

The FDA vs. Bone Cancer Patients

The EU has approved a revolutionary treatment, but Americans wait.

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By MARK THORNTON

Three years and more than 80,000 deaths ago I wrote in these pages about a travesty that had occurred at the Food and Drug Administration.

In May of 2007 two unique cancer therapies for the treatment of prostate cancer and osteosarcoma (a type of bone cancer) came under review at the FDA on the same day. Both the new agent for prostate cancer, Provenge, and the new agent for osteosarcoma, Mepact, had shown the ability to prolong lives to a significant degree. And both drugs were summarily rejected. Provenge and Mepact were tossed back to the companies developing them with the directive to do more clinical studies.

This was easy enough for Provenge, due to the return on the risk of investment possible with a new prostate cancer drug and the large number of men with the disease available for another clinical trial. The Dendreon company, makers of Provenge, worked as quickly as possible to redo their already successful trial. The results of the new trial turned out the same as the original, and the drug was finally approved by the FDA last week.

In the three years that it took to duplicate what was already known, upwards of 80,000 men lost their lives to prostate cancer. This is equal to the number of men killed in combat in the Korean, Vietnam and Iraq wars combined.

Those FDA staffers who had a role in preventing the approval of Provenge in 2007 will have to live with this sin of omission. But at least now a powerful new weapon is available to help prostate cancer patients.

Not so for those of us in the American sarcoma community waiting for Mepact. Osteosarcoma effects only 900 Americans, mostly children and young adults, each year. The FDA's stunningly cavalier demand for another clinical trial for Mepact was made with the full knowledge that the repeat effort would require about 900 new patients.

Imagine if a clinical trial for a new asthma or diabetes drug required every single patient in the country to be included not once but twice and you will begin to appreciate the profound predicament those of us in the rare-disease community face when it comes to the FDA's irrational standards.

Recently, hope has emerged that a more humane approach might be taken at the FDA to approve new drugs for exceedingly rare diseases. In an amendment to the 2010 FDA Appropriations law, Sen. Sherrod Brown (D., Ohio) and Sen. Sam Brownback (R., Kan.) inserted language that requires the FDA to explain the unfairness of its policies, which have resulted in about 200 treatments for the over 8,000 diseases categorized as rare.

In March, the new FDA Commissioner, Dr. Margaret Hamburg, appeared before the Senate to discuss the issue. In emotionally moving testimony about her own experiences as a doctor dealing with patients in desperate need of new therapies, she promised "innovative if not transformative" approaches to the problem, saying that "new regulatory pathways could be developed . . . to catalyze activity in areas where there are limited markets." She added that this issue is "of the highest priority in the White House."

In Europe, such creative pathways for regulatory approval exist, and they have resulted in the approval for Mepact in the EU. Mepact is the poster child for all that is wrong at the FDA regarding treatments for rare diseases, and many have hope that Dr. Hamburg is the right leader to address this health crisis.

The approval of Provenge, a revolutionary agent that taps the body's own immune system to fight cancer, ushers in the dawn of the age of cancer immunotherapy. Mepact similarly stimulates the immune system to prolong osteosarcoma patients lives, and it is doing so as we speak for children in Europe with this bone cancer.

Oncologists in the FDA Center for Drugs should show penance for their fatal error and join their European colleagues in approving Mepact without the need for another 10-year long clinical trial. The Americans currently living with osteosarcoma can't wait any longer.

Dr. Thornton worked as a medical officer at the FDA for six years. He is the president of the Sarcoma Foundation of America, which has received modest contributions from a U.S. subsidiary that is owned by the