Drug Companies Breathe Easier
As PDUFA VI Heads for Approval

Stephen Barlas

Mr. Barlas is a freelance writer in Washington, D.C., who covers issues inside the Beltway. Send ideas for topics and your comments to sbarlas@verizon.net.

The prescription drug user fee agreement that Congress must approve by the end of September is particularly important because it furthers some of the drug-approval process innovations in the 21st Century Cures bill passed in 2016. That bill requires the Food and Drug Administration (FDA) to take certain steps to incorporate biomarkers, real-world evidence, and alternative clinical trial designs into the agency approval process. Equally important, industry fees authorized by the Prescription Drug User Fee Act (PDUFA) fund critical FDA salaries that could not otherwise be afforded given the limited Congressional appropriation. Failure to approve the sixth iteration of PDUFA would force the agency to lay off staff.

The FDA started meeting with interested parties in 2015 to lay the groundwork for PDUFA VI and published a commitment letter in late 2016 detailing fee changes and process improvements. That agreement covers fiscal years (FYs) 2018–2022. Everyone from industry to consumer groups to members of both parties of Congress seemed happy with that agreement as 2017 began.

But President Trump threatened to upset the apple cart when he proposed in March that industry pay $1 billion more in user fees than agreed upon, spread across the five user fee programs (the others being medical devices, generics, bio-similar, and animal drugs). The assumption was that Trump expected Congress to cut the FDA’s appropriation in FY 2018 (which starts on October 1, 2017) by that amount. No one outside the White House was happy about that prospect.

Fast-forward to mid-April. House and Senate Republicans and Democrats announced an agreement on PDUFA VI that set the fees at exactly the dollar level previously determined. In PDUFA V, which covers FYs 2013–2017, total fees were $718,669,000 annually, adjusted for various factors. The base annual fee total for FYs 2018–2022 would be $878.5 million in FY 2018 and would increase over the five years. User fees account for 68% of the FDA’s review budget for prescription drugs.

The issues in PDUFA VI are much different than those underlying the generic and biosimilar user fee reauthorizations in GDUFA II and BsUFA II, both of which are finishing their inaugural five-year programs. Generics companies have had to endure long waits and numerous review cycles before receiving FDA approval. That isn’t the issue with PDUFA. The FDA is approving 90% of applications for new molecular entities in their first review cycle, often within 10 months. The agency has taken major strides in approving important medications through the breakthrough therapy designation program established in PDUFA V. To date, 170 breakthrough therapy designations have been granted, leading to 79 indications.

The major emphases of PDUFA VI, which track more or less with provisions in the 21st Century Cures bill, are enhancement of patient involvement in the drug approval process and the FDA’s allowance of biomarkers, alternative clinical trials, and real-world evidence. The FDA has had a “qualification” program for biomarkers. The PDUFA VI agreement commits the agency to implement a pilot program to seek and incorporate the input of external experts to assist in those qualifications and to verify if the use of such outside experts can make the process more timely and efficient.

Much of what the FDA plans to do to enhance use of biomarkers sounds pretty bureaucratic. The same is true of commitments to broaden FDA use of real-world data, which involves utilizing medical claims data, disease registry data, and other electronic health records to assemble a thorough picture of all the risks and benefits of a drug after it has been approved and physicians and hospitals have started using it. The drug industry fervently hopes that expanded use of real-world data will let companies obtain additional indications more quickly and with less testing for already approved drugs.

It would appear that the President’s plan to impose another $1 billion in FDA user fees is a “no go” given the bipartisan congressional agreement. Still, the uncertainty over user fees on one hand and appropriations on the other has caused the FDA to go slow on funding some new initiatives included in the Cures bill. One example is the Oncology Center of Excellence. “The reality of lagging funding, the potential of a detrimental budget future, and the presence of a hiring freeze place this transformational opportunity at significant risk,” says Jeff Allen, President and CEO of Friends of Cancer Research.

While Congress will rubber-stamp the user fees accepted by brand-name manufacturers, the bigger question is whether the FDA appropriations bill for FY 2018 will reflect the low number Trump proposed while believing that he would get an additional $1 billion in user fees. Congress has to decide whether to ignore Trump’s low-ball request. It is likely to do so. But will it fully fund the FDA? Will the approval process enhancements in the Cures bill, paid for in part by higher PDUFA fees, bear fruit—and, if so, how soon?

REFERENCES