Testimony before the
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Chairman Murray, Ranking Member Burr, and members of this committee, thank you for the opportunity to testify about the proposed FDA user fee agreements and how legislation reauthorizing these agreements can support patients and public health.

Established in 1948, The Pew Charitable Trusts is a global nongovernmental organization that seeks to improve public policy, inform the public, and invigorate civic life. Through research and analysis, Pew's projects work to improve Americans' health and well-being. We believe that evidence-based policies and investments can help expand access to life-saving treatments, spur the development of innovative drugs and medical devices, and provide effective oversight of the benefits and risks associated with these products.

Today, I would like to talk about how the user fee agreements can promote innovation and help to ensure the safety and effectiveness of medical products, ultimately leading to improvements in health.

The user fee agreements promote innovation of drugs and medical devices

Since 1992, user fees paid by the drug and device industry have provided FDA with significant and sustained resources that allow the agency to facilitate the development of urgently needed medical products, and to review those products quickly.

This issue is particularly important to Pew's antibiotic resistance project, which is working to advance policies that would spur the creation of new antibiotics and establish stewardship programs to ensure that antibiotics are prescribed only when necessary in human

health care settings. Since 2014, FDA has approved 14 antibiotics[1]—three of which represent a novel drug class or mechanism of action—to treat a variety of life-threatening bacterial infections, including community-acquired pneumonia and certain abdominal infections. These advances are critical as the world faces a dangerous shortage of antibiotics to address current and future patient needs.

Overall, the user fee programs have substantially sped up the review of new drug applications. In the decade after the first user fee agreement was passed, the median review time fell by half, from nearly 28 months to less than 14 months. Review times for drugs given priority status have also fallen significantly. Indeed, a standard review today is faster than a priority review a decade ago (around 10 months)[2].

In 2002, Congress also established a user fee program for medical devices. The fees FDA collects under these agreements provide the agency with additional resources to review applications and better facilitate the introduction of a wide variety of new medical technologies. Under the proposed agreement, the total fees collected are expected to reach at least $1.78 billion,[3] and if certain specified goals are met, the agency could collect up to $1.9 billion by 2027, up from about $1 billion in fees authorized under the previous reauthorization.[4] These funds help FDA deliver a more efficient and comprehensive oversight process that is better resourced to protect consumer safety and adapt to a rapidly evolving device market, where emerging technologies are posing challenges to traditional FDA oversight.

Today, for example, health care organizations use AI-enabled digital health tools for a growing range of clinical, administrative, and research purposes. FDA has approved or cleared

[1] K. Talkington. "Analysis Shows Continued Deficiencies in Antibiotic Development since 2014", The Pew Charitable Trusts, last modified March 9, 2021, https://www.pewtrusts.org/en/research-and-analysis/data-visualizations/2019/five-year-analysis-shows-continued-deficiencies-in-antibiotic-development
[2] https://www.fda.gov/media/102796/download?msclkid=381a13f5b03e11ec9429b69ceba87906
[3] U.S. Food and Drug Administration, "FDA Statement on Medical Device User Fee Amendments (MDUFA)," news release, March 22, 2022, https://www.fda.gov/news-events/press-announcements/fda-statement-medical-device-user-fee-amendments-mdufa
[4] FIND

nearly 350 AI-enabled devices for a broad range of applications.[5] Nearly 140 of these approvals or clearances have been granted since the start of 2020,[6] and the pace of submissions that include an AI component is only expected to grow over the next five years. These tools offer unique opportunities to improve patient care and health outcomes, but the volume of applications and the pace at which these products evolve pose unique challenges to FDA's traditional approach to oversight. These products will not only need to be reviewed to ensure they are safe and effective at the time of approval, they also need to be adequately monitored over time to ensure that they continue to be safe and effective in the real world, and when used on diverse patient populations. FDA must have sufficient resources—both through robust user fee programs and annual appropriations—as well as the internal expertise and commitment to develop regulatory policies that can facilitate ongoing innovation while still providing adequate public health safeguards for these rapidly changing products.

Similarly, 3D printing is increasingly being used at the point of care to manufacture a range of products, including anatomical models used to guide surgery planning and medical devices like surgical cutting guides. This technology allows for the decentralized manufacturing of highly customized products—which could one day include implants, pharmaceuticals, and even biological products—that are manufactured directly within health care facilities. However, 3D-printed devices—like any medical product—also carry risks, and existing laws, regulations, and guidance meant to ensure the safety of devices and drugs do not clearly map to this emerging technology. FDA needs the resources to be able to adapt its regulatory approach to meet the demands of the changing field and regulate the increasing number of sites that utilize the technology in order to ensure that medical products printed at the point of care are safe and effective.

[5] https://www.fda.gov/medical-devices/software-medical-device-samd/artificial-intelligence-and-machine-learning-aiml-enabled-medical-devices
[6] Ibid

The user fee process can support a broad range of public health priorities

Though the matter of FDA funding is central to the user fee negotiation process, each successive reauthorization of those agreements has provided an opportunity for Congress to pass additional reforms that advance public health. For example, past reauthorizations led to the establishment of the Sentinel Initiative, provided important incentives for the development of new antimicrobial drug products, strengthened FDA's ability to require postmarket trials or labeling changes in response to safety signals, and facilitated efforts to better incorporate the patient perspective within the agency's decision-making process, among many other consequential changes.[7] We urge Congress to consider other worthy opportunities to improve public health during the current reauthorization.

In particular, the issue of diagnostic test oversight, currently addressed in bipartisan legislation known as the Verifying Accurate Leading-edge IVCT Development (VALID) Act, is of critical importance. As the COVID-19 pandemic has shown, the nation's public health depends on rapid access to accurate and reliable tests that can diagnose disease or identify past infection. But faulty diagnostic tests can compromise both patient care and the nation's response to infectious diseases.

Current gaps in oversight have allowed tests that are developed and used within the same laboratories, called lab-developed tests, to come to market without FDA approval, even if those tests are otherwise high-risk. Once used to test for rare diseases for which commercially manufactured diagnostics were unavailable, these tests have now become widespread and increasingly complex. However, because there is no central registration or reporting requirements for these tests, the exact size of the market is unknown, leaving countless people exposed to potential harm from unreliable or misleading results.

[7] A. Mitchell et al. "The Prescription Drug User Fee Act", *Medical Care,* Volume 60 - Issue 4: 287-293, April 2022, https://journals.lww.com/lwwmedicalcare/Citation/2022/04000/The_Prescription_Drug_User_Fee_Act__Much_More_Than.4.aspx

As Committee members discussed at a recent hearing, the current MDUFA reauthorization is an appropriate vehicle for the VALID Act. By including VALID in the user fees legislation, Congress can strengthen and update current medical device regulations and enable FDA to adopt a risk-based approach to diagnostics oversight that balances safety and innovation.

Congress also acknowledged in its 2012 PDUFA reauthorization bill the importance of addressing the growing public health threat of antibiotic-resistant superbugs and the vital need for new antibiotics by enacting the Generating Antibiotic Incentives Now (GAIN) Act as part of that package. The GAIN Act represented an important first step that supported the launch of small biotechnology innovator companies devoted to antimicrobial research and development.

But on this front, more work is needed. A robust, market-based subscription model is necessary to ensure that these companies can earn a fair return-on-investment and continue to innovate. The sustainability of the antibiotic drug pipeline is absolutely foundational to modern medicine. To that end, the Pioneering Antimicrobial Subscriptions to End Upsurging Resistance (PASTEUR) Act proposes a unique, 'only pay for success' pull incentive that will substantially strengthen U.S. preparedness for future pandemics. We urge Congress to include that measure in its current PDUFA reauthorization.

The user fee process is no substitute for adequate funding

Review times are important insofar as they speed patients' access to potentially important products. The user fee agreements make review times a performance metric. However, it is critical to remember that true innovation is not just about getting products to market faster; it is about developing products that are safer or more effective than existing drugs and devices. While more challenging to measure than review times, protecting and promoting health is the ultimate goal of the FDA.

As important as user fees are to the efficient function of FDA, they cannot be a substitute for adequate appropriations. User fee agreements do not cover a broad range of essential health functions, such as enforcing good manufacturing practices, conducting most post-market safety activities, and regulating non-drug products, including food and the large and ever-growing market of dietary supplements. FDA needs sufficient additional resources beyond user fees to sustain these critical activities.

Furthermore, FDA is a public health agency that works to promote the health of all Americans. Because of the public interest in a well-performing FDA, the agency should receive public funds and be accountable to the public, not just to the industries it regulates.

Conclusion

In conclusion, I want to emphasize the importance of user fees to the basic functioning of the FDA. We urge Congress to ensure that FDA has continued, sustained funding to carry out its important public health mission.

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