United States Food and Drug Administration
Generic Clearance: Collection of Qualitative Feedback on FDA Service Delivery
OMB Control Number 0910-0697
Gen IC Request for Approval

Title of Gen IC: Small Group Discussions with Applicants and Other External Stakeholders Regarding Clarity, Understandability, and Usefulness of FDA's Benefit-Risk Framework (BRF)

1. Statement of Need:

Background:
Fifth Authorization of the Prescription Drug User Fee Act (PDUFA V)

FDA committed under PDUFA V to integrating a structured benefit-risk framework into the human drug and biologic review program, and also committed to conducting an evaluation of this implementation.

This evaluation was required to consider the utility of the framework in facilitating decision-making and review team discussions across disciplines, risk management plan decision-making, training of new review staff, and communicating regulatory decisions. The evaluation also considered the degree to which the framework supports or facilitates balanced consideration of benefits and risks, which is a more consistent and systematic approach to discussion and decisionmaking, and communication of benefits and risks. As part of this evaluation, a 3rd party contractor conducted a review of FDA documents and conducted interviews with both internal staff and external stakeholders (industry, patients, patient advocates, health care providers (HCPs), and academics).

Results of the PDUFA V evaluation were discussed at a public meeting on September 18, 2017), and the PowerPoint slides are available at https://www.fda.gov/industry/prescription-drug-user-fee-amendments/public-workshops-benefit-risk-considerations. The evaluation and public meeting were considered to have met the PDUFA V commitment to integrate the BRF into review and conduct an evaluation of the BRF. The findings from the evaluation were used to improve upon the FDA's implementation processes, templates, and staff trainings on the BRF.

Sixth Authorization of the PDUFA Act (PDUFA VI)

FDA committed under PDUFA VI to furthering the Agency's implementation of structured benefit-risk assessment in the human drug review program, including the incorporation of the patient's voice in drug development and decisionmaking. Results of the PDUFA V evaluation helped inform FDA's development of a benefit-risk implementation plan, available at https://www.fda.gov/media/112570/download.

As part of PDUFA VI, FDA also committed to conducting a second evaluation of this implementation, to state that this evaluation must assess how reviewers across the organization apply the BRF and identify best practices in use of the BRF. It also states that evaluation of the BRF implementation conducted in PDUFA V will serve as a baseline for this PDUFA VI assessment.

To fulfill the PDUFA VI commitment, FDA's Center for Drug Evaluation and Research (CDER) is assessing the BRF used to communicate regulatory decisions about new molecular entity (NME)

new drug applications (NDAs) and original biologics license applications (BLAs). For each application, the Framework provides a structure for documenting considerations related to a drug's: (1) therapeutic area (analysis of the condition and the current treatment options); and (2) product-specific benefits and risks, as well as any potential risk management activities. The purpose of the BRF is to convey the information numbered previously in a succinct, standard format to help both internal and external stakeholders better understand the evidence and reasoning that supported FDA's regulatory decision on the application.

The evaluation will examine BRFs prepared for NME NDAs and original BLAs that were received between June 1, 2020 and May 31, 2021 (an estimated 50 applications). As part of this assessment and to meet our PDUFA VI commitment to use the PDUFA V evaluation as a baseline FDA is seeking input from applicants and other external stakeholders on the clarity, understandability, and usefulness of these Frameworks and ways the BRF template can be improved to better meet the needs of applicants and other external stakeholders. Two target audiences are applicants and other external stakeholders who are familiar with the products being discussed in the relevant applications. Using the PDUFA V evaluation (stated in the *Fifth Authorization of the Prescription Drug User Fee Act (PDUFA V* section above) as a baseline, FDA plans to include a similar set of external stakeholders (patients, patient advocates, HCPs, and academics). With respect to applicants and other external stakeholders, CDER seeks to assess the extent to which the Framework provides a clear understanding of the reasoning behind FDA's regulatory decisions on the applications. This data collection will enable a direct comparison with the outputs of the previous evaluation, and will fulfill our PDUFA VI commitment to conduct an evaluation using the PDUFA V evaluation as a baseline.

2. **Intended Use of the Information:**

Data collected under this set of small group discussions will help FDA improve the implementation of the BRF moving forward. Specifically, understanding the extent to which the frameworks developed by FDA were clear, understandable, and useful to applicants and other external stakeholders will assist FDA in modifying the framework to meet the needs of these audiences.

This data collection is also part of a larger project that involves a detailed review of the BRF documents created by FDA staff and in-depth small group discussions with FDA staff on implementing and integrating the BRF into review processes. Information collected from applicants and other external stakeholders will complement internal perspectives on the framework to provide a broad assessment of the Framework's integration and implementation.

3. Description of Respondents:

There are two main respondent groups: (1) applicants whose NME NDAs or original BLAs were received between June 1, 2020 and May 31, 2021; and (2) non-applicant external stakeholders who may use the information in the BRF.

FDA had expected that approximately 50 NME NDAs and original BLAs would be received between June 1, 2020 and May 31, 2021. FDA's contractor for this work, Booz Allen Hamilton (Booz Allen), will perform small group discussions with applicants for those applications that were approved. Booz Allen will also hold small group discussions with applicants who receive a

Complete Response (CR) letter[1] if they receive a BRF with the CR letter. There will be a total of 25 applicant small group discussions, each of which will include up to 3 representatives from applicant teams responsible for these applications (total number of participants ≤ 75). In scheduling small group discussions, Booz Allen will request to hold small discussion groups with individuals most likely to review or have a need for the information in the BRF (e.g., Directors of Regulatory Affairs, Chief Executive Officers for smaller companies).

For other external stakeholders, FDA has asked Booz Allen to meet with three types of stakeholders: (1) patient groups; (2) HCPs; and (3) patient advocates. These groups will be further grouped by therapeutic area (e.g., oncology, infectious disease, endocrinology, allergy/immunology) or sector within a therapeutic area where appropriate. There will be a total of 50 non-applicant/external stakeholder small group discussions that will include up to 5 representatives from a given stakeholder group for a given therapeutic area or sector: 50 groups in total (total number of participants ≤ 250). In scheduling small group discussions, Booz Allen will request to meet with individuals most likely to review or have a need for the information in the BRF.

4. **Type of Collection:**

[X] Small Discussion Group

FDA will collect this information through a series of small discussion groups. FDA has contracted with Booz Allen to perform these small group discussions and plan to conduct the discussions via video conference.

Given the ongoing COVID-19 pandemic, any face-to-face small group discussions would only be conducted if and when it is safe to do so. Small group discussions are expected to last up to 90 minutes each.

5. **Confidentiality of Respondents:**

As noted above, FDA has contracted with Booz Allen to perform these small group discussions; FDA staff will not participate in any small group discussions. Booz Allen will handle the processing of data and information from these small group discussions and will not provide FDA with raw notes from the small group discussions that contain information on who participated in the small group discussions. Furthermore, Booz Allen will not associate information provided by applicants or external stakeholders with a specific participant.

FDA will know what applicant entities are within the scope of these small group discussions because, by definition, the applicant entities have submitted applications to the Agency; FDA will not know which applicant entities accepted or declined small group discussions or which individuals within the applicant entities participated in small group discussions. FDA may suggest other external stakeholder groups for the contractor to include, but will not know exactly what groups (or individuals within groups) are participating. In addition, FDA will receive updates from the contractor on the status of small group discussions (number scheduled, number conducted, common themes at aggregate level), but in no case will such updates include identifying

[1] A CR letter is an action by FDA indicating to the applicant that the review cycle has been completed, but that FDA determined the application was not ready for approval. The CR letter allows for the possibility of resubmission of the application at a later date.

information about participants. FDA fully recognizes that applicants and other external stakeholders have the right to refuse to participate in small group discussions.

The discussion scripts will include the following text:
"Your participation / nonparticipation is completely voluntary, and your responses will not have an effect on your eligibility for receipt of any FDA services. In instances where respondent identity is needed (e.g., for follow-up of non-respondents), this information collection fully complies with all aspects of the Privacy Act and data will be kept private to the fullest extent allowed by law."

6. Amount and Justification for Proposed Incentive:

Is an incentive (e.g., stipend, reimbursement of expenses, token of appreciation) provided to participants? [ ] Yes [X] No

7. Questions of a Sensitive Nature:

No questions of sensitive nature are being asked.

8. Description of Statistical Methods

No statistical methods are being used.

9. Burden:

Type/Category of Respondent	**No. of Respondents**	**Participation Time (minutes)**	**Burden (hours)**
Applicant/Small Group Discussions	75	90	113
Non-Applicant/External Stakeholders Small Group Discussions	250	90	375
Totals	325		488

10. Federal Cost:

Booz Allen has already been awarded a contract to conduct an evaluation of FDA's BRF. No cost to the federal government will be incurred beyond this already awarded contract.

11. **Date(s) to be Conducted:**

FDA will collect this information between February 2022 and September 2022. The end point of the collection corresponds to the date we will incorporate data from the small group discussions into a final report for the project.

12. Requested Approval Date: February 2022

13. FDA Contacts:

Program Office Contact	FDA PRA Contact
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14. **Certification:** In submitting this request, I certify the following to be true:

a) The collections are voluntary;
b) The collections are low-burden for participants and are low-cost for both the participants and the Federal Government;
c) The collections are noncontroversial;
d) Personally identifiable information (PII) is collected only to the extent necessary and is not retained; and
e) Information gathered will not be used for the purpose of substantially informing influential policy decisions.