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September 11, 2018

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

Re: Docket No. FDA-2018-D-1893 for "Patient-Focused Drug Development: Collecting Comprehensive and Representative Input; Draft Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders; Availability"

The Association of American Medical Colleges (AAMC) appreciates the opportunity to comment on the FDA's efforts on the methods and tools to collect meaningful information from patients and caregivers to inform regulatory decision-making and the development of medical products. The AAMC is a not-for-profit association representing all 151 accredited U.S. medical schools, nearly 400 major teaching hospitals and health systems, and more than 80 academic and scientific societies. Through these institutions and organizations, the AAMC represents nearly 173,000 faculty members, 89,000 medical students, 129,000 resident physicians, and more than 60,000 graduate students and postdoctoral researchers in the biomedical sciences.

Consistent with the requirements of Section 3002 of the 21st Century Cures Act (Pub L. 114-115) to develop guidance on the collection and submission of *patient experience data*, the AAMC commends the FDA on its efforts to gain a better understanding of the expertise and value patients, caregivers, and families can bring to the regulatory decision-making process and applauds the Agency's recent efforts to hold disease-specific *Patient Focused Drug Development* meetings and establish an Office of Patient Affairs. We believe these efforts will improve transparency around the FDA's patient engagement activities and increase public trust in the Agency's decision-making process.

The AAMC supports the collection and use of robust patient and caregiver data to inform its regulatory decisions and commends the FDA for soliciting feedback on the tools and methods related to the collection and use of this data. To help the Agency further these efforts, the AAMC offers the following recommendations:

I. Engagement of Patients, Caregivers, and Families in the Research Process

The FDA recognizes that patients and caregivers should be meaningfully involved throughout the medical development process both as study participants and partners and notes that this "collaborative process" will be beneficial to clinical trials and medical research. **However, for the FDA's patient engagement efforts to be a truly a collaborative process, the draft guidance would benefit by including recommendations on how to meaningfully engage patients, families, and caregivers in the development of the sampling methods and questions used to collect and analyze patient experience data.** This may also include soliciting feedback about what research questions matter to patients,

developing sampling methods tailored to patient populations that may be difficult to reach, or identifying communication pathways that will effectively reach patient populations who may not benefit from the infrastructure and technologies that facilitate communication through electronic and social media.

AAMC strongly recommends the FDA work with patients and other key stakeholders to develop a framework for using patient experience data during the study design phase to help define the research objectives and questions, which the Agency identifies as the first step in the eight-step process for "Conducting Studies about Patient Experience," and what the AAMC believes to be one aspect of the research process where patient input and expertise are most critical.

II. Ensuring Final Guidance is Useful for All Stakeholders

The FDA states that patient experience data can be collected by any persons, including patients, family members, caregivers, patient advocacy organizations, researchers, and drug manufacturers. However, it is unclear what stakeholders the FDA believes would be best served by this guidance document (e.g., researchers or patients). For example, the draft guidance provides a very detailed discussion of the qualitative and quantitative research methods and steps on how to compile, classify, interpret, analyze, and submit patient data to the FDA. However, a lay patient or individual who might be interested in collecting and submitting patient experience data may be unfamiliar with the specified research methodologies and therefore opt out of participating. **To ensure the final guidance serves its intended purpose - to facilitate the collection and integration of patient experience data into the regulatory process, it is important for this guidance (and forthcoming guidance) to be useful *for all stakeholders,* including patients, families and caregivers or other key stakeholders.**

III. Contacting and Submitting Patient Experience Data to the FDA

The AAMC recognizes that the FDA plans to issue additional guidance on how to submit patient experience data and encourages stakeholders interested in submitting patient data to the Agency to have "early interactions with the FDA," so they may obtain feedback from the appropriate review division on research design. While this feedback might be helpful, especially for stakeholders unfamiliar with the research process, the guidance does not clearly identify how individuals should get in touch with the FDA, whether through email or a specified agency contact that would direct them to the appropriate review division. We strongly encourage the FDA to solicit stakeholder input on how to best contact or communicate with the FDA including information about the specific format for which data should be submitted to the Agency. **If the process for contacting the FDA to receive feedback is different from the process for the submission of patient data, we encourage the FDA to include appropriate contact information *in this guidance*, once finalized, and all additional related guidance requesting stakeholders to obtain feedback from the appropriate FDA review division.**

AAMC also recommends that special consideration should be given to patient populations or individuals that may not have regular access to electronic technology. **Thus, the FDA should review its electronic communication strategies and may need to work with minority-serving health professional**

organizations and advocacy groups to support effective communication and outreach to underserved populations.

IV. Role of the FDA's Office of Patient Affairs

The FDA states that the intended purposes of the draft guidance it so "operationalize and standardize the collection, analysis, and dissemination of patient experience data" but the draft guidance fails to address how this data will be collected, maintained, and disseminated.

The FDA's recently established Office of Patient Affairs should play a key role in the internal coordination of the data submitted to the Agency in addition to serving as a central entry point for industry, patients, and other stakeholders.[1] The AAMC also encourages the FDA to develop strategies to ensure the engagement of diverse patient and advocate populations, and that the representatives or stakeholders engaged in FDA's patient-related activities are not solely professional advocates selected, trained, or funded by drug device and biotechnology companies. **We also strongly encourage the FDA to solicit feedback through a public workshop or issuance of guidance, on how its plans to collect, maintain and disseminate the information submitted to the Agency.**

V. Harmonization of Human Subject Protection Regulations

The AAMC appreciates that the FDA includes a brief discussion of relevant human subject protection regulations, recognizing that a full discussion of the laws that may apply to the collection methods discussed within this draft guidance are beyond its scope. The FDA notes that research subject to FDA regulations must satisfy the requirements for informed consent under 21 CFR part 50 and IRB requirements at 21 CFR part 56. However, research supported or conducted by the Department of Health and Human Services, must satisfy requirements under 45 CFR part 46, a different set of regulations also known as the "Common Rule." The FDA recommends that researchers work closely with Institutional Review Boards and Health Insurance Portability and Accountability (HIPAA) Privacy Boards to determine applicable law.

Section 3023 of the 21st Century Cures Act (P.L. 114-255, "Cures Act") requires the Secretary of HHS to harmonize the differences between these two sets of regulations by December 13, 2019, within three years from the Cures Act's enactment date. The AAMC appreciates that work is underway to further align the differences between the FDA and HHS human subject protections regulations in compliance with the Cures Act and encourages the FDA to move this process forward quickly, especially considering the recent revisions to the Common Rule which must be implemented by January 21, 2019.

We strongly encourage the FDA to finalize rulemaking or issue joint guidance with HHS and other Common Rule agencies and departments before January 2019, the timeframe which HHS will be

[1] AAMC Comment Letter on "Enhancing Patient Engagement Efforts Across FDA; Establishment of a Public Docket; Request for Comments" (Docket No. FDA-2017-N-0455, June 2017), https://www.aamc.org/download/480596/data/commentletteronpatientengagement.pdf

issuing critical guidance and clarifying information on the key changes to the revised regulations and regulated entities will be implementing those changes. Bringing the FDA's human subject regulations as close as possible to the revised Common Rule would minimize inconsistencies, reduce burdensome duplications of effort, and enhance efficiency, ultimately helping to ensure the integrity of the research and the protection of research subjects.

To the extent we can continue to help the FDA identify ways to further advance its patient engagement efforts or specific activities of the Office of Patient Affairs, whether through a meeting or broader discussion with AAMC's constituents and stakeholders, please contact me or my colleagues Philip Alberti, Ph.D. at (202) 828-0522 or Heather Pierce, JD, MPH, at hpierce@aamc or (202) 478-9926.

Sincerely,

Ross E. McKinney, Jr., MD
Chief Scientific Officer