2nd Annual Childhood Cancer Summit
Hosted by the House Childhood Cancer Caucus
September 23, 2011

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On behalf of the Children’s Oncology Group, I want to thank Representatives McCaul and Van Hollen, members of the Congressional Childhood Cancer Caucus, other distinguished members of Congress and guests here today, for this opportunity to speak with you about the future of childhood cancer drug development.

We are entering an era of unprecedented discovery of the underlying molecular bases of childhood cancers. The research tools we have today, and the current pace of discovery, were difficult to imagine as recently as five short years ago. Yet if we are not able to turn these discoveries into better treatments for children with cancer, then we run the risk of failing the next generation of children with cancer we are called upon to care for.

When one examines the entire pediatric drug development landscape, and not just the pediatric cancer drug development landscape, there is no question that the Best Pharmaceuticals for Children Act (BPCA) and the Pediatric Research Equity Act (PREA) have had a major impact on the lives of children. The incentives afforded the biopharmaceutical industry to conduct appropriate drug development studies in children in large measure have succeeded, with more such studies conducted over the past 10 years than likely conducted over the 5 decades prior to legislative implementation. The impact of BPCA on childhood cancer drug development, however, has been more modest, and the impact of PREA thus far minimal. Moving forward, the pediatric cancer research community will need to form an increasing number of partnerships with industry if we are to fully capitalize on scientific discoveries and improve the outcome for all children with cancer. As these important pieces of legislation approach the time for renewal, let me highlight areas that could strengthen their impact.

Let’s begin with PREA. FDA approved indications for almost all cancer drugs focuses on the drug’s use in a specific disease, such as colon cancer, lung cancer, breast cancer, and so forth. PREA applies only to those drugs developed for diseases that occur in both the adult and pediatric populations. Waivers can be granted for most new cancer drugs, as the common cancers observed in adults essentially do not occur in children. The key point, however, is that the molecular target of new cancer drugs may indeed be important for childhood cancers. An example can highlight this current limitation of PREA.

This past year a new drug, crizotinib, received FDA approval for a type of lung cancer. The new drug targets a specific molecular target, called ALK. In a small proportion of
patients with lung cancer, ALK has undergone a rearrangement, or a molecular change, in the tumor. The new drug is a true advance for patients with ALK positive lung cancers. However, since lung cancers do not occur in children, there is no requirement under PREA to conduct pediatric studies.

It turns out that a subset of a very difficult to treat childhood cancer called neuroblastoma may also be dependent on ALK, though not in a manner identical to lung cancer. Although studies are underway to determine the role of crizotinib in children with neuroblastoma, but there is no regulatory requirement under PREA to pursue such studies.

Part of the challenge for PREA then is that the manner in which drugs are labeled by the FDA has not yet fully caught up with the pace of science. Drugs are still primarily labeled for specific diseases. Improved PREA legislation can address this by including new drugs for which the drug target has a potential role in childhood cancer, even though the respective cancers in adults and children are different.

Incentives can have a major impact on pediatric drug development. The incentive offered through BPCA, however, occurs late in the life cycle of a drug. In other words, companies have time to estimate the true value of exclusivity extensions by waiting until the drug is on the market and its market value becomes known. The real need for the childhood cancer community is to start pediatric studies at an earlier time point, which is almost always prior to drug approval for the adult indication. Thus exploring mechanisms under BPCA renewal that would incentivize earlier initiation of pediatric studies could further increase the impact of this legislation.

Neither BPCA nor PREA incentivizes the biopharmaceutical industry to pursue development of new cancer drug intended specifically for a childhood cancer. That is where the Creating Hope Act can have an impact. This new legislation can help industry partner with the pediatric cancer research community in developing drugs against targets that are specific to pediatric cancers. With cancer remaining the leading cause of disease related death in children, and too many survivors of childhood cancer experiencing lifelong side effects or current day treatment, there is no question that better treatments are need. Adding the Creating Hope Act to the legislative armamentarium in our fight against childhood cancer is urged.

On behalf of the greater than 7,000 childhood cancer experts who comprise the Children’s Oncology Group, we thank you for all that you have done and will do for children with cancer,