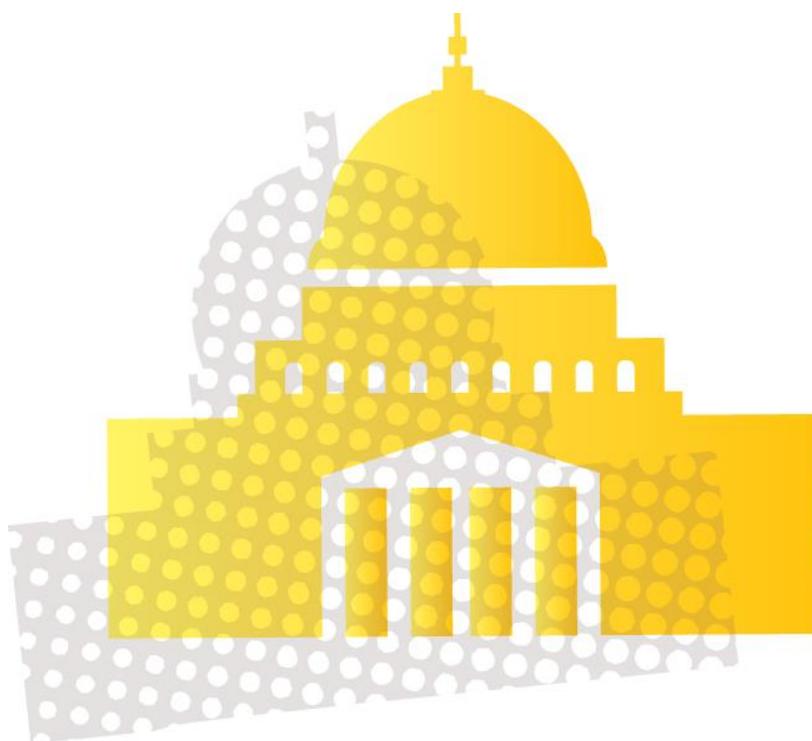


PEDIATRIC CANCER

LEGISLATIVE YEARBOOK

SEPTEMBER 2018



EDITED BY KIDS V CANCER

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OVERVIEW

LEGISLATIVE ACCOMPLISHMENTS SINCE 2012

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

LEGISLATIVE ACCOMPLISHMENTS IN 2018

1. Congress passed and fully funded the Childhood Cancer STAR Act to \$30M
2. Congress passed the Deferment for Active Cancer Treatment Act
3. Congress funded the Department of Defense Medical Research PRCRP, a portion of which goes to pediatric cancer research
4. Congress fully funded the Gabriella Miller Kids First Act, a portion of which goes to pediatric cancer research
5. The FDA held public hearings on the RACE for Children Act

LEGISLATIVE AGENDA FOR 2019

Reauthorization of Current Programs

- Permanent reauthorization of the Creating Hope Act Pediatric Priority Review Voucher Program

Funding for Current Programs

- Funding of the Childhood Cancer STAR Act
- Funding of the Department of Defense PRCRP
- Funding of the Gabriella Miller Kids First Research Act

Implementation of Current Programs

- FDA publication of Guidance for RACE for Children Act

Passage of New Programs

- Passage of Children's Program of All-Inclusive Care Act
- Passage of the Kids First Research Act 2.0
- Passage of House Resolution for DIPG

PEDIATRIC CANCER LEGISLATIVE ACCOMPLISHMENTS

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

What is the problem/opportunity:

In 2012, Congress passed the Creating Hope Act Rare Pediatric Priority Review Voucher Program (Pediatric PRV)(21 U.S.C. 355c), creating a powerful incentive for rare pediatric drug development.

However, the Pediatric PRV 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 Pediatric PRV, 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 a FDA “priority review” instead of a “standard review.” And, 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 Pediatric PRV 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 Pediatric PRV 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 before 2020.

For more information, please contact:

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

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).

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:

In 2017, The RACE for Children Act was passed into law.

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

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.

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.

Next steps:

Advocates will continue to work on implementation and appropriation of the STAR Act.

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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 successfully 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 adult cancer. Pediatric cancer advocates secured the continued inclusion of all three eligibility categories this past year.

The pediatric cancer community also successfully asked Congress for continued support of the PRCRP program, bringing the PRCRP funding level to \$90 million for FY19.

In addition, the pediatric cancer community focused its efforts on ensuring that pediatric researchers apply to the PRCRP through the three pediatric cancer eligibility programs. As a result, in total, researchers applying through these three eligibility categories which include, but are not limited to pediatrics, were awarded \$10.3 million in 2017.

Next steps:

Pediatric cancer advocates ask that the three pediatric eligibility programs be continued in the PRCRP for FY20.

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GABRIELLA MILLER KIDS FIRST ACT OF 2014 (Public Law 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 was an activist and raised support for research. In *The Truth 365* documentary, Gabriella was asked what she'd like to tell Congress about research on pediatric cancer. She answered that there needed to be "less talking, more doing... We need action." When House Majority Leader Eric Cantor saw this video, he decided to name the bill in her honor.

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.

Accomplishments:

Congress appropriated \$12.6 million per year for each of the first four years of the Gabriella Miller Kids First Research Act (Pub.L.113-94).

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.

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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%. Delinquency is particularly a challenge for students in active cancer treatment. Each year, more than 70,000 Americans ages 15 to 39 are diagnosed with cancer. 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.

The Deferment for Active Cancer Treatment Act amends the Higher Education Act of 1965 and would 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. This bill does not seek to change the terms of the public loan agreement between lender and borrower beyond placing a pause in repayments while cancer treatment is underway.

Accomplishments:

The Deferment for Active Cancer Treatment Act was passed into law in September 2018.

For more information, please contact:

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PEDIATRIC CANCER BILLS FOR 2019

CHILDREN'S PROGRAM OF ALL-INCLUSIVE COORDINATED CARE (CHIPACC) (H.R. 6560)

What is the problem/opportunity:

Medicaid, the largest purveyor of children's health insurance, has an optional hospice benefit for children, but this benefit does not cover palliative care. To qualify for the optional 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, creates an optional Medicaid benefit for hospice and palliative care programs aimed at helping children with life-threatening illnesses, such as cancer.

ChiPACCs would: 1) Improve care for children with life-limiting illnesses by providing access to concurrent hospice and palliative care, delivered by an interdisciplinary care team 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.

States such as Colorado and North Dakota have used Section 1915(c) to implement state versions of ChiPACC. When compared to intensive hospital and nursing care, **these states have produced cost savings of up to 87 percent.** Even though ChiPACCs are cost-effective, the Section 1915(c) waiver process can deter states from implementing these programs since the waiver process is burdensome.

Accomplishments:

Introduced by Reps. DeGette (D-CO), McCaul (R-TX), Butterfield (D-NC), Kelly (R-PA), and Speier (D-CA) in 2018.

Next steps:

Advocates will ask Congress to sign a Dear Colleagues Letter.

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KIDS FIRST RESEARCH ACT 2.0 (H.R. 2008)

What is the problem/opportunity:

The Gabriella Miller Kids First Research Act has provided valued funds to the NIH for pediatric research. Additional funding would create additional valued and critical research.

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 NIH. Under the direction of the NIH, the funds would be used to develop a comprehensive shared data resource for scientists researching hundreds of different 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. Denham, Jeff [R-CA] and Rep. Fred Upton [R-MI].

Next steps:

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

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HOUSE RESOLUTION FOR DIPG (H.Res. 69)

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) addresses the inadequacy of research funding for DIPG and asks that pediatric and high-risk cancers have a more elevated consideration for research grant funding both with government and private sectors. Specifically, it 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 of Representatives introduced The DIPG Awareness Resolution (H.Res. 69). 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.

As of 2017, there were 21 U.S. states that have proclaimed May 17 to be Awareness Day for DIPG.

Next steps:

Advocates will ask the House to pass the House Resolution for DIPG.

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