

## **PATIENT GROUP HOPES TWEAKS TO APPROPS BILL LEAD TO NEW FDA OFFICE**

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**Date: October 9, 2009**

An advocacy group for patients with rare disorders is pleased that lawmakers strengthened a provision in the fiscal 2010 FDA appropriations bill that would establish two FDA review groups to recommend ways to prevent, diagnosis and treat rare diseases. The Kakkis EveryLife Foundation says the language is a step in the right direction and hopes the two review groups recommend that either FDA or Congress establish a new FDA office for rare diseases.

Sens. Sam Brownback (R-KS) and Sherrod Brown (D-OH) wrote the measure, which was added as an amendment shortly before the Senate voted for the bill Aug. 3. The conference report retains the amendment but with some changes.

The amendment, now included as Section 740 of the bill, requires FDA to establish a “review group which shall recommend to the Commissioner of Food and Drugs appropriate preclinical, trial design, and regulatory paradigms and optimal solutions for the prevention, diagnosis, and treatment of the developing world.” The previous version of the amendment stated that FDA “may” establish the groups.

The foundation urged Brownback and Brown to strengthen the language when the House and Senate worked out differences between their respective appropriations bills in conference, foundation President Emil Kakkis said. The foundation would also like to see report language or further instructions from FDA defining such terms as preclinical and regulatory paradigms. The hope is the groups will recommend a new FDA Office of Drug Evaluation for Rare Biochemical and Genetic Disorders, Kakkis said.

“We would hope to push for either regulatory change or legislation in the near future that establishes the new Office and provides the guidances needed to change the way Fast Track Orphan diseases are regulated,” Kakkis wrote in an e-mail. “The amendment is a key first step in that direction.”

The conference version also omits any reference to the number of FDA employees in each group. The older version of the amendment had set that number at eight employees. “It is unclear whether any additional guidance will be given as to the composition of each review group,” according to a blog maintained by the law firm Hyman, Phelps and McNamara.

The bill also would require the review groups to submit a report to Congress describing their findings and recommendations. FDA would issue guidance and develop internal review standards based on their recommendations. -- *Jennifer C. Smith*