

Focus on FDA

Orphan Drug Advocates Continue Press For Policies Not In PDUFA Pact

Orphan drug advocates are continuing to push their own policies — including through filing a citizen petition calling for FDA reviewer flexibility — while also voicing support for provisions in the recently released drug user fee agreement. The agency-industry pact calls for policy development and staffing increases to address rare diseases, but FDA could take years to tackle topics like non-traditional clinical development programs, reviewer flexibility, and challenges with post-market studies, sources said.

The commitment letter on reauthorizing the Prescription Drug User Fee Act includes funding a staff increase of six people — one in the biologics center and five in the drug center — specifically for rare disease initiatives. Throughout the duration of the agreement, the agency will also develop policies and guidance, hold a public meeting, train staff and measure the success of the activities.

Michael Werner, an attorney at Holland & Knight, said the agreement is a step toward FDA embracing review policies outside those for traditional drugs. The pact includes provisions for the development of policies for non-traditional clinical development programs, study design, endpoints and statistical analysis.

“The big thing was how do we get the knowledge and expertise into the review aspect so it’s not just in the designation,” Werner said. He said the new staff will increase outreach to patient communities and translate into new policies. If approved, the staffing increase plan will be in place by the end of fiscal 2013 while other components, like training for all drug and biologics center review staff, will come in later years.

“We are especially delighted that this document calls for an additional five staff members in the Rare Disease Program in the FDA Center for Drug Evaluation and Research (CDER), and the creation of a liaison within the Biologics Center,” National Organization for Rare Disorders President and CEO Peter Saltonstall said in a statement. “The document reflects a clear recognition that drugs for rare diseases warrant special consideration and special staff training.”

The PDUFA agreement also comes as NORD continues its push for review flexibility with a citizen petition filed last week. The EveryLife Foundation for Rare Diseases also vowed to lobby Congress to do more before lawmakers finalize the industry-FDA pact.

NORD filed a citizen petition requesting the agency to include in guidance that is statutorily required later this year a standard policy stating that orphan drugs be afforded special flexibility in their review. The guidance is required within six months of a recent report to Congress on orphan drug development. NORD’s Chief Medical Officer Timothy Coté has pushed the policy since transitioning from the agency as its top orphan drug official (see *FDA Week*, Aug. 5).

Coté said his general policy could provide a starting point acknowledging differences between traditional and rare disease drugs, whereas the policies developed under PDUFA could drill down into the details of how orphan drugs should be treated. Further, the guidances called for in the agreement could take longer to develop. “Let’s at least start from the position that they are different,” he said.

Coté noted that there has traditionally been hesitancy against the idea of special treatment for orphan drugs, but the policy shift is not seeking to lower the approval bar and the agency has been flexible with past reviews.

“We certainly were a voice for flexibility and I will tell you that the review division has some excellent examples of flexibility,” he said.

In addition to NORD’s petition, EveryLife is pushing an FDA reorganization plan that includes creating more review specialties and a rare biochemical and genetic review division (see *FDA Week*, Aug. 19).

EveryLife president Emil Kakkis said the group will continue working with Congress, FDA and stakeholders to expand what is currently outlined in PDUFA.

“We are pleased to see that provisions for rare diseases were included in the PDUFA V technical agreement and are supportive of the other improvements to the regulatory process,” he said in a statement. “However, we hope that the provisions for rare diseases will be expanded to support changes needed to improve the specialization and academic connections of reviewers and to give rare diseases better access to the accelerated approval process. We hope that Congress does not miss this opportunity to do something larger and better to improve the long-term health and academic excellence of the agency.” — *Alaina Busch* (abusch@iwppnews.com)