POLITICS, POLICY & LAW

TARGETING PREA

BY STEVE USDIN, WASHINGTON EDITOR

Pediatric cancer advocates are renewing calls for modernizing the Pediatric Research Equity Act to make it more applicable to cancers that afflict children by changing the way cancers are defined.

For diseases that occur in both adults and children, PREA requires drug companies to study their products in children.

In September 2013, Peter Adamson, chair of the **Children's Oncology Group** (COG), told the congressional childhood cancer caucus that while PREA has stimulated pediatric drug development for other diseases, its effect on pediatric cancer has been "minimal."

"Waivers can be granted for most new cancer drugs, as the common cancers observed in adults essentially do not occur in children," he said.

The reason is that cancers are still defined by the tissue of origin rather than by molecular or genetic signatures, and because the vast majority of adult cancers — such as prostate and breast cancers — do not occur in children.

"The key point is that the molecular target of new cancer drugs may indeed be important for childhood cancers," said Adamson, who is also chief of the Division of Clinical Pharmacology and Therapeutics at the **Perelman School of Medicine at the** University of Pennsylvania.

In an interview broadcast on *BioCentury This Week* television, pediatric cancer advocate Nancy Goodman echoed the theme. "Now that we're studying cancer drug development by a genomic mutation, and kids' cancers have that same mutation, we need to ensure that drug companies study drugs in kids," she said.

Goodman is executive director of pediatric cancer advocacy group **Kids v Cancer**.

Richard Schilsky, CMO of the **American Society of Clinical Oncology** (ASCO), told BioCentury that modifying PREA to make cancer research requirements based on molecular targets "makes sense."

The approval of drugs like Xalkori crizotinib from Pfizer Inc. and Zykadia ceritinib from **Novartis AG** to treat anaplastic lymphoma kinase (ALK)-positive non-small cell lung cancer (NSCLC) illustrates the case for changing PREA, he said.

"There are drugs on the market to treat ALK-translocated non-small cell lung cancer that are effective," Schilsky noted. "The same translocation occurs in neuroblastoma in children. If you had a new targeted therapy that hits a mutation in a common adult cancer but also occurs in a completely different pediatric cancer, making that new lung cancer drug available for neuroblastoma makes sense."

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Schilsky added: "Not every targeted therapy will necessarily translate from an adult to a pediatric tumor, but it is well worth considering."

Rep. Chris Van Hollen (D-Md.), co-chair of the congressional childhood cancer caucus, told *BioCentury This Week* television that updating PREA to create mandates for testing targeted cancer drugs in children is "something that we should do either legislatively or administratively. And I think one question we're going to be putting to the FDA is what authority do they have currently to make those changes."

Rep. Michael McCaul (R-Texas) agreed. "We should look at modernizing and updating the law to the modern science," the lawmaker told *BioCentury This Week*. "The current law is not working for children and childhood cancer patients."

BioCentury This Week's interview with Goodman, McCaul and Van Hollen will be broadcast at 8:30 a.m. on Sunday, Sept. 28, in Washington, D.C., on WUSA Channel 9, and at various times on select PBS stations. It will be available online at biocenturytv.com starting at 9 a.m.

COMPANIES AND INSTITUTIONS MENTIONED

American Society of Clinical Oncology (ASCO), Alexandria, Va.
Children's Oncology Group (COG), Monrovia, Calif.
Kids v Cancer, Washington, D.C.
Novartis AG (NYSE:NVS; SIX:NOVN), Basel, Switzerland
Perelman School of Medicine at the University of Pennsylvania, Philadelphia, Pa.
Pfizer Inc. (NYSE:PFE), New York, N.Y.