07 April 2022

Senate Health, Education, Labor & Pensions Hearing on FDA User Fee Agreements: Advancing Medical Product Regulation and Innovation for the Benefit of Patients

On Tuesday (April 5), the Senate Health, Education, Labor & Pensions (HELP) Committee held a hearing entitled, "FDA User Fee Agreements: Advancing Medical Product Regulation and Innovation for the Benefit of Patients." The hearing was the first of two the committee will hold on Food and Drug Administration (FDA) user fee agreements, which authorize the FDA to collect user fees from companies and requires congressional reauthorization every five years. During the hearing, committee members discussed a range of topics related to the Prescription Drug User Fee Amendments (PDUFA VII), Generic Drug User Fee Amendments (GDUFA III), Biosimilar User Fee Amendments (BsUFA III), and the Medical Device User Fee Amendments (MDUFA V), which would authorize new user fees for their respective industries for fiscal years 2023-2027. Witnesses at Tuesday's hearings included representatives from each industry, as well as the Pew Charitable Trusts who answered lawmakers' questions on ways the latest agreements would improve access to lower cost drugs and address longstanding concerns with FDA processes.

- *For more information*: https://www.help.senate.gov/hearings/fda-user-fee-agreements-advancing-medical-product-regulation-and-innovation-for-the-benefit-of-patients

Opening statements

Committee Chair Patty Murray (D-WA): "It's important we reauthorize the user fee programs – which ensure as FDA gets new drugs or devices to consider for approval, as it gets more potentially life saving work to do, it also gets more resources to do that work. Congress has regularly reauthorized the user fee programs in a bipartisan way. … It's clear we can't simply settle for business as usual because when look at issues like the exorbitant cost of prescription drugs, the lack of diversity in clinical trials, the scourge of opioids and especially fentanyl, the lack of oversight for dietary supplements and cosmetics and how long it took to get contaminated baby formula off the market it's clear to me … that business as usual is not good enough. **…** I hope we will make progress on all of those issues and more as we work to reauthorize the user fee programs." Full statement.

Committee Ranking Member Richard Burr (R-NC): "When Members of Congress complain about the cost of prescription drugs or medical devices, we should evaluate all aspects of the pipeline, including the cost of development and regulatory review." Sen. Burr raised concerns with "the enormous growth in the oldest of the user free programs" and FDA's failure under past agreements to meet required review times. "With each reauthorization, FDA receives huge increases in resources, despite not fully delivering what it previously promised. … And with a declining percentage of congressional appropriations for the overall programs, FDA is increasingly removing itself from Congress' reach. … I question whether the agreements, both past and proposed, reflect a good deal for the patients they are designed to serve." Full statement.

Witness Testimony

Liz Richardson, Director, Health Care Products Project, The Pew Charitable Trusts: "[E]ach successive

reauthorization of [user fee] agreements has provided an opportunity for Congress to pass additional reforms that advance public health. … We urge Congress to consider other worthy opportunities to improve public health during the current reauthorization," including addressing gaps in diagnostic test oversight and passing both the VALID Act to strengthen current medical device regulations and the Pioneering Antimicrobial Subscriptions to End Upsurging Resistance (PASTEUR) Act to create a market-based subscription model for antimicrobial research and development. In addition, Richardson emphasized the importance of "adequate appropriations," which allow FDA to enforce "good manufacturing practices, [conduct] most post-market safety activities, and [regulate] non-drug products, including food and the large and ever-growing market of dietary supplements." Full Testimony

Cartier Esham, Ph.D., Chief Scientific Officer and Executive Vice President of Emerging Companies, Biotechnology Innovation Organization: "This reauthorization is an opportunity to build on lessons learned from responding to the COVID-19 public health emergency and incorporate these innovations into the regulatory paradigm. … Drug development for patient populations with unique needs, such as the pediatric community, remains a priority for BIO and our members. … PDUFA VII and BsUFA III include provisions to enhance drug development with the goal of advancing novel therapies for patients, including … rare diseases and pediatrics. … BIO strongly supports timely enactment of the PDUFA VII and BsUFA III Commitment Letters. The resources provided will serve to maintain FDA's global leadership and enable the Agency to keep pace with the medical and scientific advances of today and tomorrow." Full Testimony

David Gaugh, Senior Vice President, Sciences and Regulatory Affairs, Association for Accessible Medicines (AAM): "AAM and its Biosimilars Council strongly support congressional reauthorization of GDUFA and BsUFA as negotiated and without changes." Gaugh outlined five areas where AMA believes GDUFA III will enhance FDA's generic drug program: "advancing approvals, complex generics, inspections, suitability petitions and sustainability." He also outlined "several enhancements to FDA's biosimilars program" included in BsUFA III: "supplement reviews, meeting management, regulatory science and interchangeability, inspections, use of carryover funds and IT modernization." He said, "Timely approval of the FDA user fee agreements ensures patients will continue to benefit from new, high-quality and more affordable generic and biosimilar medicines." Full Testimony

Mark Leahey, President and Chief Executive Officer, Medical Device Manufacturers Association: "MDUFA V provides over $2B in investable funding to FDA. … under MDUFA V, FDA will be able to hire a minimum of 273 FTEs and up to 387 new FTEs to support the MDUFA program. … This represents a historic increase in both overall funds and people. … The agreement contains resources to start a pilot for the 'Total Product Lifecycle Advisory Program,' also known as 'TAP'" to support devices developed under the Safer Technologies Program (STeP). "[I]t will be critical over the coming years to meet the goals and milestones within this user fee agreement to help ensure that the United States remains the global leader in medical technology development." Full Testimony

Discussion topics

During the hearing, discussion centered on ways to increase the number of lower-cost biosimilars and generic drugs entering the market. Other topics of included the role of the accelerated approval pathway, medical device security, clinical trial diversity, and antimicrobial resistance. Summaries of the most-discussed topics are below.

Generic and biosimilar drug competition. During the hearing, several lawmakers including Committee Chair Murray and Sens. Bill Cassidy (R-LA) and Maggie Hassan (D-NH), discussed various tactics that brand name drugmakers use to prevent lower cost generics from entering the market, including misusing FDA's citizen petitions process, patenting risk evaluation and mitigation strategies (REMS), and making small updates to drugs that block access to lower-cost versions. In addition, committee members discussed FDA processes they believe

need to be overhauled to support generic drug development and approval, including accelerating the time it takes FDA to provide a therapeutic equivalence rating to complex generic drugs and information about a reference drug's inactive ingredients and concentrations. Mr. Gaugh said those processes can delay generic drugs by several months, while drugmakers' use of REMS patents can delay generic drug entry by years. Sen. Tina Smith (D-MN) said she and Sen. Cassidy are working on legislation to address the therapeutic equivalence rating issue. Mr. Gaugh also noted that BsUFA III includes several provisions to speed FDA review and approval of biosimilars.

Accelerated approval pathway. Several Republican members of the committee, including ranking member Burr and Sen. Susan Collins (R-ME), said they want the FDA's accelerated approval pathway to be used for therapies outside of oncology, such as rare diseases and neurological disorders. Ms. Esham said BIO also supports expanding the fields that use the program, noting 60% of accelerated approvals to date have been for oncology innovations. Ms. Esham highlighted several provisions within PDUFA VII that BIO believes will facilitate use of the accelerated approval pathway for rare diseases and neurological disorders, including the newly proposed Rare Disease Endpoints Advancement (RDEA) pilot program, FDA-led public workshops to discuss endpoint development for rare diseases, and the ability to engage with FDA earlier in the process to discuss novel surrogate endpoint development. While Democrats on the committee did not delve into concerns with the accelerated approval pathway during the hearing, some Senate Democrats, as well as FDA, have discussed the need for additional guardrails to ensure products approved under the pathway benefit patients.

Medical device security. During the hearing, Sens. Cassidy and Jacky Rosen (D-NV) raised concerns about cybersecurity and medical device safety. Sen. Cassidy noted that the PATCH Act, which he worked on with Sen. Tammy Baldwin (D-WI), requires a pre-market overview of cybersecurity measures and post-market updates for issues discovered later on. Mr. Leahey said the Healthcare and Public Health Sector Coordinating Council, a public-private partnership, in 2019 published a joint security plan and continue to share real-time data to protect medical devices.

Clinical trial diversity. Sens. Chris Murphy (D-CT) and Rosen discussed the need for clinical trials to reflect diverse patient populations, with Sen. Rosen placing particular emphasis on gender diversity. In response, Ms. Richardson said the user fee reauthorization process provides an opportunity for FDA to rethink policy priorities for the next five years, which could include advancing clinical trial diversity.

Antimicrobial resistance. During the hearing, Sen. Casey discussed points included in Ms. Richardson's testimony on antimicrobial resistance, noting that the financial realities of developing these new treatments is not keeping pace with their need. Richardson, in response, said "antimicrobial resistance is a public health crisis" and that creative ideas to realign current market incentives could encourage drug developers in this area. She pointed to Sen. Casey's DISARM Act and the PASTEUR Act as good starting points.

Other topics. During the hearing, Sen. Roger Marshall (R-KS) said lawmakers should address rebates to help drive down the cost of insulin, noting that proposals to cap out-of-pocket costs, would simply shift costs to others. Sen. Marshall also asked about innovations, such as using human cells, to conduct non-primate medical research. In response, Ms. Esham said BIO supports the shift toward non-primate research and believes there is a path forward to reduce use of primates in medical research. In addition, Sens. Bob Casey (D-PA) and Mike Braun (R-IN) discussed the importance of incorporating patient feedback into FDA's processes and future hearings. Meanwhile, Sen. Murphy said mental health drug development is not keeping pace with patient needs.

If you have questions, please contact Heather Meade or Heather Bell.