

Stearns To Introduce Orphan Drug Bill With Biomarker Qualification Focus

A House Energy and Commerce Committee Republican says he plans to introduce by the end of the week legislation aimed at facilitating orphan drug use of FDA's accelerated approval process. The bill is backed by about 100 rare disease groups pushing for alternate methods to qualify surrogate endpoints for the development of drugs for rare diseases that lack prior clinical data to make the qualification.

A slew of rare disease groups are pushing the Unlocking Lifesaving Treatments Act that they say would help them access the accelerated approval process, especially for the more rare diseases that lack clinical data to qualify surrogate endpoints. Rep. Cliff Stearns (R-FL) said he plans to introduce the bill by the end of the week.

"FDA has not used all the tools available to them to help bring new drugs to market to treat rare and ultra-rare diseases," Stearns said in a statement to *Inside Health Policy*. "I'm developing legislation to codify accelerated approval into statute and allow the FDA to use the full scope of scientific data when reviewing surrogate endpoints for the rare disease and orphan drug under accelerated approval."

Stearns, chair of Energy and Commerce's subcommittee on oversight and investigations, along with other committee Republicans, including Rep. Michael Burgess (TX), called for a meeting with the agency this fall to discuss the regulations surrounding the approval of ultra-rare diseases, which affect a smaller patient population. Rare diseases affect fewer than 200,000 people in the United States.

Further, FDA's approval rates are slowing overall, Stearns said, highlighting medical devices, which are the subject of several reform bills in the House.

"In 1992, the FDA created an Accelerated Approval process to make new drugs available to treat dire conditions that lack alternative therapies," Stearns said in the statement. "However, the modern FDA's approval rate for drugs and medical devices has slowed immensely. I did a hearing earlier this year on the medical device side of things."

The accelerated approval process allows for the approval of drugs based on surrogate endpoints, provided they treat serious diseases and fulfill an unmet need.

The EveryLife Foundation for Rare Diseases has been spearheading the effort, hoping to attach the proposal to the reauthorization of the Prescription Drug User Fee Act. Foundation President Emil Kakkis has pushed the idea that FDA should not require prior clinical data to qualify surrogate endpoints when the data is often not available for more rare diseases. Other science could be used to make the determination and FDA would determine the threshold, according to the group's proposal.

"The bill does not lower the standard for drug approval but just makes it clear that FDA can focus its review on existing scientific information and not feel compelled to require clinical data, which is not available for the rarest of diseases," he said in a blog post touting the bill. "This change, small in focus, is the right step forward to opening the door to treatment of the forgotten and devastating disorders that are too rare for investors to care about, and too difficult (with respect to biology) for typical drug development."

Several other groups, including the Sanfilippo Foundation for Children, are also backing the effort. The group's founder Roy Zeighami characterized it as a "chicken and egg problem" because the data is not available to push through that component of the drug development process.

"Time is of the essence and I don't want to wait," said Zeighami, the father of a child with Sanfilippo syndrome. He noted that patients with degenerative disorders need access to accelerated approvals. — *Alaina Busch*