

# ROLL CALL

## **McCaul and Butterfield: Creating Hope for Kids With Rare Diseases**

By Reps. Michael McCaul and G.K. Butterfield  
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Imagine your child fighting for his life, taking massive doses of highly toxic radiation, chemotherapy to kill the cancer in his body before it kills him. After years of pain and uncertainty, he beats the disease, only to find out that he will likely develop serious medical conditions related to the treatment that saved him.

While survival rates are up for some types of pediatric cancer, for more than half who beat the odds and proudly call themselves “survivors,” the fight is not over. Three out of five are later afflicted with life-altering and life-threatening conditions, including secondary cancers, as a result of harsh treatments developed for adults.

Since the 1980s, the Food and Drug Administration has approved only one new treatment for any type of childhood cancer. This is compared with 50 approved treatments for adult cancers during the same time period.

So why, despite this significant unmet medical need, is more not being done? The problem, as is often the case, is money.

Pharmaceutical companies have been reluctant to develop drugs for rare pediatric diseases because it requires making an investment in products that do not cover the high costs associated with their research, development, marketing and distribution.

The good news is that there is now hope for children living with cancer and other rare pediatric diseases. The Creating Hope Act, which we introduced last week, would give pharmaceutical companies an incentive to develop treatments by strengthening the FDA priority review voucher program. In short, it would allow pharmaceutical companies to receive faster FDA review of more profitable drugs in return for developing treatments for rare pediatric diseases, at no cost to taxpayers.

Pharmaceutical companies can receive a priority review voucher now if they develop novel treatments for neglected tropical diseases, such as malaria and leprosy, entitling the company to

a priority six-month review of another new drug application that would otherwise be reviewed under the FDA's standard 10-month period.

The Creating Hope Act would expand this program to offer pharmaceutical companies the same shortened review time in return for development of new treatments for children with rare diseases. This shortened review time, which can lead to earlier market entry, is estimated to be worth hundreds of millions of dollars.

While survival rates have greatly improved for many rare pediatric diseases, too many families lose children to diseases for which no treatments, or no age-appropriate ones, exist. More than 2,400 children die of cancer each year, making it the No. 1 disease killer of American children. Children who suffer from rare pediatric diseases such as cancers, AIDS, cystic fibrosis, Tay-Sachs and sickle-cell disease, make up the vast majority of the 30 million Americans who suffer from rare diseases, according to the National Organization for Rare Disorders.

Until now, pharmaceutical companies have been able to conveniently dismiss this unmet medical need as too costly and as a threat to business. Not anymore.

The Creating Hope Act will give these children a voice in Congress. It is time for pharmaceutical companies to come to the table and to work on their behalf. Congress is listening — we must pass the Creating Hope Act.

*Rep. [Michael McCaul](#) (R-Texas) is the founder and co-chairman of the Childhood Cancer Caucus, of which Rep. [G.K. Butterfield](#) (D-N.C.) is a member.*