

**Testimony of Cynthia A. Bens, Vice President of Public Policy
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**United States House of Representatives
Energy and Commerce Committee, Subcommittee on Health
Hearing on “Examining FDA’s Medical Device User Fee Program”**

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Mr. Chairman, Ranking Member Green, and Members of the Subcommittee:

It is an honor and a privilege to speak with you today about the reauthorization of the Medical Device User Fee Act (MDUFA) program, on behalf of the Alliance for Aging Research.

I am Cynthia Bens, the Vice President of Public Policy at the Alliance. The Alliance for Aging Research is the leading non-profit organization dedicated to accelerating the pace of scientific discoveries and their application to improve the experience of aging and health. We believe that advances in research help people live longer, happier, more productive lives and reduce health care costs over the long term.

Most of us are keenly aware that our population is aging at an unprecedented rate. Ten thousand Baby Boomers are turning 65 each day. This is up from 6,000 per day just 6 years ago. People age 85 and older are the fastest growing segments of our population. Right now, approximately 10 percent of the U.S. population is age 80 or older. This 80+ age group will reach 30 percent of the U.S. population by 2050.

Many older adults today are fortunate to experience better health as they age than previous generations. But the truth is that most older adults still face significant periods of illness and disability later in life, often from multiple chronic conditions that require complex care

management. They develop one or more forms of cardiovascular disease, cancer, diabetes, bone and joint degeneration, muscle wasting, vision and hearing loss, neurological diseases, and incontinence.

In our view, the need for innovative medical devices that help diagnose and respond to the physical declines people face as they age has never been greater. We believe that we will only realize the benefits of these medical technologies if the U.S. Food and Drug Administration (FDA) has access to the resources and expertise necessary to evaluate them, the medical device industry is certain that their products will be assessed in a timely manner, and patients are at the center of new product development.

For more than a decade, the Alliance for Aging Research has been working directly with the FDA, other patient advocates, researchers, and industry on ways to streamline the regulatory process for the benefit of older adults. We understand that user fees play an essential role in maintaining FDA review processes that efficiently deliver safe and effective medical devices to patients who need them, and that is why we engage in the MDUFA reauthorization process.

Historical Perspective on the MDUFA Program

Prior to the third reauthorization of MDUFA, patient and consumer organizations were not able to engage in the negotiations between the FDA and industry. Thanks to you and your colleagues in Congress, the Alliance for Aging Research and other groups were represented throughout the patient/consumer stakeholder consultation phase leading up to third reauthorization of MDUFA. We had an opportunity to provide feedback to the FDA as negotiations were taking place and propose enhancements to be included in the final commitment letter that emerged from the negotiations.

Going into monthly consultation meetings with the FDA, we had two goals for MDUFA III. The first was to make sure that the Center for Devices and Radiological Health (CDRH) had sufficient

resources under MDUFA III to carry out timely reviews. The second was to secure support for a process through which CDRH would include patient views on the benefits and risks of devices during product reviews. After several monthly meetings with the agency, industry's desire for a more predictable and collaborative review process came into focus and the FDA expressed a desire to address personnel issues within CDRH. These challenges seemed to be impeding device review and delaying patient access.

MDUFA III allowed the application of user fees to help cultivate existing CDRH staff and to recruit and retain new talent. To strengthen the FDA's device review capacity, fees were aimed at hiring additional reviewers and reducing the ratio of reviewers to managers. MDUFA III also continued the FDA's third-party review program. This program is intended to reduce the review burden for lower-risk devices, by allowing FDA to leverage external experts.

To address the need for more predictability and collaboration in the review process for devices, MDUFA III included provisions aimed at improving formal and informal communication between the FDA and device makers. FDA took an important step by developing a formalized approach to address specific questions from industry prior to their submission of applications for products. FDA also implemented revised submission acceptance criteria, including an updated "refuse to accept" checklist, by which FDA would evaluate submissions to ensure that agency resources were focused on reviewing complete applications.

FDA's creation of a process for incorporating patient views on the benefits and risks of medical devices was an Alliance priority under MDUFA III. CDRH was a leader among regulators in aggressively pursuing a transparent and structured benefit-risk framework. Finalizing a benefit-risk guidance for devices was one of CDRH's first actions in MDUFA III implementation. The benefit-risk guidance, first issued by FDA in 2015, broadly defines the benefits they are interested in understanding. The type of benefit CDRH specifically calls out are not just a device's impact on clinical management of a disease and patient health, but also patient satisfaction, improvement in quality of life, improvement in function, reduction in lost function,

reduction in probable mortality, and symptom relief. For diagnostics, benefit could be assessed according to the public health impact, the ability to identify a specific disease and potentially prevent its spread, predicting future disease onset, providing earlier diagnosis of diseases, or identifying patients more likely to respond to a given therapy.

The benefit-risk guidance also laid out the ways in which CDRH assesses the magnitude of benefit, the probability of a patient experiencing benefit, and the duration of benefit. The guidance provides details, some examples, and a copy of the worksheet that reviewers use in their benefit-risk determinations.

Benefit-risk calculation is discussed frequently but there is the potential for this type of exercise to be more tokenism than substance. We feel that CDRH got the substance of the patient experience right and we think that is because they actively engaged with the patient advocacy community to best characterize disease severity and unmet need from the start.

Recognizing that many review process improvements were instituted through MDUFA III, the Alliance for Aging Research sought further support for CDRH's workforce, expansion of patient-centered medical device development, and the utilization of real-world evidence by CDRH in MDUFA IV.

MDUFA IV Agreement Benefits to Patients

The Alliance for Aging Research was fortunate to offer patient perspectives to the FDA through monthly stakeholder consultations and public meetings held over the last year as the agency negotiated the MDUFA IV agreement. We strongly support the continuation of this user fee program. MDUFA IV contains critical commitments and funding for the FDA that will benefit patients. We are pleased that the reauthorization of the user fee agreements is a priority for this Committee. We would like to call your attention to the following sections of the agreement that we offered comment, during the stakeholder meetings.

I.) Supporting CDRH Workforce

MDUFA IV will lead to significant reductions in the time it takes the FDA to review the most common types of medical device applications. This will not only benefit industry, but also accelerate patient access. Under MDUFA IV, the FDA has committed to reduce the days for review of 510 (k) applications and for premarket approval (PMA) applications. FDA also set goals for reviewing *De Novo* applications. The number of *De Novo* requests has increased steadily since the pathway was created. The limited resources currently available to the agency for *de novo* requests have resulted in missed target dates for review in all but 40 percent of cases. Section II. E of the MDUFA IV agreement specifies that the agency set a goal of reviewing 70 percent of *de novo* requests on time by FY 2020.

Having expert FDA staff to carry out user-fee-funded activities is paramount. Without the necessary number and types of staff, the FDA will not be able to meet the ambitious performance goals for which the MDUFA IV resources are intended. Problems with FDA recruitment and hiring have existed for years because the agency lacked hiring processes and pay scales that were competitive with the private sector. The 21st Century Cures Act included some positive provisions to help FDA attract and hire new senior staff, but MDUFA IV provides CDRH with needed funding to hire across medical device review activities and cultivate existing staff. Specifically, Section III, B. of the MDUFA IV agreement, permits CDRH to apply user fees for the improvement of its scientific and regulatory review capacity. With these fees, CDRH intends to increase the retention rate of high-performing supervisors, reduce the ratio of review staff to supervisors, hire new device application reviewers, and utilize recruitment support to augment existing human resource services.

The Alliance for Aging Research is supportive of Section IV. E of the MDUFA IV agreement that seeks to bolster the third-party review program within CDRH. We advocated for the use of MDUFA III fees for the third-party review program so that CDRH's staff would have more time

to devote to higher-risk device applications. It is our understanding that third-party review continues to be valuable for lower-risk devices, but the program requires improvements to make it more efficient. We are glad that CDRH continues to have the resources and flexibility to employ outside experts as needed under MDUFA IV and that there will be improvements made to the third-party review program to ensure its integrity.

II.) Expanding Patient-Centered Medical Device Development

The Alliance for Aging Research applauds the FDA for fostering the use of patient preference information in the review and approval of medical devices. Of late, industry has begun including patient-centered endpoints in development programs, signaling a growing interest by industry to employ patient-reported outcomes in device trials with more regularity. FDA has responded by drawing patient representatives earlier into the device review process, developing a systematic benefit-risk framework for the evaluation of new devices, and creating a Patient Engagement Advisory Committee.

Section IV. F of the MDUFA IV agreement details activities that CDRH will take to further advance patient input and involvement in the regulatory process. CDRH will develop scientific expertise and expand staff capacity to respond to device submissions containing publicly available and validated, patient preference information or patient reported outcomes. This section also calls for public meetings to discuss approaches for incorporating patient preference information and patient reported outcomes as evidence in device submissions, as well as other methods of advancing patient engagement. CDRH will also explore ways to use patient input to inform clinical study design and reduce barriers to patient participation by facilitating recruitment and retention. The MDUFA IV agreement calls on the FDA to identify priority areas in which patient preference information could inform regulatory decision making and requires publication of these priorities in the *Federal Register*.

III.) Utilizing Real-World Evidence

The Alliance sought the application of MDUFA IV resources to elevate CDRH's ability to further real-world evidence generation for the purposes of informing regulatory activities. We believe that the collection of data generated through routine clinical care can help broaden our understanding of how products are working in the real world, support the incremental process of medical device development, and lead to optimal care.

Under Section IV. H of the MDUFA IV agreement, CDRH can utilize user fees to hire staff with expertise in the use of real-world evidence and establish a Coordinating Center for the National Evaluation System for health Technology (NEST). NEST will link health claims, electronic records, and registry data. In the future, these activities have the potential to decrease the number of stand-alone clinical trials, increase enrollment efficiencies, and make patient follow up less burdensome.

With MDUFA IV funds, the NEST Coordinating Committee will undertake a pilot program to explore the usability of real-world evidence for determining expanded indications for device use, new device approval, and device malfunction reporting. The NEST pilot program is particularly meaningful for our organization since older adults are not adequately represented in most clinical studies.

The Alliance for Aging Research requests one change to the MDUFA IV agreement. Section IV. H. states that "Industry representation on the NEST governing board will make up at least 25 percent of the governing board membership." MDUFA IV generally references anticipated representation of the patient community on the NEST governing board. We believe that the enacting legislation should detail the composition of the remaining 75 percent of the governing board and include representatives of patient populations most likely to be affected by increased utilization of real-world evidence (e.g. the elderly, those with multiple chronic conditions, women, etc.). If patient preference is truly a priority for the FDA and industry,

representation by patient representatives on the NEST governing board should be more clearly outlined.

Conclusion

As mentioned previously, the Alliance for Aging Research supports the continuation of the medical device user fee program through the negotiated MDUFA IV agreement. The Alliance advocates for increased overall funding of the FDA with strong emphasis on finding the right balance between user fees and appropriated funding. We think that the size of the proposed fees within the MDUFA IV agreement is necessary to increase the efficiency of regulatory processes, reduce the time it takes to bring safe and effective medical devices market, and put patients at the heart of medical product development.

Despite the opportunities afforded by MDUFA IV, we are all in jeopardy if the FDA's budget authority remains flat or is significantly reduced in the coming fiscal year. As you are aware, not all FDA activities can be supported through user fees. Crucial safety and surveillance activities as well as oversight of over-the-counter medications and other products, fall outside of the user fee programs. While FDA appropriations are not under the jurisdiction of this Committee, it is our hope that you will join us in calling for sufficient budget authority to maintain the overall health of this essential agency.

Thank you for the opportunity to present our views today. The Alliance for Aging Research looks forward to working with you on enacting legislation to reauthorize this important program. I am happy to answer any questions you may have.