

# **FDA Week**

---

**an exclusive weekly report on Food and Drug Administration policy, regulation and enforcement**

from Vol. 17, No. 33, August 19, 2011

## **EveryLife Pushes New Drug Review Structure As PDUFA Debate Nears**

A rare disease advocacy group is lobbying FDA and preparing arguments for Congress on its plan to revamp the agency's pharmaceutical review structure by increasing user fees and creating more review specialties, saying the Prescription Drug User Fee Act reauthorization agreement, which includes provisions for rare diseases, does not do enough to bring changes for orphan drug review. The EveryLife Foundation for Rare Diseases recently pushed the idea in a meeting with FDA Commissioner Margaret Hamburg and plans to advocate for the concept on Capitol Hill as user fees are debated next year.

The group is reiterating its longstanding call to create a rare biochemical and genetic review division, as well as other changes to the review structure that could benefit the broader pharmaceutical industry thereby garnering the financial support to increase the number of reviewers and allow for more specialized therapeutic expertise, said Emil Kakkis, president of the foundation.

The new review division is an idea that has been misinterpreted by several stakeholders as a separate review division for rare diseases, he said. The rare biochemical and genetic review division would cover a small segment of rare disorders and would encompass those diseases that are not adequately addressed through other review divisions.

Further, some review divisions could include a new subdivision for rare diseases, like oncology, which would separate the more rare forms from breast and colon cancers. But some divisions, such as hematology, already have enough expertise to address rare diseases, he said.

"The view is that you need to have people trained in the field," he said.

The proposed changes would not just affect products for rare diseases. The group is pushing to create more therapeutic divisions, with the increased manpower giving division heads more time to focus on one therapeutic area as opposed to several, and more time to study and keep up with the changing science.

"What we would suggest is a more significant increase in PDUFA fees, but requirement to bolster review divisions," Kakkis said. The plan would benefit the broader pharmaceutical industry in exchange for increased fees, he said.

The group brought forward a plan during PDUFA stakeholder discussions, although it did not directly lobby industry during the negotiations. Kakkis met with Hamburg in June to garner agency support before user fee debates start on Capitol Hill. Getting the agency to push for the plan, "will help drive a positive movement from Congress," he said.

The agency's agreement with industry addresses rare disease among a slew of other provisions, although the final pact has not yet been publicized. While the industry agreement is complete, "our view is that the legislation is not done," Kakkis said.

Further, the agency-industry agreement reflects activities that are already being addressed by the agency, he said, noting that more could be done to encourage orphan drug development. "It's stuff that the FDA is comfortable with and is already doing," he said. — *Alaina Busch*