PDUFA VI Public Meeting
Remarks of Cynthia A. Bens
Alliance for Aging Research

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Panel 1: Pre-Market Review and Post-Market Safety

Good morning everyone and thanks to the FDA for inviting me to participate on this panel.

I am Cynthia Bens and I am vice president of Public Policy at the Alliance for Aging Research. I also serve as executive director of the Alliance-led Accelerate Cure/Treatments for Alzheimer's Disease and Aging in Motion coalitions.

I only have three minutes, so I will not go into too much background on these groups, except to say that we have spent the last 10 years working directly with the FDA, patients, caregivers, and industry on issues affecting clinical trials for Alzheimer's disease and sarcopenia in older adults.

These are two incredibly challenging areas for drug development. Our coalitions have played a central role in facilitating meaningful communication between regulators and all stakeholders involved in the drug development process. Through this work, we have come to understand how critical early and ongoing communication with FDA is in shaping successful clinical programs.

We believe that PDUFA VI makes crucial changes to FDA's internal and external communications. My comments are limited to enhancements in the PDUFA VI

Commitment Letter that will advance regulatory science and help expedite the drug development process.

First and foremost, we **support the utilization of user fees under Section I #1 to maintain dedicated staff within CBER and CDER, focused on improved communication between FDA and sponsors during drug development.**

We were encouraged to learn that these staffs will provide ongoing training to the review divisions on best practices for communicating with sponsors, while at the same time working to better facilitate responses to general questions from sponsors on drug development and to ensure timely resolution of issues with specific INDs.

We also support the use of fees for an independent assessment of current communications practices and a public workshop to examine the results of the assessment with an eye toward issuing updated guidance on FDA-sponsor communications, if it is necessary.

The second provision we support under **Section I is #3: early consultation on the use of new surrogate endpoints**. The meetings described in this section will allow companies to engage with FDA's senior leadership on the feasibility of using a surrogate endpoint that has not previously been used as the basis for an approval and uncover any knowledge gaps that require attention.

While we do not yet have a qualified biomarker for use as a surrogate in either disease area we work in, we know that clinical trials utilizing surrogate endpoints will be increasingly important as drug development moves toward intervention

earlier in the course of disease. So, establishing this dedicated process for meetings on surrogates that can occur as early as the end of Phase 1 is a priority for us.

The third provision that is important to the Alliance **is Section I #5, advancing the development of combination products.** It is expected that the number of products in development that are combination products will increase to almost 40 percent. Because of this significant increase, it is important that FDA is able to ensure that drug-device and biologic-device combinations don't face unexpected delays in the review process.

We are pleased that PDUFA VI will provide funding for capacity building, staff training, and performance goals for CDER- and CBER-led combination product activities.

The final provision we support in Section I is the addition of **user fees for the Breakthrough Therapy Program.** During the kick-off meeting last year, I spoke about the strain the creation of this pathway placed on the agency. This is because it is so resource-intensive, and it did not come with additional funding under PDUFA V.

PDUFA VI would allow for 36 FTEs to assist with this expedited approval pathway. Breakthrough has been incredibly successful in delivering truly innovative products for serious and life-threatening conditions. We will be glad to see it continue and hope it will be a viable pathway for Alzheimer's disease and sarcopenia treatments in the near future.

Thank you all for your attention and I look forward to answering any questions.

Panel 3: Administrative Enhancements: IT, Hiring, & Financial

Good afternoon, everyone.

Many thanks again to FDA for inviting me to be on this panel.

For those of you who did not tune in for the morning session, I am Cynthia Bens, vice president of Public Policy at the Alliance for Aging Research, and I also serve as executive director of the Alliance-led Accelerate Cure/Treatments for Alzheimer's Disease and Aging in Motion coalitions.

I am going to spend the next few minutes sharing our views on one of the most critical components of PDUFA VI: **Section III of the commitment letter, improvements to FDA hiring and staff retention.**

We believe that FDA can only be successful in carrying out all of the activities we care about in PDUFA VI if it has the best and brightest people in the right positions and continued stability in its workforce. To do this, FDA needs to compete on a level playing field with the private sector and other federal agencies for highly skilled individuals.

We recognize that FDA lacks a number of tools that would allow it to maintain a robust hiring and retention function, which is why the Alliance for Aging Research pushed for a focus on hiring during PDUFA VI and during the development of H.R. 6, the 21st Century Cures Act, and the Senate Innovation Initiative.

We are really pleased to see that industry is putting resources toward hiring and retention processes at FDA, and there are several proposed enhancements under Section III that we would like to call attention to.

The first is **Section III A, modernization of FDA's hiring system.** Two highlights of this section are FDA's commitment to reviewing and cataloguing existing position announcements in order to implement a comprehensive online position classification system and its planned efforts to transition away from time-limited individual position vacancy announcements. Shifting to common vacancy announcements for use by multiple offices with continuous posting will create the greatest opportunity for applicants with key scientific and technical expertise to apply for positions regularly needed across FDA's drug review programs. We think that both of these are positive steps forward.

The second noteworthy section is **Section III B, augmentation of hiring staff capacity.** Because of chronic challenges in retaining and recruiting enough human resources professionals, FDA needs to supplement in-house staff with external expertise. PDUFA VI would allow the Agency to retain a qualified hiring contractor.

Employing this contractor will assist FDA in successfully meeting goals for recruitment of human drug review program staff. The contractor will conduct a comprehensive review of current hiring processes in an effort to identify capabilities leading to success as well as potential problems or delays in hiring within the drug review program. FDA and industry will regularly assess progress in hiring and retention throughout PDUFA VI, but a welcome opportunity for

stakeholders is that they will be able weigh in on FDA's progress through a minimum of three public meetings between 2017 and 2022.

The third section I would mention is **Section III C, establishing a dedicated unit within the Office of Medical Products and Tobacco with a continuous focus on hiring and staffing issues** so that FDA can keep pace with scientific and technologic advances. The unit would proactively reach out to qualified candidates and competitively recruit to fill vacancies. It would also analyze compensation and other factors that affect retention of key staff on an annual basis.

Last, but not least, I would like to discuss **Section III D, FDA setting hiring goals for PDUFA VI.** This section demonstrates the agency's commitment to accountability and a desire to measure progress in targeting hires within the human drug review staff. We applaud them for taking this step.

We believe that PDUFA VI will ensure that FDA can hire and retain a strong scientific and medical workforce to advance its mission to protect and promote public health, so the Alliance for Aging Research is supporting all of the provisions under Section III.

I'll stop there. Thank you all again for your attention and I look forward to your questions.