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

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

1. Congress passed the Creating Hope Act Pediatric Priority Review Voucher Act
2. Congress passed the RACE for Children Act
3. Congress passed the Childhood Cancer STAR Act
4. Congress included 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.
5. Congress passed the Gabriella Miller Kids First Act
6. Congress passed the Deferment for Active Cancer Treatment Act

LEGISLATIVE AGENDA FOR 2019

1. Passage of the Creating Hope Act Reauthorization of 2019
2. Publication of FDA Guidance for RACE for Children Act
3. Establishment and Funding of Stand-Alone Department of Defense CDMRP Pediatric Program
4. Funding of the Childhood Cancer STAR Act
5. Funding of the Gabriella Miller Kids First Research Act
6. Passage of Kids First 2.0
7. Passage of House Resolution for DIPG
CREATING HOPE 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 Rare Pediatric Disease Priority Review Voucher Program, creating a powerful incentive for rare pediatric diseases drug development.

However, the Creating Hope Act will sunset in September 2020 if it is not reauthorized by Congress.

Accomplishments:

In 2012, Congress passed the Pediatric PRV 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 from the FDA 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 $1 billion of vouchers have been traded.

Congress originally passed the Creating Hope Act as a pilot program 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 2020. In addition, Congress provided that drugs that receive rare pediatric designations by September 20, 2020 will have until September 20, 2023 to earn a voucher.

Next steps:

Advocates will ask Congress to permanently reauthorize the Creating Hope Act.

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

What is the problem/opportunity:

There are 900 drugs in the adult cancer pipeline but few of them have been studied for children with cancer. While the Pediatric Research Equity Act 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 (Research to Accelerate Cures and Equity) 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 with cancer. In addition, the RACE for Children Act ends an exemption from PREA requirements for cancer drugs that have orphan status.

In 2018, the FDA held two public meetings to solicit feedback from physicians and researchers, patients, and other stakeholders to provide input on development of a guidance.

Next steps:

By August 20, 2019, the FDA is required to publish a guidance on RACE for Children Act.

By August 20, 2020 or a year after the FDA publishes the guidance, whichever is earlier, the requirements of the RACE for Children Act will be enforced.

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CHILDHOOD CANCER STAR ACT (PUBLIC LAW 115-180)

What is the problem/opportunity:

The Childhood Cancer STAR (Survivorship, Treatment, Access, and Research) Act will expand opportunities for childhood cancer research and enhance the quality of life for childhood cancer survivors.

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:

Expanding Opportunities for Childhood Cancer Research: The Childhood Cancer STAR Act will authorize the National Cancer Institute (NCI) to expand existing efforts to collect biospecimens for childhood cancer patients.

Improving Childhood Cancer Surveillance: The STAR Act bill will authorize grants to state cancer registries to track incidence of pediatric and young adult cancers.

Improving Quality of Life for Childhood Cancer Survivors: The STAR Act will support research on the late effects of childhood cancers and on insurance coverage for childhood cancer survivors. The STAR Act will establish a pilot program for innovative models of care for survivors.

Ensuring Pediatric Expertise at the National Institutes of Health (NIH): The Childhood Cancer STAR Act will improve NCI 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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DEPARTMENT OF DEFENSE MEDICAL RESEARCH PROGRAM

What is the problem/opportunity:

The Department of Defense (DoD) has an approximately $1 billion medical research program through the Congressionally Directed Medical Research Program (CDMRP). Historically, this program had funded little or no pediatric cancer research.

Accomplishments:

In the past several 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 and adult brain cancer, 3) cancers in children, adolescents and young adults result in FY2018, researchers applying through these three eligibility categories were awarded $10.3 million in 2017.

Next steps:

Advocates will ask for the establishment of a separate Department of Defense Medical Research Program stand-alone program for pediatric cancer and adolescent and young adult cancer, funded at $30 million for fiscal year 2020.

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GABRIELLA MILLER KIDS FIRST 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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DEFERMENT FOR ACTIVE CANCER TREATMENT ACT (HR 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.

Accomplishment:

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.

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CHILDREN’S PROGRAM OF ALL-INCLUSIVE COORDINATED CARE (CHIPACC)

What is the problem/opportunity:

Medicaid, the largest purveyor of children’s health insurance, has a hospice benefit for children, but this benefit does not cover palliative care. To qualify for the hospice benefit, children must receive a certification of having less than six months to live. However, children may move in and out of the terminal stage of a disease.

The Children’s Program of All-Inclusive Coordinated Care (ChiPACC) Act would: 1) Improve care for children with life-limiting illnesses by providing access to concurrent hospice and palliative care and 2) NOT require participating children to forgo coverage for curative treatments, NOT require participating children to receive a diagnosis of having less than six months to live, and NOT require participating children to relinquish coverage for other health care services such as counseling and therapy.

Accomplishments:

Introduced by Reps. DeGette (D-CO), McCaul (R-TX), Butterfield (D-NC), Kelly (R-PA), and Speier (D-CA) in 2018 as H.R. 6560. This bill expired in December 2018 with the end of the 115th Congress.

Next steps:

Advocates will ask Congress to reintroduce CHIPACC for the 116th Congress.

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KIDS FIRST RESEARCH ACT 2.0

What is the problem/opportunity:

The Kids First Research Act 2.0 (HR 2008) 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:


Next steps:

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

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HOUSE RESOLUTION FOR DIPG

What is the problem/opportunity:

Diffuse intrinsic pontine glioma (DIPG), a children’s brain cancer, has a terminal prognosis.

The DIPG Awareness Resolution (H. Res. 69) asks the National Institutes of Health 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 (H.Res. 69). It expired in December 2018.

In 2018, 31 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 the House to reintroduce the House Resolution for DIPG.

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