Congress of the United States
Washington, DC 20515

September 23, 2011

Cosponsor the Creating Hope Act of 2011

Provides market incentives to pharmaceutical companies to develop drugs for pediatric rare diseases

Dear Childhood Cancer Caucus Colleague:

We urge you to help incentivize the development of new treatments for children with rare pediatric diseases by co-sponsoring H.R. XXXX, the *Creating Hope Act of 2011*, which we recently introduced. Senators Bob Casey (D-PA) and Scott Brown (R-MA) have introduced a companion bill, S. 606, in the Senate.

The Creating Hope Act would expand and strengthen the cost-neutral FDA priority review voucher (PRV) program, giving pharmaceutical companies an incentive to develop treatments for rare diseases that are often less profitable than treatments for more common medical conditions. Currently, pharmaceutical companies can receive a priority review voucher if they develop novel treatments for neglected tropical diseases, entitling the company to a priority six month review of another new drug application that would otherwise be reviewed under the FDA's standard ten month review period. This shortened review time, which can lead to earlier market entry, is estimated to be worth hundreds of millions of dollars.

Rare pediatric diseases, such as childhood cancers, affect significant numbers of American children and their families. Thirty-six children are diagnosed with pediatric cancer each day, and 2,300 children lose their lives each year to the disease, making it the number one disease killer of American children. For those who are fortunate enough to survive this grueling battle, three out of five are once again afflicted with life-threatening and life-altering conditions as a result of harsh treatments developed for adults.

Since the 1980s, only *one* pharmaceutical drug to treat *any* type of pediatric cancer has been approved by the FDA. Despite this significant unmet medical need, pharmaceutical companies have been reluctant to develop drugs for pediatric rare diseases because it requires making an investment in products that are unlikely to cover the high costs associated with their research, development, marketing and distribution. This is a tragedy for thousands of childhood cancer victims and their families.

We hope you will cosponsor the *Creating Hope Act,* which has broad bipartisan and bicameral support, and is strongly endorsed by the medical community, patient advocates and biopharmaceutical companies. For more information, or to become a co-sponsor, please contact Andy Taylor in Congressman McCaul's office at 5-2401 or andy.taylor@mail.house.gov or Tonya Williams in Congressman Butterfield's office at 5-3101 or tonya.n.williams@mail.house.gov.

Sincerely,

Michael T. McCaul
Member of Congress
Co-Chairman, Childhood Cancer Caucus

G.K. Butterfield
Member of Congress
Member, Childhood Cancer Caucus

Chris Van Hollen
Member of Congress
Co-Chairman, Childhood Cancer Caucus

Sue Myrick
Member of Congress
Member, Childhood Cancer Caucus

The Creating Hope Act of 2011

H.R. XXXX, the *Creating Hope Act of 2011*, will build on the cost-neutral priority review voucher (PRV) program, established under the FDA Amendments Act of 2007, to generate stronger market incentive for companies to invest in new treatments for rare diseases affecting children.

The existing PRV program is designed to spur investment in new treatments and cures for rare and neglected tropical diseases by providing them with a priority review voucher. A priority review voucher entitles the company to receive a "priority" 6-month review of another new drug application that would otherwise be reviewed under FDA's standard 10-month review period. This shortened review time, which can lead to earlier market entry, is estimated to be worth hundreds of millions of dollars.

The *Creating Hope Act* will:

- Expand the priority review voucher program to include treatments for rare pediatric diseases, such as childhood cancers and cystic fibrosis.
- Close a loophole in current law to prevent companies from receiving a voucher for products that they already market in other countries.
- Offer unlimited transferability of vouchers to create a more easily traded asset.
- Provide greater certainty to sponsors by permitting them to seek a designation from FDA before they submit their new drug application that the drug, if approved, will qualify for a voucher.
- Strengthen reporting and marketing requirements by requiring that the sponsor submit a statement of good faith intent to market the eligible drug.
- Add Chagas disease, which is responsible for more deaths in Central and South America than every other parasite-borne disease, to FDA's list of neglected tropical diseases.

The following groups and businesses have endorsed the legislation: Aeras Global TB Vaccine Foundation, American Porphyria Foundation, American Society of Clinical Oncology, American Society of Pediatric Hematology and Oncology, American Society for Tropical Medicine and Hygiene (ASTMH), Association of Pediatric Oncology Social Workers, Bio Ventures for Global Health, Chai Lifeline, Children's Brain Tumor Foundation, Children's Cause for Cancer Advocacy (CCCA), Children's Hospital Association of Texas, Children's Hospital of Philadelphia, Children's Hospital of Pittsburgh of UPMC, Children's Medical Center Dallas, CSL Behring, CureSearch, GlaxoSmithKline, Genzyme, The Kakkis EveryLife Foundation, Kids v Cancer, Leukemia & Lymphoma Society, Madisons Foundation, Medicines for Malaria Venture (MMV), Merck, Metronomix, The National Association of Children's Hospitals, The National Children's Cancer Society, National Organization for Rare Disorders (NORD), The National Tay-Sachs & Allied Diseases Association, Novartis, Pennsylvania BIO, Sarcoma Foundation of America, Shire, St. Baldrick's Foundation, TB Alliance.