The Pink Sheet

US FDA Could Require Pediatric Cancer Drug Combo Studies Under Amended User Fee Bill

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- **NEWS**

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Executive Summary

House Energy & Commerce Committee adds Give Kids a Chance Act to user fee reauthorization package during markup. Bill was modified to exclude provision giving the FDA authority to require preclinical studies.

The US Food and Drug Administration would be authorized to require companies developing new combinations of cancer drugs to conduct pediatric studies under an amendment to user fee reauthorization legislation adopted by the House Energy & Commerce Committee.

At its 18 May markup of the measure, the committee voted unanimously in favor of an amendment by Rep. G.K. Butterfield, D-NC, to include his bill, Give Kids a Chance Act, H.R. 5416, in the user fee legislation. However, in response to apparent concerns raised by industry, a provision giving the agency authority to require preclinical studies as part of pediatric study plans for cancer drugs was dropped from the bill.

Butterfield introduced H.R. 5416 in September. It would amend the federal Food, Drug, and Cosmetic Act to establish additional authorities of the FDA regarding the conduct of pediatric investigations of molecularly targeted drugs to treat cancer.

Nancy Goodman, founder and executive director of Kids v Cancer, who was the driving force behind the legislation, said it addresses a shortcoming of the Race for Children Act. That measure, included in Section 504 of the FDA Reauthorization Act (FDARA), the user fee reauthorization legislation of 2017, requires pediatric studies of certain molecularly targeted cancer drugs. (Also see "Pediatric Cancer Study Requirements Added To FDA User Fee Bill" - Pink Sheet, 11 Jul, 2017.)

Goodman said that requirement was only directed at single agent studies, while cancer scientists have said that combinations provide the best odds of achieving successful treatment.

If and when the current bill is passed into law “it will give translational oncology researchers the opportunity to have clinical trials with combination therapies,” she said. “I think it will bring better therapies for kids with cancer and hopefully curative therapies.”

Building On Past Legislation

The Give Kids a Chance Act expands on the Race for Children Act, which expanded the reach of the Pediatric Research Equity Act to cancer. PREA gave FDA the authority to require pediatric studies in certain drugs and biological products. However, its impact on pediatric oncology was limited because adult cancers for which drugs are approved tend to occur in different organs than childhood cancers. FDARA also eliminated the PREA exemption for orphan-designated drugs.

Goodman said inclusion of the Give Kids a Chance Act in the user fee reauthorization package required extensive negotiations as the Pharmaceutical Research and Manufacturers of America raised concerns about it.

“My understanding is that PhRMA tried to kill the bill,” she told the *Pink Sheet*. “I didn’t understand why PhRMA was opposed to a bill that could potentially bring curative therapy to terminally ill children with de minimis burdens on companies.”

A spokesperson for PhRMA said it has not taken a position on the bill.