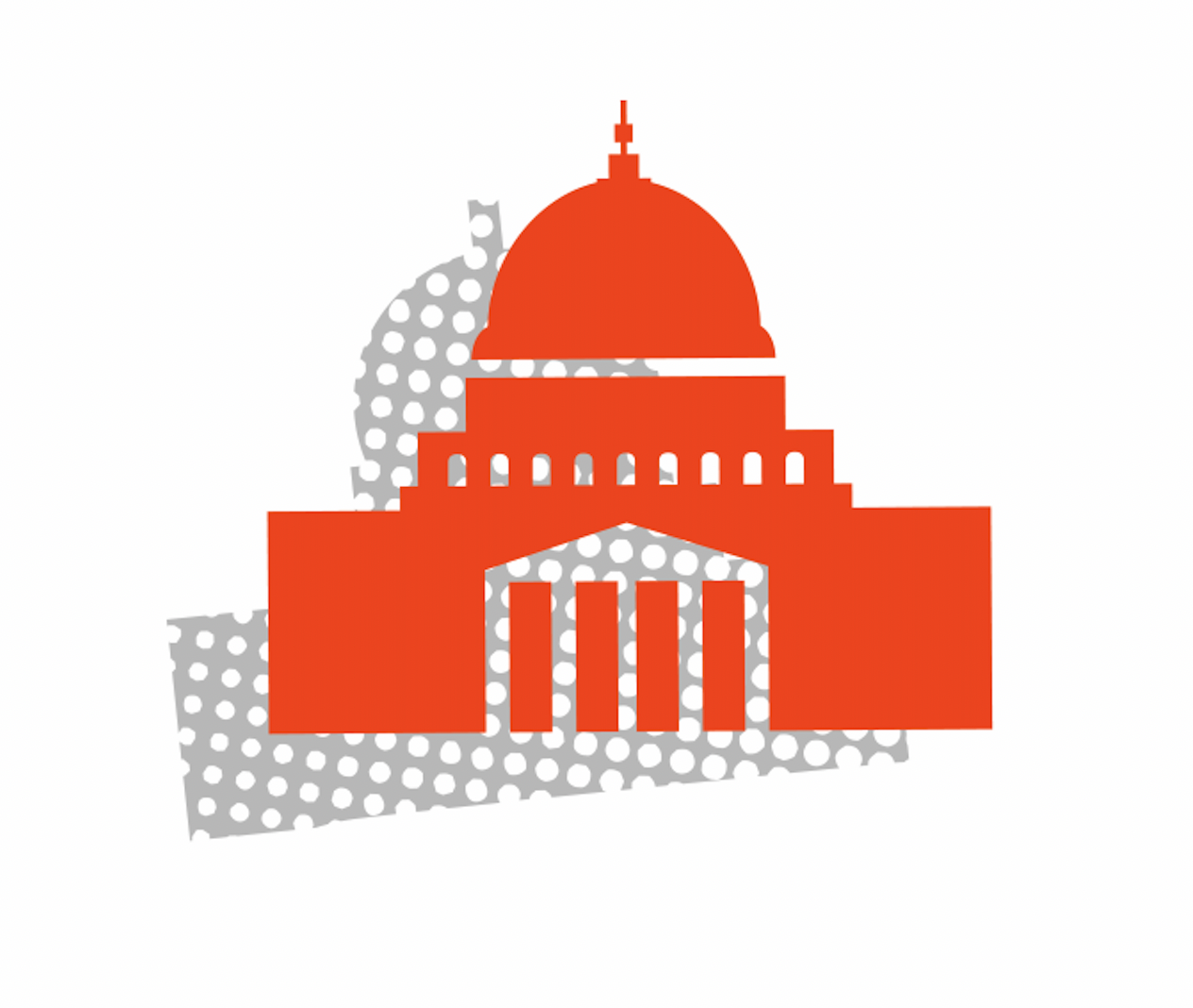
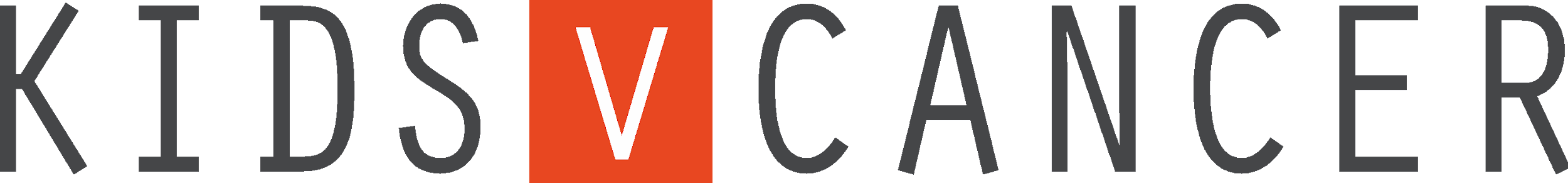
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**PEDIATRIC CANCER LEGISLATIVE YEARBOOK**

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**FEBRUARY 2024**

**Edited by**

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\*Bills championed by Kids v Cancer

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

## LEGISLATIVE ACCOMPLISHMENTS

**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*

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

**2014:** Congress passes the **Gabriella Miller Kids First Research Act**, authorizing $126 million to be allocated over 10 years to pediatric research.

*Lead advocate: Smashing Walnuts*

**2016:** Congress reauthorizes **Creating Hope Act**, **Pediatric priority review voucher program.** *Lead advocate: Kids v 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*

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

**2018:** Congress passes the **Childhood Cancer STAR Act** to fund a biospecimen bank, survivorship and tracking data. *Lead* *advocates: St Baldricks, CAC2*

**2020:** Congress reauthorizes the **Creating Hope Act** **Pediatric priority review voucher program** extending the FDA’s authority to issue vouchers until September 2024 and extending companies’ authority to exercise vouchers until September 2026.

*Lead advocate: Kids v Cancer*

**2020:** Congress includes the **Global Hope Act** in report language todirect the Department of State to address childhood cancer globally.

**2022:** Congress passes the **Childhood Cancer STAR Act 2.0**

*Lead Advocate: St Baldricks*

**2022**: Senate passes **S.Res.642**, a resolution designating May 17, 2022 as DIPG Pediatric Brain Cancer Awareness Day. *Lead Advocate*: *Jack’s Angels Foundation*

## LEGISLATIVE PRIORITIES FOR 2023

### \*GIVE KIDS A CHANCE ACT, HR 3433, S 2897

What is the problem/opportunity:

Over the past decade, cancer scientists have developed new classes of drugs with exciting potential. 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 to direct companies to study combinations of their novel cancer drugs in kids.

Accomplishments:

The Give Kids a Chance Act was included as section 714 of House PDUFA bill, HR 7667 in 2020. 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. All riders to the PDUFA bill, including Give Kid a Chance Act, we stripped in the Senate PDUFA bill, which was passed clean.

Next steps:

The Give Kids a Chance Act was reintroduced in the House and Senate with the same language that passed in the House in the 117th Congress.

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Nancy Goodman, [nancygoodman@kidsvcancer.org](mailto:nancygoodman@kidsvcancer.org)

### \*CREATING HOPE REAUTHORIZATION ACT, HR 7384

What is the problem/opportunity:

Before enactment of the Creating Hope Act in 2012, there was almost no drug development expressly for children with life threatening illnesses, including pediatric cancers, The markets were too small.

Accomplishments:

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

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. $6 billion of vouchers have been traded. Since the enactment of the Creating Hope Act, the FDA has approved 60 drugs expressly for pediatric rare diseases, six of which are pediatric cancer drugs.

Congress extended the voucher program several times most recently 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 a rare pediatric disease designation for the drug granted by the FDA by September 30, 2024.

Next steps:

Advocates will ask Congress to reauthorize the Creating Hope Act *by September 30, 2024.*

For more information please contact:

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Eric Kim (McCaul), eric.kim@mail.house.gov

Nancy Goodman, [nancygoodman@kidsvcancer.org](mailto:nancygoodman@kidsvcancer.org)

### GABRIELLA MILLER KIDS FIRST KIDS 2.0 ACT, S 1624, HR 3391

What is the problem/opportunity:

Pediatric cancer receives inadequate funding by the National Institutes of Health (NIH).

Accomplishments:

In 2014, The Gabriella Miller Kids First Research Act was signed into law, authorizing and appropriating through the NIH Gabriella Miller Kids First Research Program, $126 million over 10 years. Half of such funds go to pediatric cancer research.

Next steps:

In the last Congress, the bill passed in the House and fell short of passage in the Senate by one vote.

In this Congress, the bill passed both the House Sub-committee and full Committee with unanimous consent. It is going to Senate mark-up on 9/21/23. A

For more information, please contact:

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### ACCELERATING KIDS’ ACCESS TO CARE ACT, S 2372, HR 4758

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. However, this process can be slow and onerous.

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 onerous screening requirements.

For more information, please contact:

Megan Herber, megan.herber[@faegredrinker.com](mailto:nicholas.manetto@faegredrinker.com)

## ADDITIONAL LEGISLATIVE GOALS FOR 2024

### Pediatric Cancer Drug Supply Act, HR 6963

The Secretary shall carry out a pilot program under which the Secretary enters into agreements with manufacturers to purchase and maintain not less than a 6-month reserve supply, to be held by such manufacturers, of each covered pediatric cancer drug.

For more information please contact:

Jordan Brossi (Eshoo) [Jordan.brossi@mail.house.gov](mailto:Jordan.brossi@mail.house.gov)

### Innovation in Pediatric Drugs Act, HR [6664](https://www.congress.gov/bill/118th-congress/house-bill/6664?q=%7B%22search%22%3A%22eshoo%22%7D&s=3&r=10)

The Innovation in Pediatric Drugs Act ends the orphan drug exemption under the Pediatric Equity Act for non-cancer for required pediatric study plans. For cancer drugs, this exemption was terminated as part of the RACE for Children Act.

For more information please contact:

Jordan Brossi (Eshoo) [Jordan.brossi@mail.house.gov](mailto:Jordan.brossi@mail.house.gov)

### Childhood Cancer Clinical Trials Act, HR 5647

The Childhood Cancer Clinical Trials Act would require insurers to cover out-of-network clinical trials for routine care for children with cancer, if no in-network provider is available.

For more information, please contact:

Eric Kim (McCaul) [Eric.Kim@mail.house.gov](mailto:Eric.Kim@mail.house.gov)

### COMPREHENSIVE CANCER SURVIVORSHIP ACT, S 221, HR 4363

What is the problem/opportunity:

More than 95% of the 500,000 childhood cancer survivors have a significant health problem by the time they are 45. However, there is no standard of care for the treatment of survivors.

For more information, please contact:

Rep. Debbie Wasserman Schultz

Rep. Brian Fitzpatrick

Rep. Mark DeSaulnier

Sen. Amy Klobuchar

Sen. Ben Cardin

Steve Wosahla, Children’s Cancer Cause, swosahla@childrenscause.org

### FAIRNESS TO KIDS WITH CANCER ACT S 2624, HR 5405

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 will adjust the level of federal investment in pediatric cancer research to match the percentage of American citizens under the age of 18, based on U.S. Census data. According to 2010 Census data, 24 percent of the U.S. population is under the age of 18, meaning the allocation for pediatric cancer research would increase to 24% of the total federal investment.

For more information, please contact:

Jacqueline Collie (Fitzpatrick), Jacqueline.collie@mail.house.gov

Mina Carroll, [info@stormtheheavens.org](mailto:info@stormtheheavens.org)

### Pediatricians Accelerate Childhood Therapies Act, HR 4714, S 2345

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.

For more information, please contact:

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

### Data for pediatric brain cancer act, HR 6288

NCI shall carry out a program, to be known as the Pediatric Brain Tumor Real-World Data Registry Program, to strengthen and expand activities related to the collection, sharing, and use of real-world data for children with brain tumors.

For more information, see

Rep Ami Bera

### **Knock Out Cancer Act, HR 6807**

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.

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.

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](mailto:Matthew.Clarkin@mail.house.gov)

## APPROPRIATIONS AND REPORT LANGUAGE

### ­­­­­­­­­­­­CHILDHOOD CANCER STAR ACT REAUTHORIZATION

What is the problem/opportunity:

Pediatric cancer research is underfunded at the National Cancer Institute.

Accomplishments:

On June 5, 2018, the Childhood Cancer STAR Act was signed into law. It includes report language asking for $30 million of the NCI budget to be allocated to state cancer registries for pediatric cancers, pediatric cancer survivorship programs, and a pediatric cancer biospecimen program. Report language is not newly authorized funds. It is a request for a shift of funds within the NCI budget.

In December 2022, the Childhood Cancer STAR Act 2.0 was reauthorized through FY2028.

Next steps:

Advocates ask for report language to fund these pediatric programs each year.

For more information, please contact:

Eric.kim@mail.house.gov

### DEPARTMENT OF DEFENSE MEDICAL RESEARCH PROGRAM

What is the problem/opportunity:

The Department of Defense runs a $1.5 billion medical research program through the Congressionally Directed Medical Research Program. Within the CDMRP, the Peer Reviewed Cancer Research Program 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 cancer eligibility categories in the Peer Reviewed Cancer Research Program. For FY 2023 the Peer Reviewed Cancer Research Program 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:

Advocates ask Congress to continue to include current eligible pediatric cancers in the Peer Reviewed Cancer Research Program and to fund the Peer Reviewed Cancer Research Program.

For more information, please contact:

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Baillee Brown (Rep. Scott Peters, D-CA), [Baillee.Brown@mail.house.gov](mailto:Baillee.Brown@mail.house.gov)  
Dean Crowe, Dean@rallyfoundation.org

## PAST LEGISLATIVE ACHIEVEMENTS

### \*RACE FOR CHILDREN ACT (21 USC 355C)

What is the problem/opportunity:

Of the thousands of drugs in development for adult cancers, only a handful were also studied in children’s cancers.

Accomplishments:

In 2017, the RACE for Children Act (“RACE 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).

The RACE Act requires companies developing targeted cancer drugs for adults to also develop those drugs for children.

Pursuant to requirements of the RACE 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 RACE for Children Act took effect.

Since passage of the RACE Act, 80% of all newly approved cancer drugs relevant to pediatric cancers now have required pediatric studies.

For more information, please contact:

Nancy Goodman, nancygoodman@kidsvcancer.org

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## APPENDIX: NEW DRUGS FOR CHILDREN WITH LIFE THREATENING ILLNESSESS WHOSE FDA APPROVAL CAME WITH A RARE PEDIATRIC PRIORITY REVIEW VOUCHER

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
| **RARE PEDIATRIC DISEASE** | **DRUG NAME** | **COMPANY** | **VOUCHER AWARD YEAR AND SALE AMOUNT** | **BUYER** |
| 1. Morquio A syndrome | Vimizim | BioMarin | 2014, | Regeneron Pharmaceuticals Inc. and Sanofi SA, redeemed for Praluent |
| $67.5M |
| 2. High-risk neuroblastoma \*Cancer | Unituxin | United Therapeutics | 2015, | AbbVie. Redeemed in 2019 for upadacitinib to treat rheumatoid arthritis. |
| $350M |
| 3. Rare bile acid synthesis disorders | Cholbam | Asklepion | 2015, | Sanofi SA, redeemed for LixiLan |
| $245M |
| 4. Hereditary orotic aciduria | Xuriden | Wellstat | 2015 | AstraZeneca |
| 5. Hypophosphatasia | Strensiq | Alexion | 2015 | Redeemed for ravulizumab |
| 6. Lysosomal acid lipase deficiency | Kanuma (sebelipase alfa) | Alexion | 2015 |  |
| 7. Duchenne muscular dystrophy | Exondys 51 | Sarepta Therapeutics | 2016, | Gilead, redeemed for combination of bictegravir, emtricitabine and tenofovir alafenamide for treatment of HIV |
| $125M |
| 8. Spinal muscular atrophy | Spinraza | Biogen (Ionis Pharmaceuticals) | 2016 |  |
| 9. Duchenne muscular dystrophy | Emflaza | Marathon | 2017, | Viiv Healthcare |
| $130 million |
| 10. Batten disease | Brineura | BioMarin | 2017, | Novartis Pharmaceuticals |
| $125M |
| 11. B-cell acute lymphoblastic leukemia \*Cancer | Kymriah | Novartis |  |  |
| 12. Mucopolysacchari-dosis (MPS) VII | Mepsevii | Ultragenyx | 2017, | Novartis |
| $130M |
| 13. Biallelic RPE65 mutation-associated retinal dystrophy | Luxturna | Spark Therapeutics | 2018, | Jazz Pharmaceuticals |
| $110M |
| 14. X-linked hypophosphatemia (XLH) | Crysvita (burosumab-twza) | Ultragenyx | 2018, | Gilead. Plans to use it for approval of filgotinib, a treatment for rheumatoid arthritis |
| $80.6M |
| 15. Seizures associated with Dravet Syndrome or Lennox-Gastaut Syndrome | Epidiolex (cannabidiol oral solution) | GW Research | 2018, | Biohaven Pharmaceutical Holding, redeemed for rimegepant (Nurtec ODT) for treatment of migraine in adults |
| $105M |
| 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.  $95 million i | AstraZeneca |
| 18. Cystic fibrosis with F508del mutation | Symdeko | Vertex Pharmaceuticals | 2019 |  |
| 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 $95 million | AbbVie |
| 26. Neuroblastoma, relapsed or refractory | Danyelza (naxitamab) | Y-mAb Therapeutics | 2020, sold for $105million | United Therapeutics |
| \* Cancer |
| 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 $100 million | Alexion |
| 28. Primary hyperoxaluria type 1 | Oxlumo (lumasiran) | Alnylam Pharmaceuticals | 2020 |  |
| 29. Duchenne Muscular Dystrophy in patients with gene amenable to exon 45 skipping | Amondys 45 (casimersen) | Sarepta Therapeutics | 2021 |  |
| 30. Molybdenum Cofactor Deficiency Type A | Nulibry (fosdenopterin) | Origin Biosciences, affiliate of BridgeBio Pharma | 2021, sold for $110 million in 2022 | Undisclosed |
| 31. Plasminogen deficiency type 1 (hypoplasminogenemia) | Ryplazim (plasminogen, human-tvmh) | ProMetic Biotherapeutics /Liminal Biosciences | 2021, sold for $105 million | Kedrion Biopharma |
| 32. Pruritus in all types of progressive familial intrahepatic cholestasis (PFIC) | Bylvay (odevixibat) | Albireo Pharma | 2021, sold for $105 million | Undisclosed |
| 33. Cholestatic Pruritus in Patients with Alagille Syndrome | Livmarli (maralixibat) | Mirum Pharmaceuticals | 2021, sold for $110 million | Undisclosed |
| 34. Congenital athymia | Rethymic (allogeneic processed thymus tissue-agdc) | Enzyvant | 2021 |  |
| 35. Achondroplasia | Voxzogo (vosoritide) | BioMarin Pharmaceutical | 2021, sold for $110 million 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 $102 million. | Argenx |
| 38. Sphingomyelinase deficiency (ASMD) | Xenpozyme (olipudase alfa) | Sanofi | 2022 |  |
| 39. Cerebral adrenoleukodystrophy | Skysona (elivaldogene autotemcel) | Bluebird Bio | 2022. Sold in 2023 for $95 million | Bristol Myers Squibb |

## APPENDIX: HOW A BILL BECOMES A LAW

