PEDIATRIC CANCER LEGISLATIVE YEARBOOK

JANUARY 2023

Compiled by

KIDS V CANCER

TABLE OF CONTENTS

January 2023 marks the beginning of a new Congress, the 118th. As such, all bills Congress did not pass last year, including all the bills reviewed in this yearbook, must be re-introduced and assigned new bill numbers. As bills are re-introduced, we will update this 2023 yearbook.

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.

LEGISLATIVE ACCOMPLISHMENTS

2022: Congress passes the Childhood Cancer STAR Act 2.0
Lead Advocate: St Baldricks

2022: The House of Representatives passes Gabriella Miller Kids First 2.0 Act.
Lead Advocate: Smashing Walnuts, Four Square Clobbers Cancer

2022: Senate passes S.Res.642, a resolution expressing support for the designation of May 17, 2022, as DIPG Pediatric Brain Cancer Awareness Day.
Lead Advocate: *Jack's Angels Foundation*

2021-2022: The Give Kids a Chance Act, authorizing FDA to direct pediatric study plans of combinations of novel cancer drugs, passes in the House of Representatives.
Lead advocate: Kids v Cancer, Truth 365, Tough2gether

2020: Congress reauthorizes the Creating Hope Act multiple times, most recently in 2020, extending the priority review voucher program until September 2024.
Lead advocate: Kids v Cancer

2020: Congress includes the Global Hope Act in report language to authorize the Department of State to work with the private sector, the United Nations, and other organizations to address childhood cancer globally.
Lead advocate: American Childhood Cancer Organization

2020: Congress passes the Clinical Treatment Act requiring Medicaid to cover routine clinical trial hospital costs. One-third of kids with cancer are covered by Medicaid.

2019: President Trump requests an additional $500 million over 10 years for childhood cancer research in the State of the Union Address, resulting in annual report language in the NIH budget.

2018: Congress passes the Deferment for Active Cancer Treatment Act, enabling students in treatment to defer student loans without accrual of interest.

2018: Congress passes the Childhood Cancer STAR Act, for research, tracking of incidences and quality of life for childhood cancer survivors. In years 2018-2022, Congress provided annual report language to support the program. It is unclear whether the report language results in increases to funding equal to the report language.
Lead advocate: Alliance for Childhood Cancer, St Baldricks, CAC2

2017: Congress passes the RACE for Children Act, requiring all cancer drugs to be studied in children with cancer.
Lead advocate: Kids v Cancer

2014: Congress passes the Gabriella Miller Kids First Research Act, resulting in $126 million allocation over 10 years for the NIH Gabriella Miller Kids First Research Program. In years 2015 to 2023, Congress provided annual report language to support the program. While report language, the Gabriella Miller Kids First Research Act results in $126 million of new funds for pediatric cancer because the directed funds are to be held in a distinct NIH Common Fund.
Lead advocate: Smashing Walnuts

2012- **2022:** Congress includes in the Department of Defense (DoD) Congressionally Directed Medical Research Program (CDMRP) PRCRP eligibility categories for neuroblastoma, pediatric brain cancers, childhood cancers and adolescent and young adult cancers.
Lead advocates: Rally Foundation, National Brain Tumor Foundation, EVAN Foundation, The Nicholas Connor Institute

2012: Congress passes the Creating Hope Act Pediatric Priority Review Voucher program, resulting in an incentive for pediatric rare disease drug development.
Lead advocate: Kids v Cancer

PRIORITY LEGISLATIVE AGENDA OF KIDS V CANCER FOR 2023[1]

PASSAGE OF NEW AUTHORIZATIONS

1. Give Kids a Chance Act

2. Gabriella Miller Kids First Research Act 2.0

APPROPRIATIONS AND REPORT LANGUAGE

4. To the Department of Defense CDMRP Pediatric Program.

5. $12.6 million to the NIH Gabriella Miller Kids First Research Program pursuant to the Gabriella Miller Kids First Research Act.

6. $30 million for the Childhood Cancer STAR Act.

[1] See Appendix B for an explanation of authorizations, appropriations and report language

LEGISLATIVE AGENDA FOR 2023: AUTHORIZATIONS

GIVE KIDS A CHANCE ACT

What is the problem/opportunity:

Over the past decade, cancer scientists have developed new classes of drugs with exciting potential -- immunotherapies, precision medicines, epigenetic therapies and tumor microenvironment modulators. Now, cancer clinical trials for adults are exploring combinations of these novel therapies. These adult cancer trials are resulting in cures for otherwise terminal cancer patients.

However, children with cancer do not have access to these potentially curative clinical trials of combinations of drugs.

Pursuant to RACE for Children Act, passed by Congress in 2017, companies are now undertaking pediatric study plans for novel cancer therapies. But RACE for Children Act only requires the pediatric study plans to be of one novel drug at time – and these single drug trials are not often curative.

The Give Kids a Chance Act amends the RACE Act and authorizes the FDA, at its discretion, to require companies' pediatric study plans to be of *combinations* of novel therapies instead of single drug trials.

Accomplishments:

The Give Kids a Chance Act was included as section 714 of House PDUFA bill, HR 7667. On June 8, 2022, the Act was overwhelmingly and on a bipartisan basis passed by the House as part of HR 7667, with a vote of 392 to 28.

Next steps:

In this new Congress, advocates ask Congress to re-introduce and pass the Give Kids a Chance Act.

For more information, please contact:

Lauren Reamy (Sen. Marc Rubio), lauren_reamy@rubio.senate.gov
Santiago Gonzalez (Sen. Michael Bennet), Santiago_gonzalez@bennet.senate.gov
Zach Isakowitz (Rep. Michael McCaul), Zach.Isakowitz@mail.house.gov
Nancy Goodman, nancygoodman@kidsvcancer.org

GABRIELLA MILLER KIDS FIRST RESEARCH ACT 2.0

What is the problem/opportunity:

This bill provides for new funding to a pediatric disease research initiative within the National Institutes of Health (NIH). The Gabriella Miller Kids First Research Act 2.0 would redirect penalties for violations of Foreign Corrupt Practice Act levied against pharmaceutical manufacturers from the Treasury and towards the Kids First Pediatric Research Program at the NIH. Currently, this initiative receives funding through FY2023 from the 10-Year Pediatric Research Initiative Fund.

Accomplishments:

On July 27, 2022 this bill passed in the House of Representatives, reauthorizing $125 million to extend Kids First Research Program and Data Resource Center for an additional five years.

Next steps:

In this new Congress, advocates ask Congress to re-introduce and pass the Kids First Research Act 2.0.

For more information, please contact:

Ellyn Miller, Smashing Walnuts Foundation, ellyn@smashingwalnuts.org

CREDIT FOR CARING ACT

What is the problem/opportunity:

Families of children with cancer often are forced to take time off or quit work to care for the child.

The Credit for Caring Act of 2021 allows an eligible caregiver of an ill child or adult to receive a tax credit of up to $5,000 for 30% of the cost of long-term care expenses that exceed $2,000 in a taxable year. The bill defines eligible caregiver as an individual who has earned income for the taxable year in excess of $7,500 and pays or incurs expenses for providing care to a dependent relative with long-term care needs. The nonrefundable tax credit could be used toward expenses such as transportation, home modifications to accommodate a family member, medication management services, and training or education for the caregiver.

Accomplishments:

The Credit for Caring Act was introduced in May 2021 by Sen. Joni Ernst (R-IA) and Sen. Michael Bennet (D-CO), and currently has 22 cosponsors. It was simultaneously introduced in the House by Rep. Linda Sánchez, (D-CA-38) and Rep. Tom Reed (R-NY-23) and has 74 cosponsors in the House.

Next steps:

Advocates will ask Congress to pass the Credit for Caring Act.

For more information, please contact:

Rhonda Richards, AARP, rrichards@aarp.org, or
www.creditforcaring.com

ACCELERATING KIDS' ACCESS TO CARE ACT

What is the problem/opportunity:

When a child enrolled in Medicaid has medical needs that cannot be met by providers in his or her home state, the State Medicaid Agency and/or Medicaid Managed Care Organization in concert with the child's healthcare provider will authorize care to be provided by an out-of-state provider.

Under current law, the in-state Medicaid program of the child must screen and enroll the out-of-state providers. However, there exists no process to facilitate a streamlined screening. This creates delayed access to care and potentially harms the child's health.

The Accelerating Kids' Access to Care Act amends title XIX of the Social Security Act to require state Medicaid programs to establish a process through which qualifying out-of-state providers may enroll as participating providers without undergoing additional screening requirements.

Accomplishments:

The bill was re-introduced in the House in May 2021 by Rep. Katherine Clark (D-MA-05) and Rep. Jaime Herrera Beutler (R-WA-3), where it had 103 cosponsors, and by Sen. Chuck Grassley (R-IA) and Sen. Michael Bennet (D-CO) in the Senate, with 24 cosponsors at the end of 117th Congress.

Next steps:

Advocates are asking Congress to re-introduce and pass the Accelerating Kids' Access to Care Act.

For more information, please contact:

Megan Herber, megan.herber@faegredrinker.com.

JONNY WADE PEDIATRIC CANCER RESEARCH ACT

What is the problem/opportunity:

Presidential candidates may receive federal funds through the Presidential Election Campaign Fund to pay for the qualified expenses of their political campaigns in primary and general elections. This fund has not been utilized since 2008 and has accumulated over $370 million.

The Jonny Wade Pediatric Cancer Research Act terminates: (1) the taxpayer election to designate $3 of income tax liability for financing of presidential election campaigns, (2) the Presidential Election Campaign Fund, and (3) the Presidential Primary Matching Payment Account, and transfers the funds remaining in the Presidential Election Campaign Fund to the 10-Year Pediatric Research Initiative Fund to help identify treatments for pediatric diseases.

Accomplishments:

The bill was re-introduced in the House of Representatives in May 2021 by Rep. Rodney Davis (R-IL-13) and Rep. Josh Gottheimer (D-NJ-5).

Next steps:

Advocates ask for Congress to re-introduce and pass the bill.

For more information, please contact:

Kimberly Wade, Kids Shouldn't Have Cancer Foundation, kwade@kshcf.org

FAIRNESS TO KIDS WITH CANCER ACT

What is the problem/opportunity:

Less than 4% of the total National Cancer Institute (NCI) research budget is presently directed specifically toward childhood cancer research.

The Fairness to Kids with Cancer Act requires the share of federal funds for cancer research that is allocated to pediatric cancer research to equal the percentage of the U.S. population that is under the age of 18, which is currently 22%.

Accomplishments:

The Fairness to Kids with Cancer Act was introduced in the House of Representatives in 2019 by Rep. Brian Fitzpatrick (R-PA-01) and Rep. Josh Gottheimer (D-NJ-05), and reintroduced in the 117th Congress in March 2021. It had 9 cosponsors in the House.

Next steps:

Advocates will ask for the bill to be introduced and will ask Congress to pass the Fairness to Kids with Cancer Act.

For more information, please contact:

Jacqueline Collie (Rep. Brian Fitzpatrick), Jacqueline.collie@mail.house.gov
Mina Carroll, Storm the Heavens, info@stormtheheavens.org

PEDIATRICIANS ACCELERATE CHILDHOOD THERAPIES ACT

What is the problem/opportunity:

The PACT Act will amend the Public Health Service Act (42 U.S.C. 288) to authorize the Director of the NIH to make awards to early-career physician-scientists focusing on pediatric research, including basic, clinical, translational, or pediatric pharmacological research. The bill names pediatric cancer and precision medicine as priority research areas. It also establishes the Trans-NIH Pediatric Research Consortium to coordinate pediatric research across national health research institutions.

Accomplishments:

The bill was introduced in the House in April 2021 by Rep. John Joyce (R-PA-13), where it had 12 cosponsors in the last Congress, and by Sen. Joni Ernst (R-IA), with 5 cosponsors in the Senate.

Next steps:

Advocates will ask members of Congress to reintroduce and pass the PACT Act in the new Congress.

For more information, please contact:

Matthew Tucker (Rep. John Joyce), matthew.tucker@mail.house.gov.

CANCER PATIENT EQUITY ACT

What is the problem/opportunity:

In recent years, molecular testing has emerged as a useful tool to aid in the identification of targeted therapies that treat a specific cancer. Genomic testing is widely used in diagnosis and treatment of pediatric cancers and includes microarray analysis, DNA and RNA sequencing, whole-exome sequencing, and other next-generation sequencing.

This bill will facilitate the development of treatments for cancers by providing for coverage of laboratory and diagnostic tests for children and adults diagnosed with cancer under the Children's Health Insurance Program (CHIP), Medicaid and Medicare. The bill also calls for creation of an education and awareness program for physicians and the general public on what genomic testing is and how it can be used.

Accomplishments:

The bill was introduced in the House in September 2021 by Rep. G. K. Butterfield (D-NC-1) and gathered 19 cosponsors in the last Congress.

Next steps:

Advocates will ask members of Congress to reintroduce and pass the Cancer Patient Equity Act in the new Congress.

For more information, please contact:

Jonathan Vecchi (Rep. Gus Bilirakis), Jonathan.Vecchi@mail.house.gov
Chris Jones (Rep. Gus Bilirakis), Chris.Jones@mail.house.gov.

KNOCK OUT CANCER ACT

What is the problem/opportunity:

The KO Cancer Act aims to increase funding for cancer research by the National Institutes of Health to be more in proportion to the mortality rates of cancer. Despite the overall decrease in mortality, approximately 1,800 children and adolescents die of cancer each year in the U.S., indicating that new advances and continued research to identify effective treatments are required to further reduce childhood cancer mortality.

This bill provides additional funding to the NIH for cancer research through FY2027. The amount of additional funding each fiscal year is equal to 25% of the total amount allocated to the NIH for cancer research in FY2021.

Accomplishments:

The bill was introduced in the House in December 2021 by Rep. Brian Fitzpatrick (R-PA-1). It had 2 cosponsors in the House.

Next steps:

Advocates will ask to reintroduce and pass the KO Cancer Act in the new Congress.

For more information, please contact:

Matthew Clarkin (Rep. Brian Fitzpatrick), Matthew.Clarkin@mail.house.gov
Jacqueline Collie (Rep. Brian Fitzpatrick), Jacqueline.Collie@mail.house.gov

LEGISLATIVE AGENDA FOR 2023: APPROPRIATIONS

GABRIELLA MILLER KIDS FIRST RESEARCH ACT

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:

Ellyn Miller, Smashing Walnuts Foundation, ellyn@smashingwalnuts.org
https://commonfund.nih.gov/kidsfirst

LEGISLATIVE AGENDA FOR 2023: REPORT LANGUAGE

DEPARTMENT OF DEFENSE MEDICAL RESEARCH PROGRAM

What is the problem/opportunity:

The Department of Defense (DoD) runs a $1.5 billion medical research program through the Congressionally Directed Medical Research Program (CDMRP). Within the CDMRP, the Peer Reviewed Cancer Research Program (PRCRP) provides funding for eligible cancers.

Accomplishments:

Over the past several years, the pediatric cancer community secured the continued inclusion of pediatric, adolescent and young adult (AYA) cancer eligibility categories in the PRCRP program.

For FY 2023 the Peer Reviewed Cancer Research Program (PRCRP) eligible categories received $130 million.

To view awards visit: https://cdmrp.army.mil/prcrp/awards/awards and https://cdmrp.health.mil/funding/archive/prcrparchive

Next steps:

The community asks Congress to continue to include current eligible pediatric, adolescent and young adult cancers in the PRCRP and fund the PRCRP.

For more information, please contact:

Zach Isakowitz (Rep. Michael McCaul, R-TX), Zach.Isakowitz@mail.house.gov
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Dean Crowe, Rally Foundation for Childhood Cancer Research, Dean@rallyfoundation.org
Danielle Leach, National Brain Tumor Society, Dleach@braintumor.org
Gavin Lindberg, Gavin@theevanfoundation.org.

CHILDHOOD CANCER DATA INITIATIVE

What is the problem/opportunity:

There was no central repository of data from hospitals where children with cancer are treated.

In 2019 in his State of the Union address, President Trump asked for an additional $500 million over the next 10 years for childhood cancer research as part of the budget of the U.S. Government.

Accomplishments:

The establishment of the Childhood Cancer Data Initiative (CCDI) to enhance data sharing, collection, analysis, and access for ongoing and planned childhood and AYA cancer and survivorship research throughout the Institute, to be funded at $50 million per year.

Next steps:

Advocates will ask Congress' budget for FY2024 to include language funding CCDI at $50 million.

For more information, please contact:

The National Cancer Institute,
https://www.cancer.gov/research/areas/childhood/childhood-cancer-data-initiative

LEGISLATIVE AGENDA FOR 2023: RESOLUTIONS

HOUSE RESOLUTION FOR DIPG

What is the problem/opportunity:

The DIPG Awareness Resolution (H. Res. 404) 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.

Accomplishments:

The resolution was first introduced to the House of Representatives in January 2016, finishing the 114th Congress without a vote; it was reintroduced in January of 2017 as H. Res. 69 and finished the 115th Congress with 53 cosponsors, and then in the 116th Congress, H. Res. 114 gained the support of nearly half the House with 215 signatures recorded at the close of the session. It was introduced for a fourth time in May 2021, by Rep. Debbie Dingell (D-MI-12) and Rep. David Joyce (R-OH-14), and had 220 cosponsors in the House.

In May 2022, Senate passed S.Res.642 - A resolution expressing support for the designation of May 17, 2022, as "DIPG Pediatric Brain Cancer Awareness Day" to raise awareness of, and encourage research on, diffuse intrinsic pontine glioma tumors and pediatric cancers in general. The Senate recognizes a day for one year only, whereas the House Resolution would designate the day in perpetuity.

Next steps:

Advocates will ask the new House of Representatives to pass the resolution and for an exception to the House rule restricting resolutions for commemorative days.

For more information, please contact:

Janet Demeter, Jack’s Angels Foundation, Jacksangels1@gmail.com, Moonshot4Kids.org
Jenny Mosier, Michael Mosier Defeat DIPG Foundation, jenny@defeatDIPG.org.

PAST LEGISLATIVE ACCOMPLISHMENTS

THE CREATING HOPE REAUTHORIZATION ACT (21 U.S.C. 360ff)

Until the passage of the Creating Hope Act to establish pediatric priority review voucher (PRV) program, there was no opportunity for biotech and pharmaceutical companies to develop new therapies expressly for children with cancer and other rare and life-threatening diseases because the markets were too small.

In 2012, Congress passed the Creating Hope Act to establish the Rare Pediatric Priority Review Voucher Program.

Congress extended the voucher program several times, and passed a four-year reauthorization on December 27, 2020. Under this reauthorization, after September 30, 2024 FDA may only award vouchers for approved rare pediatric disease product applications if companies have rare pediatric disease designation for the drug granted by the FDA by September 30, 2024.

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 39 drugs expressly for pediatric rare diseases, three of which are pediatric cancer drugs.

(See Appendix A for list of vouchers awarded and sold)

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) 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. In 2019, the FDA published a draft Guidance on RACE for Children Act.

In August 2020, the requirements of the RACE for Children Act took effect.

CHILDHOOD CANCER STAR REAUTHORIZATION ACT OF 2022 (PUBLIC LAW 117-350)

On June 5, 2018, the Childhood Cancer STAR Act was signed into law.

In December 2022, the Childhood Cancer STAR Act 2.0 was reauthorized through FY2028. It modifies pediatric cancer research and related programs, including expansion of collection of and access to relevant biospecimens and supporting the transition of pediatric cancer survivors to primary care.

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

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

The STAR Act establishes a pilot program for innovative models of care for survivors, including research on the late effects of childhood cancers and collaboration among providers.

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

Advocates will ask for report language of $30 million to pediatric programs each year.

APPENDIX A

CREATING HOPE ACT RARE PEDIATRIC DISEASE VOUCHERS AWARDED AND SOLD[2,3]

Between 2014 and 2022, the FDA awarded 39 Rare Pediatric Disease priority review vouchers, representing 39 new drugs developed for rare pediatric diseases, including three for pediatric cancers (*).

RARE PEDIATRIC DISEASE	DRUG NAME	COMPANY	VOUCHER AWARD YEAR AND SALE AMOUNT	BUYER / REDEMPTION
1. Morquio A syndrome	Vimizim (elosulfase alfa)	BioMarin	2014. Sold for $67.5M	Regeneron Pharmaceuticals Inc. and Sanofi SA, redeemed for Praluent
2. High-risk neuroblastoma (*Cancer)	Unituxin (dinutuximab)	United Therapeutics	2015. Sold for $350M	AbbVie. Redeemed in 2019 for upadacitinib to treat rheumatoid arthritis.
3. Rare bile acid synthesis disorders	Cholbam (cholic acid)	Asklepion	2015. Sold for $245M	Sanofi SA, redeemed for LixiLan
4. Hereditary orotic aciduria	Xuriden (uridine triacetate)	Wellstat	2015	AstraZeneca
5. Hypophosphatasia	Strensiq (asfotase alfa)	Alexion	2015	Redeemed for ravulizumab
6. Lysosomal acid lipase deficiency	Kanuma (sebelipase alfa)	Alexion	2015	
7. Duchenne muscular dystrophy	Exondys 51 (eteplirsen)	Sarepta Therapeutics	2016. Sold for $125M	Gilead, redeemed for combination of bictegravir, emtricitabine and tenofovir alafenamide for treatment of HIV

[2] https://www.pharmalawgrp.com/blog/3/fda-extends-its-rare-pediatric-disease-prv-program/

[3] https://www.kidsvcancer.org/priority-review-vouchers/

RARE PEDIATRIC DISEASE	DRUG NAME	COMPANY	VOUCHER AWARD YEAR AND SALE AMOUNT	BUYER / REDEMPTION
8. Spinal muscular atrophy	Spinraza (nusinersen)	Biogen (Ionis Pharmaceuticals)	2016	
9. Duchenne muscular dystrophy	Emflaza (deflazacort)	Marathon	2017. Sold for $130M	Viiv Healthcare
10. Batten disease	Brineura (cerliponase alfa)	BioMarin	2017. Sold for $125M	Novartis Pharmaceuticals
11. B-cell acute lymphoblastic leukemia (*Cancer)	Kymriah (tisagenlecleucel)	Novartis		
12. Mucopolysacchari-dosis (MPS) VII	Mepsevii (vestronidase alfa-vjbk)	Ultragenyx	2017. Sold for $130M	Novartis
13. Biallelic RPE65 mutation-associated retinal dystrophy	Luxturna (voretigene neparvovec-rzyl)	Spark Therapeutics	2018. Sold for $110M	Jazz Pharmaceuticals
14. X-linked hypophosphatemia (XLH)	Crysvita (burosumab-twza)	Ultragenyx	2018. Sold for $80.6M	Gilead. Plans to use it for approval of filgotinib, a treatment for rheumatoid arthritis
15. Seizures associated with Dravet Syndrome or Lennox-Gastaut Syndrome	Epidiolex (cannabidiol oral solution)	GW Research	2018, $105M	Biohaven Pharmaceutical Holding, redeemed for rimegepant (Nurtec ODT) for treatment of migraine in adults
16. Adenosine deaminase-severe combined immunodeficiency (ADA-SCID)	Revcovi (elapegademase-lvlr) injection	Leadiant Bioscience	2018	
17. Primary haemophagocytic lymphohistiocytosis (HLH)	Gamifant (emapalumab-lzsg)	Sobi and Novimmune SA	2018. Sold for $95M in 2019	AstraZeneca
18. Cystic fibrosis with F508del mutation	Symdeko (tezacaftor/ivacaftor and ivacaftor)	Vertex Pharmaceuticals	2019	

RARE PEDIATRIC DISEASE	DRUG NAME	COMPANY	VOUCHER AWARD YEAR AND SALE AMOUNT	BUYER / REDEMPTION
19. Spinal muscular atrophy (SMA) with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene	Zolgensma (onasemnogene abeparvovec-xioi)	AveXis/Novartis	2019	
20. Cystic fibrosis	Trikafta (elexacaftor/ivacaftor/tezacaftor)	Vertex Pharmaceuticals	2019	
21. Duchenne muscular dystrophy (patients with error in exon 53)	Vyondys 53 (golodirsen)	Sarepta Therapeutics	2019. Sold in 2020 for $111M	Vifor, which plans to redeem it for vadadustat, a treatment for anemia due to chronic kidney disease
22. Neurofibromatosis type 1 (NF1)	Koselugo (selumetinib)	AstraZeneca	2020	
23. Spinal muscular atrophy in pediatric and adult patients	Evrysdi (risdiplam)	Genentech	2020	
24. Duchenne Muscular Dystrophy in patients amenable to Exon 53 Skipping	Viltepso (viltolarsen)	Nippon Shinyaku Co./NS Pharma Inc.	2020	
25. Hutchinson-Gilford Progeria syndrome and Processing-Deficient Progeroid Laminopathies	Zokinvy (lonafarnib)	Eiger BioPharmaceuticals	2020. Sold for $95M	AbbVie
26. Neuroblastoma, relapsed or refractory (* Cancer)	Danyelza (naxitamab)	Y-mAb Therapeutics	2020. Sold for $105M	United Therapeutics
27. Obesity due to proopiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1) or leptin receptor (LEPR) deficiency	Imcivree (setmelanotide)	Rhythm Pharmaceuticals	2020. Sold for $100M	Alexion
28. Primary hyperoxaluria type 1	Oxlumo (lumasiran)	Alnylam Pharmaceuticals	2020	
29. Duchenne Muscular Dystrophy	Amondys 45 (casimersen)	Sarepta Therapeutics	2021	

RARE PEDIATRIC DISEASE	DRUG NAME	COMPANY	VOUCHER AWARD YEAR AND SALE AMOUNT	BUYER / REDEMPTION
30. Molybdenum Cofactor Deficiency Type A	Nulibry (fosdenopterin)	Origin Biosciences, affiliate of BridgeBio Pharma	2021. Sold for $110M in 2022	Undisclosed
31. Plasminogen deficiency type 1 (hypoplasminogenemia)	Ryplazim (plasminogen, human-tvmh)	ProMetic Biotherapeutics	2021. Sold for $105M	Kedrion Biopharma
32. Pruritus in all types of progressive familial intrahepatic cholestasis (PFIC)	Bylvay (odevixibat)	Albireo Pharma	2021. Sold for $105M	Undisclosed
33. Cholestatic Pruritus in Patients with Alagille Syndrome	Livmarli (maralixibat)	Mirum Pharmaceuticals	2021. Sold for $110M	Undisclosed
34. Congenital athymia	Rethymic (allogeneic processed thymus tissue-agdc)	Enzyvant	2021	
35. Achondroplasia	Voxzogo (vosoritide)	BioMarin Pharmaceutical	2021. Sold for $110M in 2022	Undisclosed
36. Seizures of genetic epilepsy	Ztalmy (ganaxolone)	Marinus Pharmaceuticals	2022, Sold in 2022 for $110 million.	Undisclosed
37. Beta thalassemia	Zynteglo (betibeglogene autotemcel)	Bluebird Bio	2022. Sold in 2022 for $102M.	Argenx
38. Sphingomyelinase deficiency (ASMD)	Xenpozyme (olipudase alfa)	Sanofi	2022	
39. Cerebral adrenoleukodystrophy	Skysona (elivaldogene autotemcel)	Bluebird Bio	2022. Sold in 2023 for $95M	Bristol Myers Squibb

APPENDIX B

LEGISLATIVE PROCESS 101 – AUTHORIZATIONS, APPROPRIATIONS & REPORT LANGUAGE

WHAT IS AN AUTHORIZATION OR APPROPRIATION?

Congress makes decisions about how to spend public money in two steps: **authorizations** and **appropriations**.

First step, Congress "authorizes" that up to a certain amount of funds may be spent on a specific program or agency. For example, in the Gabriella Miller KIDS 1.0 program, Congress authorized $12 million dollars a year for eight years to the NIH Common Fund to fund pediatric research.

Second step, Congress "appropriates" the funds, e.g. it decides if it will spend any of the funds it previously authorized, and if so, what portion of the total authorized funds it will spend. For example, every year Congress considers whether to spend any funds on the NIH Common Fund and if so, how much to spend up to $12 million for that year. (To date, all authorized funds have been appropriated, thanks to Ellyn Miller and Smashing Walnuts).

The bills Kids v Cancer champions – Creating Hope Act, RACE for Children Act, and Give Kids a Chance Act – are all only authorizing bills and do not have appropriations associated with them. So, once Congress passes the bills, the programs are ready to be fully implemented.

WHAT IS REPORT LANGUAGE?

"Report language" refers to information provided in reports accompanying committee appropriation legislation. Report language directs the agencies receiving funds to spend a portion of the funds on a specific program. For example, when President Trump asked Congress to spend $50 million a year for five years on pediatric cancer research, this directive was put into report language for the funding of the National Cancer Institute. It directs the NCI to allocate an additional $50 million of its $7.3 billion budget to pediatric cancer programs. The Childhood Cancer STAR Act is also report language. It asks the NCI to spend an additional $30 million from its budget on certain pediatric programs. The CCDI is also a NCI report language supported program. The Department of Defense CDMRP and is report language program as well.

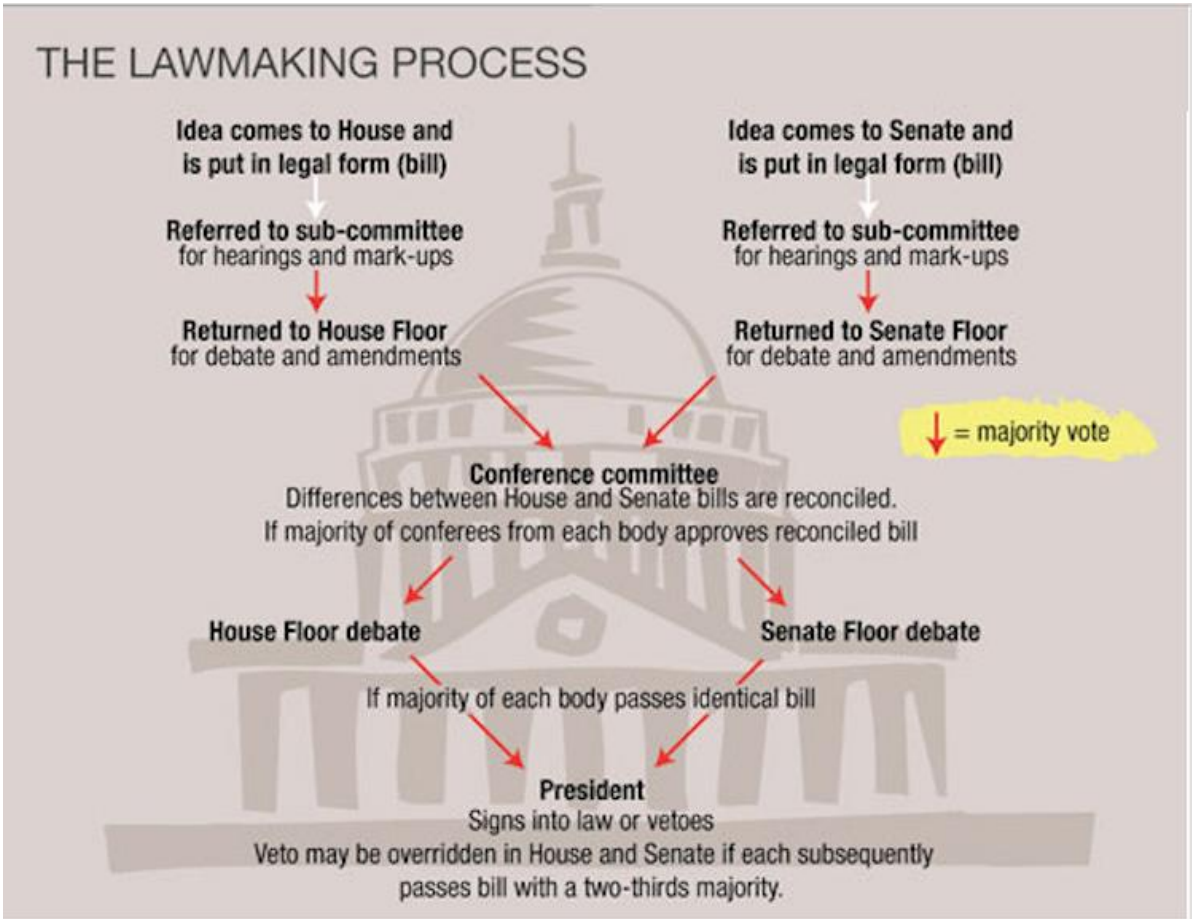
Report language does not have statutory force and agencies including NCI and DoD are not bound to comply with Congressional requests in report language. They do not view report language as new money, but a request to move their total funds around.

The DoD report language has in fact resulted in increases in the CDMRP program that are equal to what has been requested by Congress. For this program, the report language has proven to be as beneficial as an appropriation.

Other agencies do not as fully comply with report language. Historically, the NCI has not fully funded the STAR Act, President Trump's $50 per year and the CCDI programs because NCI does not view the increases in its budget as new money for specific pediatric cancer programs. NCI views the increases as funds that are already accounted for in increased costs to run the NCI.

APPENDIX C

LEGISLATIVE PROCESS 101 – HOW A BILL BECOMES A LAW



"How a Bill Becomes a Law: Process in Numerical Order[4]

1. **Introduction:** Bill is introduced in either House or Senate (First Reading), submitted to the Clerk or Secretary, and is given a title and number.

2. **Assigned:** to a committee(s).

3. **Hearings:** are held - Committee may ask interested citizens to testify for or against.

4. **Amendments:** a committee may amend a Bill, to the point of writing a new version that is substituted for the original (a "Clean Bill").

[4] For infographic and text, see: https://guides.nyu.edu/govdocs/lawmaking

5. **Committee Vote:** on whether to "report" the Bill to the full House or Senate. A Bill is usually reported favorably. If a committee is against the enactment of a particular Bill into Law, it will simply table the Bill (allows the Bill to die in Committee).

6. **Scheduled:** Leaders of the chambers schedule the Bill for debate and vote.

7. **Congressional Debate**: Bill is reported back to either the House or the Senate where it will be debated.

8. **Second Reading:** At the conclusion of the debates, the Bill is read in sections, at which time amendments can be offered.

9. **Third Reading:** Bill is read by title and voted on by the entire Senate.

10. **Senate Debate:** Bill goes through the same steps in the opposite House.

11. If the Bill is passed in substantially the same form by both House and the Senate, it is sent to the President for Signing.

12. **Conference Committee:** If different versions of the Bill are passed in each chamber, a conference committee composed of members of both chambers work out the differences and the revised bill is returned to each chamber for vote.

13. **Revision:** Revised Bill from Conference Committee is voted on again in each house.

14. **President:** President signs or vetoes the Bill. If signed, the Bill becomes a law; if vetoed, each chamber must approve the Bill by two-thirds majority for it to become law.

15. **Override:** If the President vetoes the Bill, the Congress can override the veto with a 2/3 vote."