PEDIATRIC CANCER LEGISLATIVE YEARBOOK

DECEMBER 2021

Compiled by

KIDS V CANCER

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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.

If you would like to get involved in the legislative process, please contact us. We also host CLIMB THE HILL DAY zooms for kids and young adults to meet with their Senators and Representatives. Please contact Jenn@kidsvcancer.org.

LEGISLATIVE ACCOMPLISHMENTS

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 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 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 – 2021: Congress passes the Childhood Cancer STAR Act, for research, tracking of incidences and quality of life research for childhood cancer survivors.
Lead advocate: Alliance for Childhood Cancer

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- 2021: 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.
Lead advocate: Smashing Walnuts

2012- **2021:** Congress includes in the DoD medical research 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

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

LEGISLATIVE AGENDA FOR 2022: AUTHORIZATIONS

GIVE KIDS A CHANCE ACT, H.R.5416

What is the problem/opportunity:

Adult cancer clinical trials are currently experiencing a great step forward and are achieving curative outcomes through *combination* therapies of novel drugs for terminally ill adult cancer patients. However, these clinical trials of combinations of novel therapies are not available to kids.

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 even theoretically 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. The Give Kids a Chance Act also authorizes FDA to require companies undertake preclinical studies for their pediatric study plans.

Accomplishments:

In September 2021, Rep. G.K. Butterfield (D-NC-1) and Michael McCaul (R-TX-10) introduced the bill in the House of Representatives.

Next steps:

Advocates ask Congress to pass the Give Kids a Chance Act as part of the PDUFA VII bill in September 2022.

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GABRIELLA MILLER KIDS FIRST RESEARCH ACT 2.0, H.R 623, S.1521

What is the problem/opportunity:

The Gabriella Miller Kids First Research Act 2.0 would redirect penalties from violations of Foreign Corrupt Practice Act levied against pharmaceutical manufacturers, medical device manufacturers, cosmetic manufactures and natural supplement manufacturers, towards the Kids First Pediatric Research Program at the National Institutes of Health.

Accomplishments:

The Kids First Research Act 2.0 was introduced in the House in January 2020 by Congresswoman Jennifer Wexton (D-VA-10) and Rep. Tom Cole (R-OK-04), and in the Senate by Senators Tim Kaine (D-VA) and Jerry Moran (R-KS).

The bill has gone through a hearing by the Energy and Commerce Committee. It has 82 House cosponsors. The bill has six Senate cosponsors.

Next steps:

Advocates ask Congress to pass the Kids First Research Act 2.0 into law.

For more information, please contact:

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

CREDIT FOR CARING ACT OF 2021, H.R. 3321, S.1670

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 20 cosponsors. It was simultaneously introduced in the House by Rep. Linda Sánchez, (D-CA-38) and Rep. Tom Reed (R-NY-23) and has 64 cosponsors.

Next steps:

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

For more information, please contact:

www.creditforcaring.com

ACCELERATING KIDS' ACCESS TO CARE ACT, H.R. 3089, S. 1544

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 determine that care be delivered out-of-state.

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 has 54 cosponsors, and by Sen. Chuck Grassley (R-IA) and Sen. Michael Bennet (D-CO) in the Senate, with 14 cosponsors at present.

Next steps:

Advocates are asking Congress to pass the Accelerating Kids' Access to Care Act.

For more information, please contact:

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JONNY WADE PEDIATRIC CANCER RESEARCH ACT, H.R.3032

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). It currently has 5 cosponsors.

Next steps:

Advocates ask for Congress to pass the bill.

For more information, please contact:

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FAIRNESS TO KIDS WITH CANCER ACT, H.R.2210

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 current Congress in March 2021. It has 9 cosponsors in the House.

Next steps:

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

For more information, please contact:

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LEGISLATIVE AGENDA FOR 2022: APPROPRIATIONS

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

DEPARTMENT OF DEFENSE MEDICAL RESEARCH PROGRAM

What is the problem/opportunity:

The Department of Defense (DoD) has a shadow NIH, a $1.5 billion medical research program through the Congressionally Directed Medical Research Program (CDMRP). Within the CDMRP, a $5 million Peer Reviewed Cancer Research Program (PRCRP) provides funding for certain cancers.

Accomplishments:

Over the past few years, the pediatric cancer community created and secured the continued inclusion of three pediatric cancer eligibility categories in the PRCRP program.

The three pediatric cancer eligibility categories are: 1) neuroblastoma, 2) pediatric brain tumors, and 3) cancers in children, adolescents and young adults (AYA).

In 2020 these three categories were awarded $18.8 million:

- $10.8 million for cancers in children and AYAs
- $4.8 million for neuroblastoma
- $3.2 million for pediatric brain tumors.

The 2017-2020 total for these categories is $55 million.

In 2020, Rep. Ann Kirkpatrick (D-AZ), a member of the DoD Subcommittee, championed a stand-alone program for cancers in children and AYAs, aiming for $30 million for fiscal year 2021. The stand-alone program was not authorized, but the $115 million PRCRP report language included cancers in children and AYAs, neuroblastoma, and pediatric brain tumors.

Next steps:

The community ask Congress to continue to include pediatric cancers in the PRCRP and fund the PRCRP.

For more information, please contact:

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LEGISLATIVE AGENDA FOR 2022: REPORT LANGUAGE

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, FY20, and FY21.

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 each year.

For more information, please contact:

Sarah Milberg, Alliance for Childhood Cancer, smilberg@allianceforchildhoodcancer.org

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.

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.

In 2020, NCI has started the following tasks in alignment with CCDI's goals: Conduct a landscape analysis of existing childhood and AYA cancer data, tools and repositories that can be connected under the CCDI, develop the National Childhood Cancer Registry (NCCR), support ongoing research to develop a preclinical data commons for childhood cancers, establish a framework, or technical infrastructure, that will allow various types of childhood cancer and clinical care data and tools, including the NCCR and preclinical data commons, to be connected to one another, expand comprehensive data collection to involve more institutions engaged in childhood cancer and survivorship research, and other initiatives.

Next steps:

Advocates will ask Congress' budget for FY2023 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 2022: RESOLUTIONS

HOUSE RESOLUTION FOR DIPG (H.Res. 404, S.231)

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. 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 House Rules restricted so-called "commemorative" resolutions in the 1990's due to excessive overuse at the time, to conserve important floor-time. An exception clause was introduced in 2016 for bereavement and urgently needed action. In order to bypass the restriction rule either a super-majority (290) cosponsors are needed in the 117th Congress, or presenting the case to House Leadership that this resolution fits the requirements for being considered exceptional. There are currently 110 cosponsors.

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 with 22 original cosponsors, by Debbie Dingell (D-MI-12) and David Joyce (R-OH-14).

Senator Marco Rubio (R-FL) and Senator Jack Reed (D-RI) first introduced the DIPG/Pediatric Brain Cancer Awareness Resolution in the U.S. Senate in 2019, and again in 2020 and 2021 with passage of unanimous consent on each occasion. 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 House E&C Committee for an official referral or deferral of jurisdiction to House leadership to ultimately decide the fate of this Resolution, and an exception to the House rule restricting resolutions for commemorative days.

For more information, please contact:

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PAST LEGISLATIVE AUTHORIZATIONS

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

What is the problem/opportunity:

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.

Accomplishments:

Congress extended the PRV 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 34 drugs expressly for pediatric rare diseases, three of which are pediatric cancer drugs.

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

For more information, please contact:

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

What is the problem/opportunity:

There are over a thousand 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 cancers in adults.

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.

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.

Next steps:

The FDA will publish a final Guidance on the RACE for Children Act.

For more information, please contact:

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APPENDIX A

LIST OF CREATING HOPE ACT RARE PEDIATRIC DISEASE VOUCHERS AWARDED[1,2]

Between 2014 and 2021, the FDA awarded 34 Rare Pediatric Disease priority review vouchers, representing 34 new drugs developed for rare pediatric diseases, including three for pediatric cancers (*).

- 2014, BioMarin: Awarded for Vimizim (elosulfase alfa) to treat Morquio A syndrome. Sold to Sanofi and Regeneron for $67 million. Redeemed for approval of Praluent (alirocumab).
- (*)2015, United Therapeutics: Awarded for Unituxin (dinutuximab) to treat high-risk neuroblastoma. Sold to AbbVie for $350 million. Unused.
- 2015, Asklepion Pharmaceuticals: Awarded for Cholbam (cholic acid) to treat rare bile acid synthesis disorders. Transferred to Retrophin. Sold to Sanofi for $245 million. Redeemed for approval of type 2 diabetes drug.
- 2015, Wellstat Therapeutics: Awarded for Xuriden (uridine triacetate) to treat hereditary orotic aciduria. Transferred to AstraZeneca. Unused.
- 2015, Alexion Pharmaceuticals: Awarded for Strensiq (asfotase alfa) to treat hypophosphatasia. Unused.
- 2015, Alexion Pharmaceuticals: Awarded for Kanuma (sebelipase alfa) to treat lysosomal acid lipase (LAL) deficiency. Redeemed for approval of Ultomiris (Ravulizumab-Cwvz) to treat paroxysmal nocturnal hemoglobinuria (PNH).
- 2016, Sarepta Therapeutics: Awarded for Eteplirsen to treat Duchenne muscular dystrophy. Sold to Gilead for $125 million. Redeemed for approval of HIV treatment.
- 2016, Ionis Pharmaceuticals: Awarded for Spinraza (nusinersen) to treat spinal muscular atrophy (SMA). Unused.
- 2017, Marathon Pharmaceuticals: Awarded for Emflaza (deflazacort) to treat Duchenne muscular dystrophy. Unused.
- 2017, BioMarin: Awarded for Brineura (cerliponase alfa) to treat Batten disease. Sold to an undisclosed party for $125 million.
- (*)2017, Novartis: Awarded for Kymriah (tisagenlecleucel) to treat B-cell acute lymphoblastic leukemia. Unused.
- 2017, Ultragenyx Pharmaceutical: Awarded for Mepsevii to treat mucopolysaccharidosis (MPS) VII. Sold to Novartis for $130 million. Redeemed for approval of siponimod to treat secondary progressive multiple sclerosis (SPMS).

[1] https://www.pharmalawgrp.com/blog/3/fda-extends-its-rare-pediatric-disease-prv-program/
[2] https://www.kidsvcancer.org/priority-review-vouchers/

- 2017, Spark Therapeutics: Awarded for Luxturna (voretigene neparvovec-rzyl) to treat biallelic RPE65 mutation-associated retinal dystrophy. Sold to Jazz Therapeutics for $110 million. Unused.
- 2018, Ultragenyx: Awarded for Crysvita (burosumab-twza) to treat X-linked hypophosphatemia (XLH). Sold to an undisclosed party for $80.6 million.
- 2018, GW Pharma: Awarded for Epidiolex (cannabidiol) to treat Lennox-Gastaut syndrome and Dravet syndrome. Unused.
- 2018, Leadiant Bioscience: Awarded for Revcovi (elapegademase-lvlr) to treat Adenosine Deaminase-Severe Combined Immunodeficiency (ADA-SCID). Unused.
- 2018, Sobi and Novimmune: Awarded for Gamifant (emapalumab-lzsg) to treat primary haemophagocytic lymphohistiocytosis (HLH). Sold the voucher for $95 million to AstraZeneca.
- 2019, Vertex Pharmaceuticals: Awarded for Symdeko (tezacaftor/ivacaftor and ivacaftor) to treat Cystic fibrosis with F508del mutation.
- 2019, AveXis/Novartis: Awarded for Zolgensma (onasemnogene abeparvovec-xioi) for treatment of spinal muscular atrophy (SMA) with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene.
- 2019, Vertex Pharmaceuticals: Awarded for Trikafta (elexacaftor/ivacaftor/tezacaftor), a treatment for cystic fibrosis.
- 2019, Sarepta Therapeutics: Awarded for Vyondys 53 (golodirsen) to treat Duchenne muscular dystrophy (patients with error in exon 53). Sold for $111 million to Vifor Pharma.
- 2020, AstraZeneca: Awarded for Koselugo (selumetinib), a treatment for Neurofibromatosis type 1 (NF1).
- 2020, Genentech: Awarded for Evrysdi (risdiplam) to treat spinal muscular atrophy in pediatric and adult patients.
- 2020: Nippon Shinyaku Co./NS Pharma: Awarded for Viltepso (viltolarsen) to treat Duchenne Muscular Dystrophy in patients amenable to Exon 53 skipping.
- 2020, Eiger BioPharmaceuticals: Awarded for Zokinvy (lonafarnib) to treat Hutchinson-Gilford Progeria syndrome and Processing-Deficient Progeroid Laminopathies. Sold for $95 million to AbbVie.
- (*)2020, Y-mAb Therapeutics: Awarded for Danyelza (naxitamab) to treat relapsed or refractory neuroblastoma. Sold for $105 million to United Therapeutics.
- 2020, Rhythm Pharmaceuticals: Awarded for Imcivree (setmelanotide) to treat obesity due to proopiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1) or leptin receptor (LEPR) deficiency. Sold for $100 million to Alexion.
- 2020, Alnylam Pharmaceuticals: Awarded for Oxlumo (lumasiran) to treat Primary hyperoxaluria type 1.
- 2021, Sarepta Therapeutics: Awarded for Amondys 45 (casimersen) to treat Duchenne Muscular Dystrophy in patients with gene amenable to exon 45 skipping.

- 2021, Origin Biosciences: Awarded for Nulibry (fosdenopterin) as a treatment for Molybdenum Cofactor Deficiency Type A.
- 2021, ProMetic Biotherapeutics /Liminal Biosciences: Awarded for Ryplazim (plasminogen, human-tvmh) to treat Plasminogen deficiency type 1 (hypoplasminogenemia). Sold for $105 million to Kedrion Biopharma.
- 2021, Albireo Pharma: Awarded for Bylvay (odevixibat) to treat pruritus in all types of progressive familial intrahepatic cholestasis. Sold for $105 million to unnamed party.
- 2021, Mirum Pharmaceuticals: Awarded for Livmarli (maralixibat) to treat cholestatic pruritus in patients with Alagille syndrome. Sold for $110 million to unnamed party.
- 2021, Enzyvant: Awarded for Rethymic (allogeneic processed thymus tissue-agdc) to treat congenital athymia.

In addition, Teva Pharmaceuticals paid $150 million for a rare pediatric priority review voucher purchased from an undisclosed company in 2017 and redeemed it for expedited review of their migraine med, Ajovy (fremanezumab-vfrm).

Incyte Corp. bought a voucher in September 2020 for $120 million from unnamed party and redeemed it for Jakafi (ruxolitinib) approval to treat adults with certain types of myelofibrosis and polycythemia vera, and adults and children 12 years of age and older with acute graft-versus-host disease (GVHD).

APPENDIX B

LEGISLATIVE PROCESS 101 – AUTHORIZATIONS, APPROPRIATIONS & REPORT LANGUAGE

WHAT IS AN AUTHORIZATION OR APPROPRIATION?[3]

Congress is charged by the Constitution with making decisions about how to spend public money. In practice, these spending decisions are split into two steps: **authorization** and **appropriations**.

"Authorization" is done by Congress via legislation that "can establish, continue, or modify an agency, program, or activity for a fixed or indefinite period of time," per the Congressional Research Service. In other words, authorization is Congress saying that money **may** be spent on a given item—not that it necessarily **will** be spent on that item." The House Energy and Commerce Committee and the Senate Health, Education, Labor and Pensions Committee are both authorization committees.

"Appropriations" are done by Congress via legislation that authorizes agencies to make payments from the federal Treasury (i.e. it allows them to spend the money that had previously been authorized). Appropriations bills are ordinarily passed each year, but in recent years it has been common for Congress to fund the government "on autopilot" via **continuing resolutions** that simply allow agencies to continue spending the same amount of money they were spending under the previous funding bill."

WHAT IS REPORT LANGUAGE?[4]

"Report language refers to information provided in reports accompanying committee reported legislation as well as joint explanatory statements, which are included in conference reports. Report language contains more detailed guidance to departments and

[3] See https://indivisible.org/resource/legislative-process-101-authorization-vs-appropriation.

[4] Appropriations Bills: What Is Report Language? Congress and the Legislative Process March 23, 2010, Congressional Research Service, (see: https://www.everycrsreport.com/files/20100323_98-558_cba7e28584033a268db936509835e7948a369635.pdf)

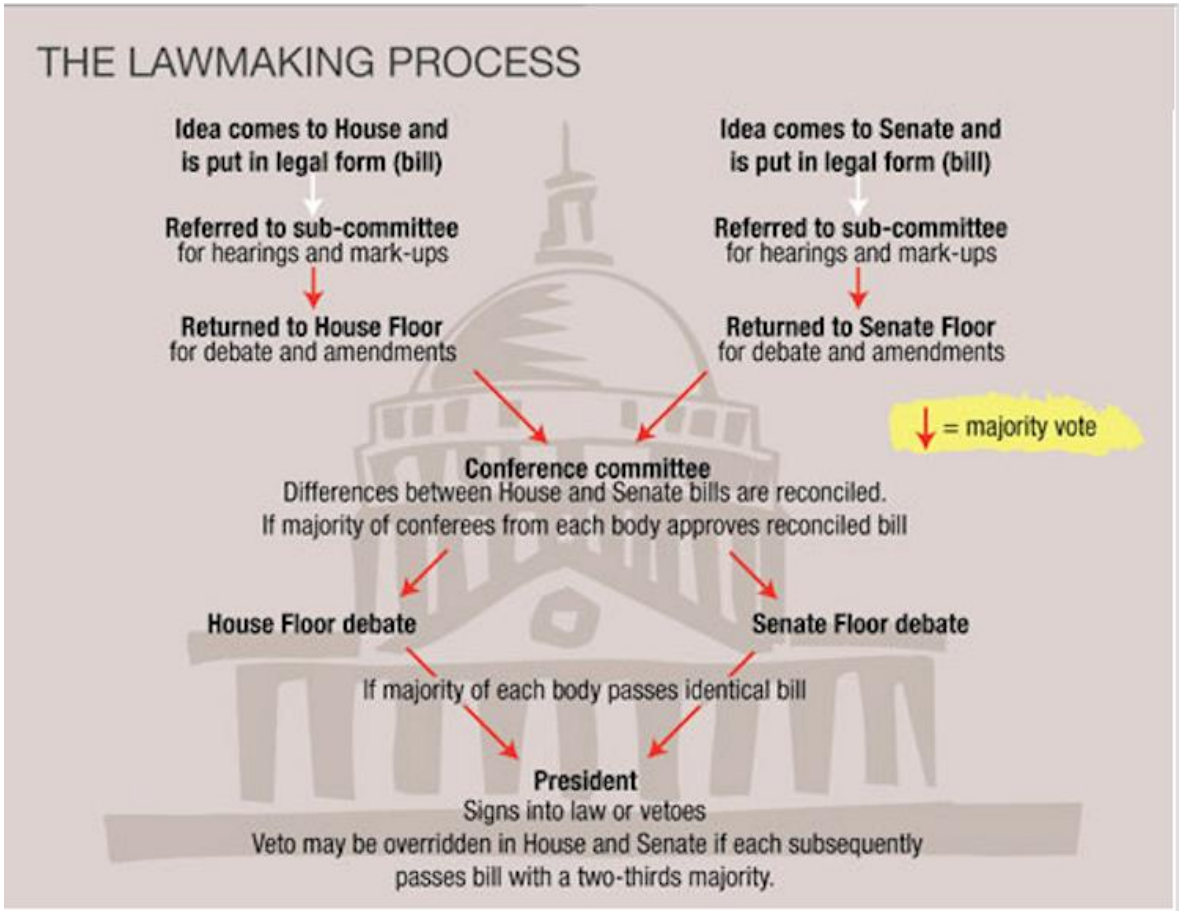
agencies than is provided in related appropriations bills or legislative text in conference reports."

Specifically, the pediatric cancer community has several initiatives that use report language to ask for increased funding – eg the STAR Act, the DoD program and the CCDI program.

Report language does not have statutory force and NCI and DoD are not legally bound to it. However, NCI and DoD take them seriously because they must justify their budget requests annually to the appropriations committees.

APPENDIX C

LEGISLATIVE PROCESS 101 – HOW A BILL BECOMES A LAW



"How a Bill Becomes A Law: Process in Numerical Order[5]

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").

[5] 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."