is represented through early, meaningful, proactive patient engagement. FDA outreach to industry and the scientific community should stress the benefits of engaging patient organizations – and organizations that specialize in reaching patients in underserved populations specifically - early so that underserved population considerations can be incorporated into all aspects of product development. Clear guidance to clinical trial sponsors and manufacturers that enables development of evidence on divergent responses to new treatments, including differences in clinical benefit and adverse events, without inhibiting efficient study design or jeopardizing product approval is an important step toward achieving OMHHE's objectives.

Conclusion

We appreciate the opportunity to provide input into the areas and types of engagement FDA's OMHHE should prioritize in the coming year(s), and potential mechanisms that can be used to implement them. We continue to support the FDA's work to identify, address, and reduce disparities in health care experienced by underserved, and underrepresented populations through meaningful patient engagement. We look forward to continuing to engage with the agency, including the OMHHE, as it further defines and executes its strategic priorities.

Please do not hesitate to contact Eric Gascho, Vice President of Policy and Government Affairs, if you or your staff would like to discuss these issues in greater detail. He is reachable by phone at 202-973-0545 or via e-mail at egascho@nhcouncil.org.

Sincerely,

Marc Boutin, JD
Chief Executive Officer National Health Council