

"The Gray Sheet"

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New FDA Groups For Rare, Neglected Diseases Could Speed Path To Market

Two new FDA review groups to recommend solutions for the prevention, diagnosis and treatment of rare diseases and neglected diseases of the developing world would help get treatments to market faster, patient advocates and lawmakers say.

Each group - one for rare diseases, the other for neglected diseases of the developing world - would recommend "appropriate preclinical, trial design and regulatory paradigms and optimal solutions for the prevention, diagnosis and treatment of rare diseases," according to the legislation.

By Oct. 8, both the House and Senate had approved an appropriations bill including the creation of a rare diseases group and neglected diseases group in FDA. The proposal was originally contained in a Senate floor amendment co-sponsored by Senators Sam Brownback, R-Kan., and Sherrod Brown, D-Ohio.

One year after the review groups have been established, the Brownback/Brown amendment requires FDA to submit a report to Congress with their findings and to issue, not later than 180 days after submission of the report, guidance and internal review standards based on the groups' recommendations.

According to the National Institutes of Health, a rare disease is one that affects less than 200,000 people in the U.S. at any given time. Neglected diseases are conditions that inflict severe health burdens on the world's poorest people. Many of them are prevalent in tropical climates in areas with unsafe drinking water, poor sanitation, substandard housing and little or no access to health care, NIH says.

Patient Advocacy Groups Weigh In

The Kakkis Everlylife Foundation, founded to create "science-based changes to public policy around rare diseases states on its ¹ [Web site](#) that a streamlined development path will shorten the timelines and reduce the financial risks of developing therapies for rare diseases.

Kakkis expects a "surge in development activity" for even extremely rare diseases that will allow patients with rare biochemical and genetic disorders to access effective treatment faster, according to Kakkis.

The Brownback/Brown amendment is the first step toward defining the problems and understanding the changes needed at FDA to tackle rare and neglected diseases, Kakkis says.

"It is truly an important amendment for the rare disease community because it states and identifies that the FDA and the current process to get drugs [and devices] to market and ... approved is broken ... and that changes need to be made," Nicole Boice, founder and president of The Children's Rare Disease Network, explained in an interview.

"Especially within the rare disease community and having children that are ill and dying, we need to come up with solutions, we need to come up with treatments much more quickly," she added.

Brownback had previously sponsored the "Elimination of Neglected Diseases Act" amendment to the FDA Reauthorization Bill of 2007.

In a related statement, Brownback cited World Health Organization statistics showing that roughly one billion people are affected by at least one tropical disease, such as tuberculosis, malaria, cholera and leprosy.

WHO estimates one in three people in the world is infected with dormant tuberculosis bacteria that can become active as a result of a weakened immune system, and there are presently no simple, rapid and accurate tests to diagnose tuberculosis.

Companies including BD are working with an organization called FIND (The Foundation for Innovative New Diagnostics) to create faster, more cost-effective tests for drug-resistant tuberculosis in developing countries (² ["The Gray Sheet" Feb. 4, 2008](#)).

"Private companies have the potential to be major players in the fight against neglected tropical diseases," Brownback stated.

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