Source: Kids v Cancer
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Do Kids with Cancer Deserve Drugs?

Now that Childhood Rare Disease Drugs are Profitable, They Might Get Them

WASHINGTON (September 8, 2014) -- As we mark the fourth annual observance of National Childhood Cancer Awareness Month, Kids v Cancer celebrates recent achievements in pediatric drug development.

The greatest hurdle in battling childhood cancer and rare diseases has long since been the lack of treatments developed specifically for children. Most sick children are left with pass-me-down adult drugs due to a lack of funding and trials for pediatric drugs.

"Out of 900 drugs in the cancer pipeline, almost none are for kids," said Nancy Goodman, Founder and Executive Director of Kids v Cancer. "That is about to change."

Congress passed the Creating Hope Act in 2012 to create market-based incentives that allow companies to pursue treatments tailored specifically for our children.

On July 30th, we saw the first use of the legislation's FDA voucher program when BioMarin sold a Creating Hope Act priority review voucher for $67.5 million. This deal provides proof that good, risk-adjusted returns can be made by investing in pediatric drug development.

BioMarin received the voucher for developing Vimizim - a drug that combats Morquio A Syndrome, a terrible childhood disease. Regeneron and Sanofi purchased the voucher to receive faster FDA review of another drug they have under FDA review.

"We have now reached a tipping point in pediatric drug development," said Goodman. "Where it was once financially unfeasible, businesses now have a reason to develop drugs for kids with cancer and other rare diseases."

Through a tremendous partnership between the public and private sectors, there is now, for the first time, a path for medical breakthroughs in treating our children's worst afflictions. While seizing this opportunity, we must also innovate and identify new strategies to create more pediatric cancer and rare disease drugs.

Learn more at www.kidsvcancer.org

Kids v Cancer is a 501(c)(3) organization focused on changing the landscape of pediatric

cancer research by identifying structural impediments at key junctures in the research process – new drugs, tissue donation and access to funding – and developing strategies to address them.

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