Insight

FDA User Fee Reauthorization in the House and the Senate

MARGARET BARNHORST | JUNE 29, 2022

Executive Summary

- The Food and Drug Administration (FDA) user fee programs for prescription drugs, medical devices, generic drugs, and biosimilars—used to fund the agency's technology and workforce, speed up review of industry product applications, and bring new products more quickly to market—must be reauthorized for the next five years by September 30, 2022, the end of the fiscal year.
- The Senate Committee on Health, Education, Labor, and Pensions marked up its version of the "must-pass" legislation, which, like the House-passed bill, contains several riders unrelated to the user fees that would expand the FDA's authority.
- Both bills would reauthorize user fee programs and reform the accelerated approval process, though the House bill also seeks to improve clinical trial diversity and manufacturing inspections, while the Senate bill would address the infant formula crisis and increase regulation of cosmetics, dietary supplements, and in-vitro tests.

Introduction

The Food and Drug Administration (FDA) collects user fees from the drug industry to fund its technology and workforce, speed up review of industry product applications, and bring new products more quickly to market. In exchange for paying user fees, the drug industry receives a commitment from the FDA that the agency will meet certain performance and hiring goals to improve and speed up the drug review and approval process. User fee programs make up over half of the FDA's overall budget and are thus essential to the agency's ability to accomplish its mission. While some user fees are permanently authorized, the Prescription Drug User Fee Act (PDUFA), the Medical Device User Fee Act (MDUFA), the Generic Drug User Fee Act (GDUFA), and the Biosimilar User Fee Act (BsUFA) must be reauthorized every five years. The current user fee programs must be reauthorized before September 30, 2022—the end of the fiscal year.

Earlier this month, the House passed its user fee package, the Food and Drug Amendments of 2022 (FDA22), with overwhelming bipartisan support under suspension of rules, and the Senate Committee on Health, Education, Labor, and Pensions (HELP) recently advanced its own user fee reauthorization package, the FDA Safety and Landmark Advancements (FDASLA) Act, to the full Senate floor as a manager's amendment. Given the "must-pass" nature of the legislation, Congress typically uses the reauthorization process as a way to address other regulatory concerns within the FDA. This year, both the House and Senate bills would reauthorize the four main user fee programs listed above and reform the accelerated approval process, yet they also include a vast array of different, though not contradictory, riders related to clinical trial diversity, regulation of cosmetics and dietary supplements, and manufacturing inspections. In the coming months, Congress will be tasked with reconciling the differences between the two packages ahead of the September 30, 2022, deadline in order to reauthorize FDA user fee programs for another five years from 2023-2027.

This insight will explore areas of agreement between the two bills, as well as several of the various riders included in each package that lawmakers must reconcile in the final text.

Areas of Agreement

User Fee Reauthorizations

Both the House and Senate packages would reauthorize PDUFA, MDUFA, GDUFA, and BsUFA, largely maintaining congressional reporting requirements and the fee structures agreed upon during prior negotiations between the FDA and respective industry groups. The base fees increased for all four user fee programs since 2022 and the amounts were updated as follows for fiscal year 2023: $1.15 billion for PDUFA, $130.2 million for MDUFA, $582.5 million for GDUFA, and $43.4 million for BsUFA. Notably, the MDUFA reauthorization also adds for the first time the potential for performance-related fee increases later in the five-year cycle if the FDA meets certain hiring and review goals.

Accelerated Approval Reforms

The Accelerated Approval Program within the FDA allows for the early approval of innovative therapies based on preliminary data markers that predict clinical benefit, while drug sponsors theoretically conduct additional post-marketing studies to confirm the product's benefit. The process has recently come under scrutiny, however, following the approval of Aduhelm to treat Alzheimer's disease despite a panel of experts recommending against its approval due to limited efficacy data.

The House and Senate reauthorization bills both attempt to reform the accelerated approval pathway by streamlining the process for the FDA to remove products from the market that either fail to show clinical benefit or are not being tested in confirmatory trials and allowing the FDA to require sponsors to begin post-approval studies before a drug goes to market. Conditions for post-approval studies—such as enrollment targets or target dates for study completion—would need to be specified at the time of approval. Such provisions are slightly more industry-friendly compared to previous proposals to reform the accelerated approval pathway, as they do not impose penalties on companies that fail to meet the requirements, nor do they enforce an automatic expiration date for studies not completed within five years after initial FDA approval.

One notable difference between the accelerated approval reforms in the two chambers' bills, however, is a provision in the current version of FDASLA that would establish an intra-agency coordinating council within the FDA to meet at least three times per year to evaluate the accelerated approval pathway and address any outstanding issues.

Generic Drug Competition

The current versions of both the House and Senate bills include riders broadly related to improving generic drug competition and facilitating generic drug development and approval. One such provision found in the current versions of both bills would require the FDA to provide information to generic drug sponsors upon request about differences in ingredients between the generic and reference drug. Under the current generic drug approval process, the FDA may simply indicate that a generic drug is not similar enough to the reference drug to gain approval and is not required to provide any further guidance to the generic manufacturer. Proposals in the reauthorization bills would require the FDA to issue explanations on how it determines whether a generic drug is the same as the reference product, both quantitatively and qualitatively. Another provision in both the FDA22

and the current version of FDASLA would allow generic drugs to be approved even if the proposed labeling is different from that of the brand drug, within certain constraints, for changes made to the brand-name drug label within 90 days from the reference drug's approval.

The bills also propose a handful of reforms related to biologics and biosimilars, including aligning reporting requirements for biologics with those for other drugs, such as requiring holders of approved biologic license applications to report to the FDA when withdrawing a product from market and conduct a one-time study to confirm that their products listed in the Purple Book—the FDA's list of licensed biological products—are still available for sale. Additionally, the current version of FDASLA would require the FDA to update the Purple Book based on such reports.

Other Program Reauthorizations

Both bills also reauthorize several additional programs through 2027, including the Best Pharmaceuticals for Children Program—which offers incentives for pediatric clinical trials—and the Critical Path Public-Private Partnership—which supports medical product innovation. Notably, FDA22 reauthorizes the Critical Path Public-Private Partnership at $10 million annually through 2027, while the current version of FDASLA reauthorizes the program at current funding levels, $6 million annually. Additional five-year reauthorizations found in both bills include orphan drug grants, pediatric device consortia grants, exemptions from certain requirements for devices designed to treat or diagnose rare diseases, and the exclusivity of drugs containing single enantiomers.

Where the Packages Differ

Beyond those similarities, the two packages contain several different, though not contradictory, proposals. Except for a handful of drug pricing amendments proposed during the Senate HELP Committee markup, some of which were tabled, many of the following riders found in only one of the two bills have bipartisan support and are largely uncontroversial and thus likely to be included in the final reauthorization package.

Improving Diversity in Clinical Trials

The House FDA22 bill includes several provisions aimed at improving diversity in clinical trials not included in the Senate bill. These include requiring clinical trial sponsors to submit diversity action plans as part of their clinical trial proposals to the FDA and requiring the agency to host public workshops with stakeholders about increasing the enrollment of historically underrepresented groups in clinical trials. Other provisions would require the FDA to publish and submit to Congress annual reports summarizing the diversity action plans received, as well as host a public meeting within 180 days after the end of the COVID-19 public health emergency to discuss clinical trial disruption mitigation strategies.

Manufacturing Inspections

To address limitations in the FDA's inspection authorities and delays exacerbated by the COVID-19 pandemic, the House FDA22 bill would allow the FDA to conduct remote data reviews for certain inspections in lieu of in-person visits and would also explicitly allow foreign government inspections to be sufficient for preapproval inspections, provided the FDA has entered into an agreement with that foreign government. Additionally, FDA22 would require the FDA to publicly share information related to drug facility inspection timelines and conduct a pilot program to increase unannounced surveillance inspections of foreign drug facilities.

Cybersecurity of Medical Devices

FDA22 would require manufacturers of devices that have software, connect to the internet, or "could otherwise be vulnerable to cybersecurity threats" to include in premarket submissions information regarding their processes and plans to ensure their devices are secure and cybersecurity vulnerabilities addressed. The bill would also authorize the FDA to exempt certain devices from these requirements, as well as allow the FDA to deny accelerated premarket clearance if such cybersecurity information is inadequate.

FDASLA includes provisions also related to the cybersecurity of medical devices, though these are different in substance to FDA22's. An amendment included in the current version of FDASLA would require the FDA, in consultation with the Cybersecurity and Infrastructure Security Agency, to review and update its medical device cybersecurity guidelines within two years of enactment of the legislation. Additionally, within 180 days of enactment of the legislation, as well as annually after that, the FDA would be required to update public information on the FDA website with tips to identify and address potential cyber vulnerabilities within health care systems and medical device manufacturers.

Give Kids A Chance Act

The Give Kids a Chance Act included in the House FDA22 package would allow children with cancer to participate in trials of multi-drug combination cancer-treatment therapies, not only single-drug trials as allowed under current law. Although the Give Kids a Chance Act is not included in the current version of FDASLA, Chair Patty Murray indicated during the HELP Committee markup that she was working with Senators Marco Rubio and Michael Bennet, the bill's sponsors, to include it in the user fee package when it comes in front of the full Senate.

Cosmetics and Dietary Supplements

Provisions in FDASLA would require cosmetics manufacturers to register products and manufacturing facilities with the FDA, as well as label their products according to specific guidelines. Additionally, cosmetic product labels would be required to include contact information for reporting adverse events; manufacturers would be required to record any such adverse events and report them to the FDA within 15 days. The bill includes exemptions for certain small businesses and over-the-counter (OTC) drugs and devices.

FDASLA also includes similar provisions related to the regulation of dietary supplements, including requiring companies to publicly list their supplements in an FDA database and prohibiting the fraudulent marketing of certain products as dietary supplements.

Diagnostic and In-Vitro Clinical Tests

Language from the Verifying Accurate Leading-Edge In-Vitro Clinical Tests (IVCT) Development (VALID) Act was included in FDASLA and would enhance the FDA's role in evaluating effective laboratory-developed tests, such as those used throughout the COVID-19 pandemic. Currently, the Centers for Medicare and Medicaid Services (CMS) indirectly regulates laboratory-developed tests through regulation of the laboratories performing such tests—however, the CMS review focuses narrowly on analytical validity of tests and is often completed once the tests are on the market. In contrast, FDA review includes both the analytical and clinical validity of the tests and is typically conducted pre-market.

Provisions in FDASLA would create a risk-based framework for IVCT review, differentiating between high-risk and lower-risk tests, similar to the existing regulation of medical devices. According to the bill text, only tests designated as "high-risk," such as those for which a false negative result could lead to serious harm or death to the patient, or those likely to result in delay or discontinuation of life-sustaining treatment, would be required to undergo pre-market review. A handful of laboratory-developed tests would be exempt from such provisions, including those used to diagnose rare conditions. Language in FDASLA explicitly states that such provisions would not modify CMS's authority to regulate IVCTs, though the provisions would certainly complicate future regulation given the overlap between laboratories and laboratory-developed tests.

Infant Formula Crisis

The current version of FDASLA includes several provisions to improve infant formula supply, address the FDA's role in the ongoing infant formula crisis, and improve FDA oversight to prevent future crises. Noteworthy provisions require the FDA to resolve mailroom issues that delayed its response to the ongoing shortage, publish a list of appropriate substitutes for infant formula products in shortage, submit an annual report to Congress on infant formula inspections, and meet with representatives from other countries to resolve differences in regulatory requirements for infant formula, among others. The bill would also require manufacturers to notify the FDA within five days of any manufacturing disruptions that may affect formula supply and would require the FDA Commissioner to notify Congress of any formula recalls within 24 hours of the recall. FDASLA would also establish the Office of Critical Foods within the FDA's Center for Food Safety and Applied Nutrition to increase oversight and coordination of activities related to infant formula and foods required for certain medical purposes.

Additionally, a handful of amendments proposed during the Senate HELP Committee markup were passed and included in the current version of FDASLA to address the infant formula crisis. Notably, Americans would be allowed to import up to a three-month supply of formula from Canada or countries in the European Union for 90 days following enactment of the legislation. Another amendment would grant the FDA authority to waive certain requirements for the importation of specialty formulas during shortages. Such provisions would simplify current, and often complicated, guidelines surrounding the importation of formula for personal use, such as those regarding compliance with FDA labeling guidelines or prior notice of shipments.

Over-the-Counter Hearing Aids

During the 2017 user fee reauthorization process, Congress required the FDA to establish a category of OTC hearing aids, publish proposed rules by August 2020, and finalize the proposed rules within 180 days of the close of the comment period. The FDA eventually published proposed rules in October 2021, more than a year after the initial deadline, with the comment period concluding on January 18 of this year—meaning the 180-day deadline for the FDA to issue a final rule on OTC hearing aids is July 17, 2022. Congress does not appear confident the FDA will meet the upcoming July deadline, however, given that the current version of FDASLA would require the agency to issue a final rule on OTC hearing aids within 30 days of the enactment of the user fee legislation.

Drug Importation

Provisions in the current version of FDASLA would eliminate the FDA waiver requirement for personal importation of prescription drugs from Canada, under conditions that "the importation for personal use will not increase the public's exposure to counterfeit products or exacerbate the opioid epidemic." It's unclear, however,

how the FDA will make such safety determinations.

During the Senate HELP Committee markup, Senator Bernie Sanders proposed a few additional amendments related to drug importation, including one that would allow individuals, pharmacies, and wholesalers to import drugs from Canada and the United Kingdom—and, after two years, from other member countries of the Organization for Economic Cooperation and Development—and another that would limit the price of prescription drugs to no more than the average price in five reference countries. A handful of similar reference pricing proposals were introduced during the Trump Administration, yet analysis from the American Action Forum found that implementing foreign price controls would significantly reduce drug maker revenues, consequently stifling innovation and limiting Americans' access to new therapies.

Chair Murry eventually tabled these amendments during the HELP Committee markup—noting they were too controversial, lacked sufficient bipartisan support, and would risk delaying passage of the broader reauthorization package—though Senator Sanders could attempt to re-introduce such amendments when FDASLA is brought to the full Senate floor.

Conclusion

The passage of the House user fee reauthorization bill earlier this month, and the recent advancement of the Senate bill to the full chamber, mark important steps in the FDA user fee reauthorization process that occurs every five years. Congress is now tasked with reconciling the differences between the two packages before the September 30, 2022, expiration of current user fee programs—though at this point, it is unclear how and when House and Senate leaders plan to do so. Discussions over the next few months will ultimately determine which of the various riders will survive in the final bill to reauthorize user fee programs until 2027.