June 12, 2017

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

Re: Docket No. FDA-2017-N-0455: Enhancing Patient Engagement Efforts Across the Food and Drug Administration

Dear Sir/Madam:

The Biotechnology Innovation Organization (BIO) thanks the Food and Drug Administration (FDA) for the opportunity to submit comments on Enhancing Patient Engagement Efforts Across the Food and Drug Administration.

BIO is the world's largest trade association representing biotechnology companies, academic institutions, state biotechnology centers and related organizations across the United States and in more than 30 other nations. BIO members are involved in the research and development of innovative healthcare, agricultural, industrial, and environmental biotechnology products.

The Food Drug and Safety Innovation Act (FDASIA) and the authorization of the fifth Prescription Drug User Fee Agreement (PDUFA V) enacted in 2012, directed FDA to develop and implement strategies to solicit the views of patients to inform drug development and regulatory decision activities. Specifically, FDA was directed to encourage participation of patient representatives as special government employees and to conduct a series of public meetings to solicit patients' perspectives on their condition and available therapies to treat their condition. These activities have served to provide FDA and industry with a better understanding of what matters most to patients and their families impacted by certain diseases.

The 21st Century Cures Act enacted in 2016 and the proposed reauthorization of the sixth Prescription Drug User Fee Agreement (PDUFA VI) build upon the FDASIA and PDUVA V efforts to incorporate patient perspectives into regulatory decision making by embedding experts within review divisions and advancing the science of collecting, analyzing, interpreting, and integrating disease state patient information into drug development and regulatory decision making processes (e.g., benefit risk assessments and incorporation of patient driven information in the label of a product). The resources, processes, and mechanisms established to meet these objectives include:

- Enhancing FDA's staff capacity by embedding experts within review teams (clinical, statistical, psychometric, health economics/outcomes experts) to facilitate the development and use of patient-focused methods (e.g., clinical outcomes assessment (COA) such as patient-reported outcomes (PROs)) to inform drug development and regulatory decisions.
- The development of a series of guidance documents over a 5 year period that focus on approaches and methods to bridge the initial patient-focused drug development

meetings of PDUFA V to fit-for-purpose tools to collect meaningful patient and caregiver input for use in regulatory decision making.
- Build on the implementation of PDUFA V structured benefit-risk assessment by collecting patient input at defined points throughout the drug life-cycle for inclusion in product benefit-risk assessment.
- Maintain a repository of patient-focused drug development tools for stakeholders.
- Establish a process for stakeholders to develop and submit proposed draft guidances relating to patient experience data to FDA.
- Require FDA, upon approval of a drug or biologic, to make public, a brief statement regarding patient experience data and information, if any, that was submitted and reviewed as part of the application.

The Role of OPA

BIO applauds the Agency for its continued commitment to advancing the science of integrating patient perspectives into drug development and regulatory review processes. We also support FDA's proposed establishment of a central Office of Patient Affairs (OPA) to provide a more transparent, accessible, and positive experience for patient communities. Such a centralized office could serve to support and coordinate patient engagement activities across medical product centers as well as other offices that engage with patients and their advocates on matters pertaining to medical products. There is a need for patient engagement infrastructure that benefits all Centers and OPA should be ideally situated to take on this work.

The Federal Notice proposes that the responsibilities of OPA include offering a single, central entry point to the Agency for the patient community; providing triage and navigation services for inbound inquiries from patient stakeholders; hosting and maintaining robust data management systems that would incorporate and formalize knowledge sharing with FDA by patient stakeholders and FDA's relationships with patient communities, as well as; developing a scalable and forward-looking platform for communicating with patient stakeholders. The Notice also states that OPA would be directly accountable to the Medical Product Centers through clear governance structures.

BIO agrees that the proposed OPA could serve to receive inquiries about and better coordinate patient focused activities occurring across the Agency (e.g., FDA Patient Representative Program, Professional Affairs and Stakeholder Engagement and others[1, 2, 3, 4]). It could also play a transformative role in developing a forward looking platform that enables the patient community and the broader scientific community to more easily obtain

[1] Externally-led Patient-Focused Drug Development Meetings.
https://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm453856.htm

[2] The Voice of the Patient: A Series of Reports from FDA's Patient-Focused Drug Development Initiative.
https://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm368342.htm

[3] Enhancing Benefit-Risk Assessment in Regulatory Decision-Making.
https://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm326192.htm

[4] Patient-Focused Drug Development: Disease Area Meetings Planned for Fiscal Years 2013-2017.
https://www.fda.gov/forindustry/userfees/prescriptiondruguserfee/ucm347317.htm

information about patient focused drug development activities (e.g., publicly available information about patient perspective data submitted regarding the impacts of specific disease, Voice of the Patient Reports, relevant public meetings). Enhancing transparency on how patient perspectives are integrated could build greater trust between all stakeholders, and help patients better understand how they can be involved in new ways.

OPA could also serve as a valuable resource for patients, patient advocacy organizations, and the larger scientific community. Patients and patient advocates have suggested that it can be difficult to navigate the FDA, particularly when trying to identify the appropriate point of contact for different types of inquiries. In addition to patients and patient advocacy groups being able to directly engage with relevant Review Divisions, the proposed Office could serve as a centralized point of entry to the Agency, consolidating relevant information, resources, and contact information in one clear and easily accessible portal. OPA could also be used as an educational resource for patients and patient organizations to raise awareness of drug development process. For example, OPA could assist patient stakeholder organizations seeking to submit draft guidance relating to patient experience data; and access to information about Advisory Committee meetings.

In addition to supporting patients, OPA should ensure timely communication and transparency with sponsors by helping inform an overall approach to be taken to incorporate patient perspective data into an Investigational New Drug Application (IND) and Marketing Application. Additionally, OPA should support and coordinate with pharmaceutical sponsors that seek to include the patient voice in drug development as well as benefit-risk assessments. OPA should assist sponsors seeking to determine what, if any, patient experience data was submitted or reviewed as part of an application and how it will be or was used to inform regulatory decision-making. It is imperative that the Agency, sponsors, and patients engage collectively on efforts to improve and enhance patient engagement via a consistent overarching framework.

Governance

It is critical, however, that any of the activities conducted by OPA are designed to be additive to and not supplant the crucial aspects of the proposed PDUFA VI agreement. Specifically the provision that directs FDA to embed experts within the review divisions to facilitate development as well as use of patient-focused methods to inform drug development and regulatory decisions. More information about how OPA would be "directly accountable to the medical product centers through clear governance structures" is needed to ensure that the objectives and activities directed by 21st Century Cures and PDUFA VI are implemented. OPA should not divert critical activities and resources away from the review divisions as they should retain overall responsibility for evaluating patient input. OPA could also, for example, gather and make publicly available "performance metrics" regarding its activities and the extent to which it is implementing its mission effectively, thus providing information and transparency to all stakeholders.

Data Management Systems

BIO requests that the Agency provide more information about what is meant by "hosting and maintaining robust data management systems that would incorporate and formalize knowledge share with FDA by patient stakeholders and FDA's relationships with patient communities." The 21st Century Cures Act and the proposed PDUFA VI agreement direct

FDA to provide guidance on how to collect patient perspective data in a manner that can inform regulatory decision making. The development of a robust management system may be premature as guidance on methodologies for the collection, use, and communication of patient perspective data is not yet available.

In closing, BIO commends the Agency's commitment to advancing the systematic integration of patient perspective data to inform drug development and regulatory decision-making. Enhancing staff capacity and developing guidance and tools that will better enable the incorporation of the patient voice throughout the lifecycle of product will support our shared goal of ensuring that medicines are truly meeting the needs of patients and their families.

BIO appreciates this opportunity to submit comments on Enhancing Patient Engagement Efforts across the Food and Drug Administration. We would be pleased to provide further input or clarification of our comments, as needed.

Sincerely,

/S/

Cartier Esham, Ph.D.
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Biotechnology Innovation Organization