Pharma Companies to Receive Incentives to Address Unmet Pediatric Medical Need

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Legislation by representatives Michael McCaul (R, TX) and G.K. Butterfield (D, NC), offering drug companies millions of dollars in incentives to develop treatments for rare pediatric disease, was recently signed by the president.

New legislation created to address unmet medical need for children was recently signed into law. The Creating Hope Act, spearheaded by representatives Michael McCaul (R, TX) and G.K. Butterfield (D, NC), aims to incentivize pharmaceutical companies to develop treatments for rare pediatric diseases, including certain types of cancer and sickle cell anemia.

Since 1980, the FDA has only approved 1 new drug expressly for the treatment of a childhood cancer, but has developed dozens of cancer treatments for adults. Passage of the Act would allow pharmaceutical companies to receive vouchers to expedite the FDA review process of their more profitable, blockbuster drugs in exchange for the development of treatments for rare pediatric diseases.

Developing treatments for children is notoriously difficult; clinical trials containing subjects younger than 18 years are controversial, and Pharma companies are reluctant to "make an investment in products that are unlikely to cover the high costs associated with their research, development, marketing, and distribution," according to a press release from Rep. McCaul's camp.

The priority review voucher concept was first introduced in 2007, when Sherrod Brown (D, OH) sponsored an amendment to the FDA Amendments Act of 2007. This amendment relied on a similar voucher arrangement in order to generate funds for the creation of new drugs and vaccines for the treatment of neglected tropical diseases. The Creating Hope Act would extend Section 524 of the Federal Food, Drug, and Cosmetic Act, the legislation that creates a priority review voucher for tropical diseases.

Rep. McCaul is confident that the Creating Hope Act will help change the way drug companies look at rare pediatric disease. "The Creating Hope Act has the ability to actually save lives and hand down a better America to our children," he said. "In times of tough budgetary constraints, this legislation incentivizes pharmaceutical companies to finally start creating new cures for rare pediatric diseases with no cost to taxpayers."

According to Kids v Cancer, a nonprofit organization supporting pediatric cancer research, the Creating Hope Act legislation would:

- Expand the priority review voucher program to include treatments for pediatric cancers
- Close a loophole in current law to prevent companies from receiving a voucher for products they already market in other countries
- Offer limited transferability of vouchers to create a more easily traded asset
- Provide greater certainty to sponsors that their new drug, if approved, would qualify for a voucher, by allowing them to seek a designation by the FDA before they submit their new drug application
- Require sponsors to submit a statement of good faith intent to market the eligible drug, as well as a report describing the demand and distribution of the ultimate product
- Add Chagas disease to the list of neglected tropical diseases to Section 524 of the Federal Food, Drug, and Cosmetic Act.

Congress estimates that drug manufacturers who participate with the voucher program could make up to $500 million in additional sales on blockbuster medications as a result of a shortened FDA review period, according to reports.