

**PEDIATRIC CANCER
LEGISLATIVE YEAR BOOK
2016**

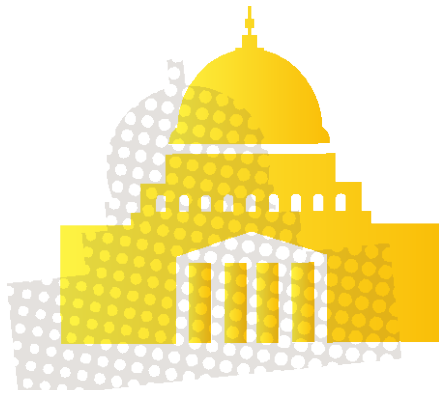


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The Pediatric Cancer Yearbook 2016 was compiled by Kids v Cancer, www.kidsvcancer.org, info@kidsvcancer.org. All efforts were made to contact the lead advocates of each program to review the program summaries.

If you would like to be on the mailing lists of all the legislative efforts and their lead advocates, please sign up here: <http://bit.ly/2kDmo1x>.

BILLS PASSED INTO LAW IN 2016

**CREATING HOPE ACT REAUTHORIZATION
PEDIATRIC PRIORITY REVIEW VOUCHER (PEDIATRIC PRV) 21 U.S.C. 360ff**

What Is the Problem/Opportunity:

Until recently, there was very little development of drugs expressly for children. The problem was that although pediatric cancer is so terrible, few children have cancer. The markets were too small for drug companies to develop drugs for kids with cancer. The only drugs available to children with cancer were drugs first developed for adults that also happened to benefit children.

In 2012, Congress passed the Creating Hope Act as a pilot program to create a financial incentive for companies to develop drugs expressly for kids with rare diseases, including pediatric cancers.

The Creating Hope Act works on a voucher system and has been wildly successful. Creating Hope Act vouchers have been sold for as much as \$350 million, and about \$1 billion of vouchers have been traded, constituting a powerful incentive for pediatric cancer and rare diseases drug development. Now, there is more interest in drug development for kids with cancer.

Pursuant to the Creating Hope Act, a company that develops a drug for pediatric cancer or another pediatric rare disease – and receives FDA approval – also receives from the FDA a Priority Review Voucher. The voucher comes with rights to faster FDA review of any future drug, enabling the voucher holder to receive a FDA “priority review” instead of a “standard review.” And, the voucher is transferable.

The Creating Hope Act was set to expire in 2016.

2016 Accomplishments:

In December 2016, as part of the 21st Century Cures Act, Congress reauthorized the Creating Hope Act.

Pursuant to Congress’ Creating Hope Act reauthorization, drugs that receive rare pediatric designations by September 20, 2020 will have until September 20, 2023 to earn a voucher. The Creating Hope Act will expire in September 2020.

The Creating Hope Act was championed by Representative Michael McCaul, G.K. Butterfield and Chris Van Hollen; and by Senators Bob Casey and Johnny Isakson.

Next Steps:

Advocates will begin to prepare to ask Congress to reauthorize the Creating Hope Act again in 2020. To prepare for that reauthorization, Kids v Cancer welcomes contact from companies seeking designations for vouchers.

For More Information, Please Contact:

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BILLS AND APPROPRIATIONS PROVISIONS ON 2017 LEGISLATIVE AGENDA

**RACE FOR CHILDREN ACT OF 2016 (S 3239, HR 5858)
RESEARCH TO ACCELERATE CURES AND EQUITY FOR CHILDREN ACT**

What Is the Problem/Opportunity:

The Creating Hope Act addresses drug development expressly for children. However, it does not ensure that drugs developed for adults will also be developed for children.

In fact, there are 900 drugs in the adult cancer pipeline but few of them are studied for children with cancer. Those drugs that are studied in children are often studied years and even decades after they are developed for adults.

The RACE for Children Act of 2016 (S 3239, HR 5858) provides that companies developing drugs for adults with cancer also undertake certain pediatric studies. If the RACE for Children Act is passed, children with cancer will have a much greater chance of being treated with the newest and most promising therapies that are in adult trials. Pediatric oncologists who seek exciting new drugs for pediatric cancer clinical trials will be able to get supplies of the drugs and their studies may be funded by the drug company. The RACE for Children Act will result in many more drugs available to kids with cancer, and will dramatically increase industry financing of pediatric cancer research.

The RACE for Children Act does not create a new obligation for drug companies. The RACE for Children Act modifies an existing law, the Pediatric Research Equity Act (PREA) that requires companies developing drugs for adults to also undertake drug studies for kids. Until now PREA has not applied to kids with cancer because kids often have cancers in different organs than do adults. However, cancer drug development is now guided by molecular targets that are often present in both adult and pediatric cancers. The RACE for Children Act updates PREA so that the PREA pediatric studies will be required by molecular target as well. In addition, the RACE for Children Act ends an exemption to PREA requirements for cancer drugs that have orphan status and are relevant to a pediatric cancer as a result of their molecular target.

2016 Accomplishments

In July 2016, a bipartisan, bicameral the RACE for Children Act was introduced into the Senate and House of Representatives by Senators Michael Bennet and Marco Rubio, and Representatives Michael McCaul, G.K. Butterfield, Chris Van Hollen and Sean Duffy.

The RACE for Children Act was not passed into law by the House or Senate.

The RACE for Children Act has been endorsed by *Nature Journal*, and by scores of hospitals and over a hundred of advocate organizations.

Next Steps:

2017 begins a new Congress, the 115th Congress. Advocates will ask the Senate and House of Representatives to re-introduce the RACE for Children Act. Advocates will then ask Congress to pass the RACE for Children Act into law as part of the Prescription Drug User Fee Act (PDUFA) in May–July, 2017.

For More Information, Please Contact:

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STAR ACT OF 2015 (S 1883, HR 3381)
CHILDHOOD CANCER SURVIVORSHIP, TREATMENT, ACCESS, AND RESEARCH ACT

What Is the Problem/Opportunity:

The Childhood Cancer Survivorship, Treatment, Access, and Research (STAR) Act would expand opportunities for childhood cancer research, improve efforts to identify and track childhood cancer incidences, enhance the quality of life for childhood cancer survivors, and ensure publicly accessible expanded access policies that provide hope for patients who have run out of options.

Expanding Opportunities for Childhood Cancer Research: Due to the relatively small population of children with cancer and the geographic distance between these children, researching childhood cancer can be challenging. As such, the Childhood Cancer STAR Act would authorize the National Cancer Institute (NCI) to expand existing efforts to collect biospecimens for childhood cancer patients enrolled in NCI-sponsored clinical trials to collect and maintain relevant clinical, biological, and demographic information on all children, adolescents, and young adults with cancer.

Improving Childhood Cancer Surveillance: Building upon previous efforts, this bill would authorize grants to state cancer registries to identify and track incidence of child, adolescent, and young adult cancer. This funding would be used to identify and train reporters of childhood cancer cases, secure infrastructure to ensure early reporting and capture of child cancer incidence, and support the collection of cases into a national childhood cancer registry.

Improving Quality of Life for Childhood Cancer Survivors: Unfortunately, even after beating cancer, as many as two-thirds of survivors suffer from late effects of their disease or treatment, including secondary cancers and organ damage. This legislation would enhance research on the late effects of childhood cancers, including a study on insurance coverage and payment of care for childhood cancer survivors; improve collaboration among providers so that doctors are better able to care for this population as they age; and establish a new pilot program to begin to explore innovative models of care for childhood cancer survivors.

Ensuring Patients Access to Publicly Available Compassionate Use Policies: Compassionate use – the process by which a patient with a serious or life-threatening illness can be granted access to therapies still in development and outside of the clinical trial setting, when there is no comparable alternative – is often challenging for patients and their doctors to navigate. This legislation would ensure that pharmaceutical companies have publicly accessible compassionate use policies and would require FDA to finalize its guidance and address regulatory uncertainties for industry on the issue.

Ensuring Pediatric Expertise at the National Institutes of Health (NIH): The Childhood Cancer STAR Act would require the inclusion of at least one pediatric oncologist on the National Cancer Advisory Board and would improve childhood health reporting requirements to include pediatric cancer.

2016 Accomplishments

In December 2016, the House passed the STAR Act by unanimous consent with 270 cosponsors.

The STAR Act was not passed into law because the Senate did not vote on the STAR Act before the conclusion of the 114th Congress.

The compassionate use provisions of the STAR Act, which were also in the Andrea Sloane CURE Act (HR 909), were passed as part of 21st Century Cures.

Next Steps:

2017 marks a new Congress, the 115th Congress. Advocates will ask the Senate and House of Representatives to re-introduce the STAR Act and to subsequently pass the STAR Act into law.

For More Information, Please Contact:

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DOD CDRMP FUNDING FOR AYA & PEDIATRICS

What Is the Problem/Opportunity:

The Department of Defense (DoD) funds medical research of diseases that affect military families through the Congressionally Directed Medical Research Program (CDMRP). Historically, this program has provided extensive cancer research funding for breast, prostate, ovarian, lung and other cancers with the average age of diagnosis being 66 years old. With respect to pediatric cancers, this program has funded a handful of pediatric brain tumor and neuroblastoma projects.

Given that 86% of the active military is under the age of 40 and over 90% of the active military have children, we believe the DoD medical research program should also fund research for cancers prevalent in adolescents and young adults (AYA) and children. Cancer is the #1 disease killer of this population. We are requesting the creation of \$30 million Peer-Reviewed Young Adult, Adolescent and Pediatric Cancer Research Program (also known as "DoD Funding for AYA and Pediatrics").

This is a unique opportunity because we are asking to be included in a pre-existing medical research budget that has been increasing over the last decade.

2016 Accomplishments

In 2016, Congressmen Michael McCaul and Scott Peters each submitted appropriation requests to the House Defense Appropriation Subcommittee for \$30 million in DOD Funding for AYA and Pediatrics. As a compromise, the FY17 House Defense Appropriation Bill includes the eligibility language (below) in the Peer-Reviewed Cancer Research Program instead of the creation of a separate cancer research program dedicated to AYA and pediatrics cancers.

"The funds provided in the peer-reviewed cancer research program are directed to be used to conduct research in the following areas: bladder cancer, brain cancer, colorectal cancer, listeria vaccine for cancer, liver cancer, lymphoma, melanoma and other skin cancers, mesothelioma, pancreatic cancer, stomach cancer, *and cancer in children, adolescents, and young adults.*" (page 272, House DoD Appropriation Bill, 2017)

A Senate office did not submit a FY17 appropriation request for the \$30 million program to the Senate Defense Appropriation Subcommittee. Therefore, neither a \$30 million program or eligibility language such as "cancer in children, adolescents, and young adults" was incorporated in the FY17 Senate Defense Appropriation Bill.

Next Steps:

Since a continuing resolution (CR) was passed, the FY16 budget will remain in effect until April 2017. At that time, the FY17 budget will be voted on and we hope Congress will pass a final Defense Appropriation Bill that includes our language. We will be monitoring the progress on the FY17 appropriations and continue to work with the new Congress to keep "cancers of children, adolescents and young adults" in the final FY17 Appropriation Bill.

At the same time, we will continue to work with our Congressional champions and ask them to submit a FY18 Defense appropriation request for the \$30 million AYA and Pediatric Cancer Research Program which will be considered by Congress in May 2017.

For More Information, Please Contact:

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<https://rallyfoundation.org/why-we-rally/advocacy/>

GABRIELLA MILLER KIDS FIRST ACT (H.R. 2019; Pub.L. 113–94)

What Is the Problem/Opportunity:

Pediatric cancer receives inadequate funding by the National Institutes of Health (NIH).

Gabriella Miller was a girl who died of cancer on October 26, 2013 at the age of 10. While she was ill, she worked as an activist to raise support for research into childhood illnesses like cancer. Through her activism, she raised hundreds of thousands of dollars and helped launch the Smashing Walnuts Foundation to fund pediatric cancer research. In *The Truth 365* documentary, Miller in response to a question what she'd like to tell American leaders about research on pediatric cancer, said that there needed to be "less talking, more doing... We need action." Then House Majority Leader Eric Cantor saw this video and decided to name the bill in Gabriella's honor.

In 2014, The Gabriella Miller Kids First Research Act was signed into law, authorizing a pediatric research initiative through the NIH of \$126 million over 10 years.

2016 Accomplishments

Congress appropriated the entire \$10 million in authorized funds for 2017 pursuant to the Gabriella Miller Kids First Research Act.

The NIH Common Fund Kids First Program is soliciting applications to provide samples for whole genome sequencing that will help researchers understand the genetic contribution to childhood cancers and structural birth defects. The genetic and clinical data from these cohorts will become part of the Kids First Data Resource.

Next Steps:

Advocates will ask Congress to authorize the funds that were appropriated under the Gabriella Miller Kids First Research Act for each of the 10 years of the program.

In addition, advocates are working with the NIH to ensure that the research funds from the Gabriella Miller Kids First Act are dedicated specifically to pediatric cancer.

For More Information, Please Contact:

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<https://commonfund.nih.gov/kidsfirst>

RESOLUTIONS AND REPORT LANGUAGE ON 2017 LEGISLATIVE AGENDA

SENATE REPORT LANGUAGE ON SLOW GROWING PEDIATRIC BRAIN TUMORS

What Is the Problem/Opportunity:

In the FY15 Labor, Health and Education Senate Appropriations Committee report, the NIH section provided language that was requested by the PLGA Foundation (aka A Kids' Brain Tumor Cure) regarding slow growing children's brain tumors. This language urges the NIH to translate its understanding of the pathways for PLGA into new therapies for kids battling PLGA tumors.

Based on recent discoveries in understanding the pathways that are active in PLGA tumors, the NCI launched a precedent-setting clinical trial which is directed at these genomic characteristics. To further these efforts, the Senate Committee language urges the NCI to utilize existing programs (i.e., the SPORE and CTEP programs) to encourage researcher grant submissions.

Precision Medicine is the next big policy opportunity to impact children with rare pediatric brain tumors. Because the research is still evolving around therapeutic targets, precision medicine holds great promise in providing more targeted treatment opportunities for children with brain cancer. NCI's Precision Medicine Initiative (PMI) and the pediatric MATCH trial are widely viewed as the next big scientific and policy opportunities for identifying and developing new therapies for these children.

2016 Accomplishments

In FY17's appropriations cycle, the PLGA Foundation worked with Congressman Rodney Davis to secure language in the House-passed Labor, Health, and Human Services Appropriations Subcommittee report that encourages NCI to continue to prioritize rare cancers in the MATCH trial, and requests that NCI provide an update on its plans to utilize the PMI and MATCH to identify and test more effective, less toxic treatments, and to improve the targeting of treatments for children battling brain cancer in the fiscal year 2018 budget request.

The FY17 appropriations bill accompanying the report language did not get passed and signed into law.

NIH is showing increased interest in this area as a result of all of this work done.

Next Steps:

In the coming year, the pediatric cancer community should seek follow-on language in the Congressional Appropriations Committee reports to NIH, based upon feedback received from scientists in the field on what the next opportunities are in this area, and should also seek other legislative opportunities to focus federal investment in research

on this terrible brain cancer. More needs to be done to stitch together the research findings, increase collaboration among researchers, pharmaceutical companies, and hospitals in different regions of the country on this disease, increase access to tissue samples, and to develop animal models for the disease. All of these efforts are focused on driving the oncological breakthroughs needed to find a cure.

For More Information, Please Contact:

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JD Derderian, Imagine an Answer.

HOUSE RESOLUTION FOR PEDIATRIC BONE CANCER

What Is the Problem/Opportunity:

In 2015, Representative Blake Farenthold (R-TX) introduced a House Resolution 102, expressing support for designation of September 25, 2015 as "National Pediatric Bone Cancer Awareness Day." The resolution was not passed by the House or Senate.

For More Information, Please Contact:

Congressman Blake Farenthold's office.

HOUSE RESOLUTION FOR DIPG

What Is the Problem/Opportunity:

DIPG has received, over the last 50 years, scant attention with research funding and mainstream media despite its invariably terminal prognosis and lack of solutions. Standard treatment protocol (radiation, palliative care) has not changed since Neil Armstrong's daughter died of DIPG in 1962.

In raising awareness for DIPG, the 2nd most common pediatric brain tumor, and representing some 20,000 years of human life lost each year, we can make a strong statement with regard to our government funds purposed for cancer research: let *mortality rate* and *years of life lost* be factors in the decision-making process with NCI research grants and other research investors. This is the most important language in the bill. Without stipulating policy or allocating funds, it simply starts the conversation for our children, and for those certain to die, to be greater priorities in our medical research system, using the poignant example of the suffering that DIPG children and their families endure. It's a non-political and practical strategy for the #morethan4 movement, and we hope to attract that population's support. Just as importantly, it honors the tens of thousands of children who have bravely faced their death in the last 50 years, and lets those currently struggling know that we are fighting for them.

2016 Accomplishments

House Resolution 586 was introduced. <http://hres586.com>
<http://jacksangelsfoundation.com/?p=3825>

It was not passed into law by the House or Senate

Next Steps:

Re-introduction of the House Resolution in 2017, working with the date of May 17 to be designated National DIPG Awareness Day.

Support of Jenny Mosier's 50-State project for each state to designate a state DIPG Awareness Day, "DIPG Across the Map."

For More Information, Please Contact:

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**BILLS PASSED IN 2016 THAT AFFECT CHILDREN WITH CANCER, BUT ARE NOT PEDIATRIC
CANCER-SPECIFIC**

21st CENTURY CURES: NIH FUNDING, CANCER MOONSHOT AND COMPASSIONATE USE ACCESS

In 2016, the U.S. Congress passed the 21st Century Cures Act, which included funding of the National Institutes of Health and the Cancer Moonshot. The Cancer Moonshot report included numerous research initiatives including pediatric projects in the areas of immunotherapy and fusion oncoproteins. 21st Century Cures Act included the compassionate use access language of the Andrea Sloane Act, also in the STAR Act.