



KIDS **V** CANCER

2014 ANNUAL REPORT

Friends of Kids v Cancer: **Thank you**

With your support, we have achieved notable results this year in our efforts to change the landscape of pediatric cancer.

Proving the Creating Hope Act
a Success: Priority Review
Vouchers Mobilized Nearly
\$200 million for Research and
Drug Development

As you may recall, in 2012, Kids v Cancer succeeded in getting the Creating Hope Act passed by Congress and signed by President Obama. It created a market incentive for the development of drugs for pediatric cancer and other rare pediatric diseases by creating “priority review vouchers” which are transferable and which allow the holder to secure faster FDA review of another new drug. The theory was that such priority review vouchers would prove valuable and would, therefore, encourage pediatric drug development.

The great news this year: The theory worked.

On July 31st, the first Creating Hope Act voucher was sold by BioMarin to Sanofi/Regeneron for \$67.5 million, allowing Biomarin to reinvest the proceeds in further drug development for rare diseases.

But that was just the start. Last month, based on Biomarin’s success, Knight Therapeutics auctioned off a priority review voucher it had earned for developing a drug for a tropical disease for \$125 million to Gilead.

These valuations are proof of the principle that good risk adjusted returns on investment can be made by investing in drug development for pediatric rare diseases, including cancer.

These successes are already having a profound impact on the landscape of pediatric cancer research. We have heard from numerous companies that are now increasing the priority they put on pediatric drug development because of this market incentive. Moreover, we have heard from many academic centers and philanthropic organizations that intend to use this program to finance their current and future research.

Secured FDA Draft Guidance on Pediatric Priority Review Vouchers

In November of this year, the Creating Hope Act hit another milestone when the FDA published its draft guidance for the program. This guidance provides additional information to companies and researchers as to how the FDA intends to implement the Creating Hope Act. We look forward to providing public comments to the FDA on the draft guidance as it undertakes a final draft this winter.

The Creating Hope Act Goes to Europe

Kids v Cancer continues to work with a consortium of advocates, pharmaceutical executives, pediatric oncologists and European regulators to identify strategies to create incentives for pediatric cancer

research in Europe. As part of that effort, our European counterparts have expressed strong interest in the Creating Hope Act, and we are engaging in a series of meetings and conferences to change the landscape of pediatric cancer drug development on both sides of the Atlantic.

Encouraging Companies to Test Promising Cancer Drugs on Kids

The Creating Hope Act is playing an important role in incentivizing the development of drugs specifically for pediatric cancer.

In the meantime, there is a pipeline of 900 drugs for adult cancers which could benefit kids with cancer, but many of those opportunities are never realized because companies discontinue development of the drugs if they do not prove to be effective for adult indications before they ever test the drugs in children. Kids v Cancer is looking forward

to working with Members of Congress and officials of the National Institute for Health, National Cancer Institute and U.S. Food and Drug Administration to identify strategies to address this challenge next year.

In addition, for those drugs in the adult cancer pipeline that are approved for adult cancers, kids with cancer often must wait years after the drugs have been approved before they can benefit from pediatric clinical trials of those drugs. Kids v Cancer has been working with and advising pediatric cancer researchers on strategies available under Best Pharmaceuticals for Children Act (BPCA) to win earlier access of drugs under development for adult indications.

Advocating for Expanded Access and Compassionate Use

In April, 2014, Kids v Cancer joined with other pediatric cancer advocates and Bill Burns to press for access to a life saving drug from Chimerix for Bill's 7-year old nephew, Josh Hardy, who suffers from kidney cancer. We approached the CEO and Board Members of Chimerix, undertook a groundbreaking social media campaign and attracted national television attention. In the end, Josh was able to receive the drug and survived.

The #SAVEJOSH campaign marks a turn in compassionate use access. Too many children are limited in their ability to access exciting new investigational drugs because drug companies undertake pediatric clinical research so late in the drug development process and because companies are reluctant to provide compassionate use access to kids.

Since April, as a consequence of the #SAVEJOSH campaign, compassionate use reform has been a priority topic in Washington, DC Nancy has been interviewed by numerous media outlets on the subject, including BioCentury, CNBC, PBS and BusinessWeek.

Encouraging Tissue Donation

Kids v Cancer continues to facilitate the donation of tumor tissue of terminally ill children with pediatric brain cancers to academic researchers. This is resulting in exciting advances in the understanding of pediatric brain cancers is drawing young researchers to pediatric brain cancer as a field of interest. We are grateful for the generous donations of families who have lost children and chose this brave step.

Raising Awareness of Pediatric Cancer Issues

Kids v Cancer has continued to work hard to raise awareness about the challenges of pediatric cancer research and drug development. This year, Nancy was honored to be chosen as a member of “The One Hundred” top cancer leaders by Massachusetts General Hospital. She also served as a member of the National Cancer Institute (NCI) Board of Scientific Counselors, the FDA Pediatric Oncology Drug Advisory Committee (ODAC), and the Department of Defense Congressionally Directed Medical Research Program (CDMRP). In addition, Nancy was asked to speak at the BIO CEO Forum, the National Organization for Rare Disease (NORD) Annual Meeting, Drug Information Association (DIA), plus numerous Congressional events and conferences at the US Food and Drug Administration (FDA), the National Cancer Institute (NCI) and the National Press Club.

This year, Nancy appeared on CNBC, BioCentury, Fox and Friends and PBS. The work of Kids v Cancer and the priority review voucher were discussed in dozens of articles, including in the Wall Street Journal, BusinessWeek, the Financial Times, the San Francisco Business Times and Readers Digest. We have many listed on the NEWS page of our website, Kidsvcancer.org.

Organizing Kids v Cancer Youth Leaders

Kids v Cancer is proud to announce that this year, we organized a team of high school youth leaders to raise awareness of pediatric cancer issues and promote pediatric research and drug development through volunteer work in New York City as well as advocacy in DC. We look forward to building on this program next year as we create our first Youth Board.

Building Kids v Cancer

Kids v Cancer continues to operate in a highly efficient manner. In addition to our terrific current staff, Jenn Flynn, who runs our social media and community outreach program, and Kunal Joshi, who is our program director, we are pleased to welcome Katie Miller, a health care economist, to the Kids v Cancer team.

We have only been able to accomplish what we have done because of the contributions of family and friends like you. Nancy volunteers her time and to be as effective as possible at developing policy, DC, Kids v Cancer does not accept financial contributions from the pharmaceutical industry. Thank you, as always, for your generous support. With it, we look forward to making 2015 another milestone year.

Warmest regards,
Nancy Goodman

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Changing the landscape of pediatric research

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