PDUFA Reauthorization Stakeholder Meeting
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Good afternoon everyone. I'd like to thank the FDA for allowing the Alliance for Aging Research to comment on some of our priorities for the sixth reauthorization of PDUFA. I first want to acknowledge that user fees are critical source of funding for the agency and our suggestions for this reauthorization are not intended to alter the program's primary purpose of ensuring the effectiveness and timeliness of the drug review process.

The following are enhancements that we believe can improve the way in which patient advocacy groups and industry interact with the FDA to advance more meaningful drug development and allow the Agency to continue demonstrating leadership in shaping methods that are most beneficial for engaging patients and caregivers earlier in the research and development process.

On behalf of the Alliance, I commend CDER for the success of the Patient-Focused Drug Development (PFDD) Initiative initiated under PDUFA V. The PFDD meetings held to date have produced impactful testimony of patients for medical reviewers who will be evaluating

products for diseases and conditions they may not have first-hand experience with; they have provided FDA with a better understanding of diseases and conditions for which there are no current treatments; and they have identified goals patients have for effective treatment that in many cases relate more to quality of life than length of life.

In addition to these direct benefits to reviewers and the review process, we feel that the PFDD Initiative is responsible for one of the most transformational shifts in therapeutic development. It focused the attention of industry back onto the complex needs of patients and placed increased value on the voices of patient and caregivers in defining outcomes that are meaningful.

The Alliance has had the opportunity to work with the Agency for the last 10 years on issues related to clinical trial deign in Alzheimer's disease through our role as chair of the Accelerate/ Cure Treatments for Alzheimer's Disease Coalition. More recently we have engaged on clinical development issues for sarcopenia through the Aging in Motion Coalition. Because of these positive experiences, we were not surprised by FDA's commitment to patient focused drug development.

We are heartened to see that industry and other patient advocacy groups are embracing PFDD after the creation of FDA's initiative under

PDUFA V. There are coalitions, consortia and individual efforts launching in increasing numbers to conduct PFDD work. For all of these to positively contribute to the R&D process, it is important for FDA to provide additional information on how to gather and employ patient input effectively. We encourage FDA to resist efforts to privatize PFDD and we hope that you can remain committed to leading the effort.

The Alliance supports a proposal raised in the October 21, 2015 PDUFA VI Regulatory Development Tools Working Group meeting between FDA and industry to have an information repository created by the FDA that catalogs PFDD activities the Agency is aware of to help guide patient organizations, sponsors, and FDA staff. This repository could demonstrate methods for patient engagement that are acceptable to the Agency, foster collaboration, and reduce duplication of effort that often occurs in an information vacuum. We support this and another proposal made during the October 21 meeting to develop a series of guidances and additional public workshops on PFDD.

To further these two proposals, we urge FDA to focus guidance, and at least one workshop, on building out the principles that underlie their Roadmap to Patient Focused Outcome Measurement. If FDA were to explicitly lay out elements of this Roadmap in more detail, it could allow the Roadmap to serve as a diagnostic tool that outside stakeholders can

run on particular disease areas to identify evidence gaps that should be filled by patient organizations, industry, or research consortia. We believe FDA should also be provided with resources under PDUFA VI to expand its Critical Path Innovation Meeting (CPIM) program. The CPIM is an existing mechanism that allows groups to actively engage with FDA on PFFD activities that are aimed at developing endpoints or outcome measures that are in the conceptualization phase. Having this type of early conversation is important in planning development of new endpoints, including biomarkers.

Other PFFD-relevant guidances that should be considered in PDUFA VI include new guidance on performance outcome measures, observer reported outcome measures, and clinician reported outcome measures. It is difficult to develop these instruments without guiderails from the agency. We also suggest a follow-up public workshop on the feasibility and reliability of incorporating PROs in clinical trials. Even with PRO guidance and a preliminary public workshop on PROs, challenges exist in employing PROs effectively for complex degenerative diseases, like Alzheimer's disease.

Lastly, we were pleased with the emphasis in PDUFA V on expanding the availability of clinical trials data on age, sex, and ethnicity. CDER's public process for developing an action plan on subpopulations and

placing snapshots of data that is available from clinical trials on FDA's website was a positive first step in trying to address the current lack of diversity in clinical trials. It is encouraging that the October 21, 2015 PDUFA VI Regulatory Development Tools Working Group meeting included discussion of at least one additional workshop on patient engagement in clinical trials, recruitment issues, participant retention and trial testing requirements. We support this proposal and hope that further discussion with industry results in resources allowing FDA to increase activity on clinical trial participation and diversity.

The Alliance looks forward to continuing to offer feedback to FDA on proposed enhancements as the reauthorization process moves forward. Thank you again for the opportunity to comment today, and for your attention.