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The Pediatric Cancer Yearbook is edited and compiled by Kids v Cancer (www.kidsvcan.org). For comments or suggested additions, please contact Elena@kidsvcan.org and NancyGoodman@kidsvcan.org.
OVERVIEW

LEGISLATIVE ACCOMPLISHMENTS SINCE THE ESTABLISHMENT OF THE U.S. HOUSE OF REPRESENTATIVES CHILDHOOD CANCER CAUCUS IN 2010

2012: Congress passes the Creating Hope Act Pediatric Priority Review Voucher Act

2012 to today: Congress includes eligibility categories in the Department of Defense Medical Research PRCRP for: 1) neuroblastoma, 2) pediatric and adult brain cancers, and 3) childhood cancers and adolescent and young adult cancers

2014: Congress passes the Gabriella Miller Kids First Research Act

2017: Congress passes the RACE for Children Act

2018: Congress passes the Childhood Cancer STAR Act and fully funds the STAR Act at $30 million per year for FY19

2018: Congress passes the Deferment for Active Cancer Treatment Act

2019: FDA publishes the Rare Pediatric Disease Priority Review Vouchers Draft Guidance for Industry

2019: The President’s 2020 budget proposal includes a new request for $500 million over 10 years for NCI for childhood cancer research.

LEGISLATIVE AGENDA FOR 2020

1. Passage of the Creating Hope Act Reauthorization Act
2. Request for $50 million to NIH for pediatric cancer as part of the proposed $500 million 10-year childhood cancer research initiative
3. Publication of FDA Draft Guidance for RACE for Children Act
4. Establishment and Funding of Stand-Alone Department of Defense CDMRP Pediatric Program
5. Funding of the Childhood Cancer STAR Act
6. Funding of the Gabriella Miller Kids First Research Act
7. Passage of Kids First 2.0
8. Passage of House Resolution for DIPG
9. Passage of Palliative Care and Hospice Education and Training Act
THE CREATING HOPE REAUTHORIZATION ACT
PEDIATRIC PRIORITY REVIEW VOUCHER (21 U.S.C. 360ff)

What is the problem/opportunity:

Historically there have been an inadequate number of drugs developed expressly for children with cancer.

In 2012, Congress passed the Creating Hope Act to establish the Rare Pediatric Priority Review Voucher Program. However, the Creating Hope Act will sunset in September 2020 if it is not reauthorized by Congress.

Accomplishments

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

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

Pediatric vouchers have been sold for as much as $350 million and are now trading at $100 million. Over $2 billion of vouchers have been traded. Since the enactment of the Creating Hope Act, the FDA has approved 19 drugs expressly for pediatric rare diseases, two of which are pediatric cancer drugs.

Congress originally passed the Creating Hope Act with a sunset provision, but has extended the program several times. In December 2016, as part of the 21st Century Cures Act, Congress reauthorized the Creating Hope Act until September 30, 2020. In addition, Congress provided that drugs that receive rare pediatric designations by September 30, 2020 will have until September 30, 2023 to earn a voucher.

Next steps:

Advocates will ask Congress to pass the Creating Hope Reauthorization Act of 2019.

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RACE FOR CHILDREN ACT OF 2017 (21 U.S.C. 355c)

What is the problem/opportunity:

There are thousands of drugs in the adult cancer pipeline but few of them have been studied for children with cancer. While the Pediatric Research Equity Act (PREA) requires companies to develop their adult drugs for children as well, PREA has not been applied to cancer because kids often have cancers that arise in different organs than adult cancers.

Accomplishments:

The RACE for Children Act was passed into law as Title V of the FDA Reauthorization Act to amend the Pediatric Research Equity Act (PREA) (21 U.S.C. 355c) in 2017.

The RACE for Children Act updates the Pediatric Research Equity Act by requiring companies developing targeted cancer drugs for adults to also develop those drugs for children. In addition, the RACE for Children Act ends an exemption from PREA requirements for cancer drugs that have orphan status.

Pursuant to requirements of the RACE for Children Act, in 2018, the FDA held two public meetings to provide input on development of a guidance. Additionally, FDA published lists of molecular targets to guide submissions for pediatric study plans.

Next steps:

In 2019, the FDA is required to publish a final Guidance on RACE for Children Act. The FDA will first publish a draft Guidance.

By August 20, 2020, the requirements of the RACE for Children Act will be enforced.

For more information, please contact:

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CHILDHOOD CANCER STAR ACT (PUBLIC LAW 115-180)

Accomplishments:

On June 5, 2018, the Childhood Cancer STAR Act (Public law 115-180) was signed into law. Congress fully funded the STAR Act at $30 million per year for FY19.

The Childhood Cancer STAR Act will support: NCI collection of biospecimens; tracking of childhood cancer incidence; survivorship research; and NCI focus on pediatric cancer.

The Childhood Cancer STAR Act authorizes the National Cancer Institute (NCI) to expand existing efforts to collect biospecimens for childhood cancer patients.

The STAR Act authorizes grants to state cancer registries to track incidence of pediatric and young adult cancers.

The STAR Act establishes a pilot program for innovative models of care for survivors.

The STAR Act directs NIH childhood health reporting requirements to include pediatric cancer.

Next steps:

Advocates will ask for full appropriation of the STAR Act every year for the next four years.

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CHILDHOOD CANCER DATA INITIATIVE

What is the problem/opportunity:

There are a lack of efficient ways to collect, share, and integrate data from individual hospitals where children with cancer are treated. This limits the potential for researchers to collaborate.

The aim of the Childhood Cancer Data Initiative (CCDI) is to establish more efficient ways to share and use childhood cancer data to help identify novel therapeutic targets and approaches, better understand the biology of childhood cancers, and advance new drug development.

Accomplishments:


Next steps:

The administration’s budget request includes a request of $500 million over the next 10 years for childhood cancer research. Congress’ budget for FY2019 included language supporting a pediatric cancer program at the NIH.

For more information, please contact:

The National Cancer Institute,
DEPARTMENT OF DEFENSE MEDICAL RESEARCH PROGRAM

What is the problem/opportunity:

The Department of Defense (DoD) has an approximately $1.5 billion medical research program through the Congressionally Directed Medical Research Program (CDMRP). Additionally, under the CDMRP, there is a Peer Reviewed Cancer Research Program (PRCRP) which identifies cancers eligible for research funding. The advocates’ goal is to include funding for young adult and pediatric cancer research.

Accomplishments:

Over the past few years, the pediatric cancer community created and secured the continued inclusion of three pediatric cancer eligibility categories in the Department of Defense Medical Research Programs through the Peer-Reviewed Cancer Research Program (PRCRP).

The three pediatric cancer eligibility categories are: 1) neuroblastoma, 2) pediatric brain tumors, 3) cancers in children, adolescents and young adult cancer. As a result, researchers applying through these three eligibility categories were awarded $10.5 million in 2018 and $10.3 million in 2017.

Next steps:

The community will continue to advocate for the inclusion of neuroblastoma and pediatric brain tumors within the PRCRP and continued funding of the PRCRP. In addition, advocates will ask for the establishment of a separate Department of Defense Medical Research Program stand-alone program for cancers in children, adolescents and young adults, funded at $30 million for fiscal year 2020.

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GABRIELLA MILLER KIDS FIRST RESEARCH ACT OF 2014 (PL 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. Then House Majority Leader Eric Cantor named the bill in her honor.

Accomplishments:

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

Congress appropriated $12.6 million per year for each of the first five years of the Gabriella Miller Kids First Research Act.

Next steps:

Advocates will again ask Congress to fully fund The Gabriella Miller Kids First Research Act.

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
KIDS FIRST RESEARCH ACT 2.0

What is the problem/opportunity:

The Kids First Research Act 2.0 is a continuation of the Gabriella Miller Kids First Research Act. It would redirect approximately $320 million in existing, reserved and unused government funds from the Presidential Election Campaign Fund to the Gabriella Miller Kids First Pediatric research program at the National Institutes for Health.

The funds would be used to develop a comprehensive shared data resource for scientists researching pediatric cancers and structural birth defects and would support the development of computational tools to analyze these large, complex genomic and clinical datasets.

Accomplishments:

The Kids First Research Act 2.0 was introduced in the House in April 2017 by Rep. Jeff Denham [R-CA] and Rep. Fred Upton [R-MI] as H.R. 2008. The bill expired in December 2018 with the end of the 115th Congress. Currently funding sources for Kids Frist Act 2.0 are being secured.

Next steps:

Advocates will ask Congress to reintroduce the Kids First Research Act 2.0.

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DEFERMENT FOR ACTIVE CANCER TREATMENT ACT (H.R 2976)

What is the problem/opportunity:

In the last 10 years, nearly 70% of all college graduates have funded their education through student loans. The national delinquency rate on these loans is 11.2%.

Many adolescents and young adults diagnosed with cancer face the risk of delinquency on their student loans when treatment interferes with their ability to work or attend school.

Accomplishments:

The Deferment for Active Cancer Treatment Act was passed into law in September 2018 (20 U.S.C. 1078, 1087e&dd).

The Deferment for Active Cancer Treatment Act amends the Higher Education Act of 1965 to enable students who are diagnosed with cancer and who are actively receiving treatment to defer payments on public student loans without interest accruing and compounding during the deferment period.

The Department of Education has amended the Federal Student Aid rules for student loan deferments due to treatment for cancer and made available a new cancer treatment deferment form. Borrowers who are undergoing cancer treatment and wish to request a deferment of their federal student loan payments should complete the form and submit it to their loan servicer.

For more information, please contact:

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HOUSE RESOLUTION FOR DIPG (H.Res. 114)

What is the problem/opportunity:

Diffuse intrinsic pontine glioma (DIPG), a children's brain cancer, has a terminal prognosis. There has been no significant change in standard treatments or prognosis in over 40 years.

The DIPG Awareness Resolution (H. Res. 114) asks the NIH to elevate the consideration of mortality rate and years of life lost in the grant decision-making process. The Resolution would also establish a national “DIPG Awareness Day” on May 17 to raise awareness of the disease.

In addition, the Michael Mosier Defeat DIPG Foundation's "Across the Map" project is petitioning for every state to issue a proclamation also designating May 17 as DIPG Awareness Day.

Accomplishments:

In 2017, the House introduced The DIPG Awareness Resolution. It expired in December 2018. It was re-introduced in the House in February 2019 by Rep. Jackie Speier and referred to House Energy and Commerce Committee. It currently has 50 cosponsors.

By 2019, 32 States issued either a gubernatorial proclamation or a legislative resolution for DIPG Awareness Day May 17, with four state measures in perpetuity.

Next steps:

Advocates will ask Congress to pass House Resolution for DIPG.

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PALLIATIVE CARE AND HOSPICE EDUCATION AND TRAINING ACT
(PCHETA, H.R 647, S.2080)

What is the problem/opportunity:

The goal of pediatric palliative care is to optimize quality of life and well-being of children with life-threatening illnesses by anticipating, preventing, and treating suffering in all its forms, from the time of diagnosis and throughout treatment.

The Palliative Care and Hospice Education and Training Act (PCHETA) establishes palliative care and hospice education centers to improve the training of interdisciplinary health professionals in palliative care; develop and disseminate curricula relating to palliative care; support continuing education; provide students with clinical training in appropriate sites of care. It directs the NIH to use existing authorities and funds to expand palliative care research to advance clinical practice and improve care delivery for patients with serious or life-threatening illnesses.

Accomplishments:

In January 2019 PCHETA was introduced in the House of Representatives; it now has 281 co-sponsors. In July 2019 PCHETA was introduced in the Senate, where it has 29 co-sponsors.

Next steps:

Advocates will be asking Congress to pass PCHETA.

For more information, please contact:

http://patientqualityoflife.org