

PATIENT GROUP SEEKS NEW FDA RARE DISORDER OFFICE IN APPROPS BILL

Date: August 28, 2009

An advocacy group for patients with rare diseases is urging that lawmakers strengthen in conference a provision in the Senate FDA appropriations bill that would establish two FDA review groups to recommend solutions for the prevention, diagnosis and treatment of rare and neglected diseases. The Kakkis EveryLife Foundation says the current language is a step in the right direction, but it would rather 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 bill says the FDA commissioner may establish two review groups to recommend "appropriate preclinical, trial design, and regulatory paradigms and optimal solutions for the prevention, diagnosis, and treatment" of rare and tropical or neglected diseases in the developing world. Each committee would consist of eight FDA disease experts.

Kakkis supports the provision but would rather have the bill create an FDA Office of Drug Evaluation for Rare Biochemical and Genetic Disorders. The bill should make it clear "that consolidated high level expertise in a new review office is needed to support the regulation of certain rare disease treatments," Kakkis President Emil Kakkis said. "The FDA should also be allocated funding for this and other initiatives to allow sufficient time and staff to do the critical and difficult work needed by patients." He said the review group assigned to work on rare disease issues could help define the roles and responsibilities for a new review office.

Lawmakers should also require FDA to establish the groups instead of asking the agency, Kakkis said. Brownback wants the language changed in conference, a source added. Brownback's office did not return several messages.

There has also been talk that Sen. Susan Collins (R-ME), who is on the conference committee, is hedging her support, a source said. Her office did not return messages, but Kakkis said he would like to meet with her staff.

National Institutes of Health Director Francis Collins supports the provision. The National Organization for Rare Disorders doesn't have a formal position on the language, Vice President for Public Policy Diane Dorman said. She said Brownback's staff consulted the group prior to introducing the amendment. Brown's office said it would be pleased if NORD endorsed the language but "believes the amendment stands on its own without the need for formal endorsements."

The provision also would require the FDA review groups to submit a report to Congress describing their findings and recommendations, and to issue guidance and develop internal review standards based on their recommendations. It appears to elaborate on language in the committee report that accompanies the Senate FDA spending bill, according to the FDA Law Blog, which is maintained by Hyman, Phelps & McNamara. According to the Senate report: "Where appropriate, FDA should engage in partnerships and collaborations to identify compounds that may be suitable to treat this subset of orphan diseases and work in a proactive way to identify compounds to treat such diseases." -- *Jennifer C. Smith*