

# Using the Federal Review Process to Expedite Access to Treatments

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I easily understood the frustration of Mark Thornton, MD, when I read his recent opinion piece in the *Wall Street Journal*.<sup>1</sup> People with chronic conditions—especially individuals who have no viable treatments or cures—share his anger.

Thornton is president of the Sarcoma Foundation of America, and he pointed out that 2 unique therapies for the treatment of prostate cancer and osteosarcoma were rejected by the US Food and Drug Administration (FDA) in 2007 and sent back to their respective companies for more clinical study. The prostate treatment was finally approved for patient use this year; the osteosarcoma treatment still is in the review process, even though it has been approved in the European Union.

Patient advocacy groups understand firsthand the importance of improving patient access to effective new treatments. Beginning this year, we have an opportunity to have a say in a federal program that could have a marked impact on the availability of such drugs.

The Prescription Drug User Fee Act (PDUFA) authorizes the US FDA to collect fees from companies that produce certain human drug and biologic products. The user fees currently fund about half of new drug review costs and have played an important role in expediting the drug approval process. PDUFA was enacted in 1992 and renewed in 1997 (PDUFA II), 2002 (PDUFA III), and 2007 (PDUFA IV).<sup>2</sup> The reauthorization process for PDUFA V began with a public hearing in April 2010.

The patient advocacy community has been, and continues to be, supportive of PDUFA. In the best of all possible worlds, US FDA programs would be funded entirely with tax dollars. However, the patient advocacy community recognizes there are fiscal constraints. It is essential that PDUFA be reauthorized in order to expedite patient access to new treatments.

We have seen great results under PDUFA I, II, and III. The program created the resources that the US FDA has effectively used to improve the process for bringing new products to market while still meeting safety and efficacy standards. Our challenge is that we're not seeing the same efficiencies under PDUFA IV. According to a US FDA fiscal

year 2008 report, 71% of the priority applications for new molecular entities and biologics reviewed under PDUFA were completed on time.<sup>3</sup>

As US FDA Commissioner Margaret A. Hamburg, MD, pointed out in her fiscal year 2008 report, the transition to PDUFA IV provided unprecedented challenges to the US FDA. "Expanding the work force, training and mentoring new staff, and adapting to new requirements including new broad authorities . . . have limited the US FDA's ability to review as high a percentage of applications and submissions on time as in previous years," she wrote.<sup>4</sup>

The patient advocacy community fought for changes in PDUFA as a means to take the agency's work to a new level—to encourage faster approval of new treatments that could be delivered to market more quickly.

I believe there are 3 ways to help the US FDA meet the intent of PDUFA and accomplish the goals sought by people with chronic conditions.

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First, under PDUFA V, there needs to be patient involvement at all levels of the US FDA approval process. Patients are their own experts. One cannot underestimate the knowledge that informed patients have about their conditions. If you have a rare disorder, you probably know more about your condition than anyone else.

People who take medications on a daily basis understand there is a risk associated with new drugs. They are willing to take that risk to have a shot at a more normal, productive, and happy life. The patient advocacy community can give credibility to the US FDA process when the agency has to

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make the tough decisions about the balance between the benefit and risk of potential new treatments.

Too often the decisions about benefit and risk are made by people who are healthy and not dependent on drugs on a daily basis. The National Health Council urges the US FDA to involve people with chronic conditions in their decision-making processes, including the development of risk evaluation and mitigation strategies (REMS).

In 2007, the US FDA was given the authority to require the submission and implementation of a REMS if the agency determined the strategy was necessary to ensure that a drug's benefits outweigh its risks.

The goals of REMS are to achieve the targeted health outcomes or level of knowledge related to known safety risks of a drug.<sup>5</sup> Instead of trying to identify every possible risk in the testing phase, a treatment should be released to market quicker with a postmarketing strategy to monitor the drug. A REMS covers elements such as medication guides, patient package inserts, a communications plan to educate healthcare providers, and other requirements to ensure safe use of the drug, including the monitoring of patient usage.

Second, we need now an independent review of the REMS process. The National Health Council has recommended that the Government Accountability Office conduct an analysis of the impact of REMS to determine whether that process is impeding the access to safe and effective treatments, rather than speeding it up. Remember, many people with chronic conditions die as a result of their condition while waiting for a treatment.

Third, under PDUFA V, there needs to be a way to encourage the development of innovative trial designs.

In the 1980s, the patient community came out and demanded change for the approval process of new HIV and certain cancer treatments. In 1988, the US FDA implemented new regulations designed to make promising therapies available sooner.<sup>6</sup> The patient advocacy community supported PDUFA I in 1992 because it streamlined the process and made it more efficient, and we got products to market more quickly.

We have seen some treatment advances in the cancer community and for those with HIV and heart disease. But we still are not getting new treatments to market as quickly as the patient advocacy community would like.

Many of the efficiencies under PDUFA have been accomplished because PDUFA has resulted in more US FDA staff and resources. Our next opportunity is to figure out

how to develop clinical trials that streamline the approval process for new medications that target the specific population that a particular drug is designed to reach.

For example, we need new methodologies for conducting noninferiority trials, which are clinical trials designed to determine whether a new treatment is equivalent to an existing standard treatment. We need trial protocols for specific therapeutic areas. We need to figure out how to better define the populations for whom we are going to develop treatments. And perhaps most important from the patient perspective, we need biomarker and surrogate end point qualifications, rather than using just the clinical trial protocols of the past for the new drug therapies of today.

It has become clear to me that many of the challenges of bringing new treatments to people occur at the *back end* of the development process—in the regulatory approval stage. We need to come up with better procedures for getting new treatments through the regulatory system.

The AIDS epidemic proved that we can do this. So why should somebody with lupus, or amyotrophic lateral sclerosis, or Alzheimer's, or Parkinson's, or lung cancer not get the treatments that they need now?

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