Austin American-Statesman

McCaul and Butterfield: Hope for Children with Cancer

Reps. Michael McCaul and G.K. Butterfield, U.S. House of Representatives

Published: 7:10 p.m. Thursday, Sept. 22, 2011

Imagine your child fighting for his life, taking massive doses of highly toxic radiation and 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.

Though survival rates are up for some types of pediatric cancer, for more than half of those 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 as a result of harsh treatments developed for adults, including secondary cancers.

Since the 1980s, the Food and Drug Administration has approved only one new treatment for any type of childhood cancer. This compares 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, comes down to 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 in the U.S. House of Representatives this 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.

Presently, pharmaceutical companies can receive a priority review voucher 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 review period.

The Creating Hope Act would expand this program to offer pharmaceutical companies the same shortened FDA 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.

Though 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 treatments, exist. More than 2,400 children die of cancer each year, making it the number one disease killer of American children, more than asthma, diabetes, cystic fibrosis and AIDS combined.

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.

Children are not old enough to vote and they do not have lobbyists fighting for them in Washington — and yet many of them are sick and in need of new treatments. 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.

McCaul, R-Texas, is founder and co-chairman of the Childhood Cancer Caucus. Butterfield, D-North Carolina, is a member of the caucus.