

FDA Approved Drugs For Childhood Cancers *

updated 06/10/2021

Drug	Approved for	Type	Original Approval	Pediatric Approval	Indication
Mercaptopurine	Adults/Peds	Chemo	9/11/1953	4/28/2014	ALL
Cyclophosphamide	Adults/Peds	Chemo	11/16/1959	****	Leukemia, lymphoma, NBL, retinoblastoma 
Vincristine	Adults/Peds	Chemo	7/10/1963	***	ALL, lymphomas, Wilms, rhabdomyosarcoma, NB
Dactinomycin	Adults/Peds	Chemo	12/10/1964	8/23/2013	Ewing Sarcoma, sarcoma botryoides
Cytarabine	Adults/Peds	Chemo	6/17/1969	***	Acute non-lymphocytic leukemia
Procarbazine	Adults/Peds	Chemo	7/22/1969	***	Hodgkin lymphoma
Daunorubicin	Adults/Peds	Chemo	12/19/1979	1/30/1998	ALL 
Pegaspargase	Peds/AYA	NME**	2/2/1994	4/24/2006	ALL *ct
*FDAMA, enacted Nov. 21, 1997, amended the Federal Food, Drug, and Cosmetic Act relating to the regulation of food, drugs, devices, and biological products					
Clofarabine	Pediatrics	NME**	12/28/2004	9/1/2017	Refractory ALL *ct
Nelarabine	Adults/Peds	NME**	10/28/2005	9/1/2017	T-cell ALL *ct
Dasatinib	Adults/Peds	Targeted Therapy	6/28/2006	11/9/2017	Ph+CML in the chronic phase *ct
Imatinib	Adults/Peds	Targeted	9/27/2006	11/9/2017	Ph+ ALL and PH+ CML * ct
Nilotinib	Adults/Peds	Targeted	10/29/2007	3/22/2018	Ph+CML in the chronic phase
Ipilimumab	Adults/Peds	MAB***	3/25/2010	7/21/2017	Unresectable or metastatic melanoma ≥ 12 yrs
Everolimus	Adults/Peds	Chemo	10/29/2010	9/25/2012	SEGA / subependymal giant cell astrocytoma.
Asparaginase Erwinia	Adults/Peds	NME**	11/18/2011	11/18/2011	ALL *ct
			3/14/2017		refractory classical cHL
			5/23/2017		Micosatellite instability-high (MSI-H) or mismatch repair deficient solid tumor
			6/13/2018		Adult and pediatric patients with refractory primarymediastinal largeB-cell lymphoma
Pembrolizumab	Adults/Peds	MAB***	9/4/2014	12/19/2018	MatasticMerkel cell carcinoma(≥ 12 years) *ct
			6/6/2020		Tumor mutational burden-high (TMB) solid tumors
			10/14/2020		Relapsed or refractory classical Hodgkin lymphoma (CHL)
Dinutuximab	*PRV	Pediatrics	NME**	3/10/2015	3/10/2015 High risk NB See *NB basic research note below *ct
Avelumab	Adults/Peds	MAB***	3/23/2017	3/23/2017	MatasticMerkel cell carcinoma(≥ 12 years)
Blinatumomab	Adults/Peds	MAB***	7/12/2017	7/12/2017	B-cell acute lymphoblastic leukemia
Tisagenleclucel	*PRV	Pediatrics	NME**	8/30/2017	8/30/2017 Relapsed or refractory ALL *ct
Nivolumab	12 yrs or older	MAB***	7/11/2018	7/11/2018	mismatch repair-deficient and microsatellite instability-high colorectal cancer
Iobenguane I 131	12 yrs or older		7/30/2018	7/30/2018	malignant pheochromocytoma paraganglioma
Calaspargase Pegol-mnk1mo -21 yrs.	Multi-Agent Component		12/20/2018	ALL	Used with combination chemotherapy *ct
Tagraxofusp-erzs	Adults/Peds	Targeted	12/21/2018	12/21/2018	Blastic plasmacytoid dendritic cell neoplasm
Larotrectinib	Adults/Peds	NME**	11/26/2018	11/26/2018	Solid tumor with (NTRK) gene fusion *ct
entrectinib	Adults/Peds	Targeted	8/15/2019	8/15/2019	12 years or older to treat solid tumors that have certain changes in a gene called NTRK
tazemetostat	Adults/Peds		1/23/2020	1/23/2020	epithelioid sarcoma 16 years and older whose cancer cannot be removed by surgery.
selumetinib sulfate*PRV	Pediatrics	Targeted	4/10/2020	4/10/2020	2 yrs and older who have plexiform neurofibromas
selpercatinib	Adults/Peds	Targeted	5/8/2020	5/8/2020	12 years or older to treat advanced or metastatic RET-mutant medullary thyroid cancer (MTC)
naxitamab	*PRV	Pediatrics	MAB***	11/25/2020	11/25/2020 1 yr & older with certain types of high-risk neuroblastoma
pralsetinib	Adults/Peds	Targeted	12/1/2020	12/1/2020	12 years or older to treat advanced or metastatic RET-mutant medullary thyroid cancer (MTC)
Gemtuzumab	Adults/Peds	MAB***	5/17/2000	6/16/2020	1 mo. & older Relapsed or refractory CD33+AML
crizotinib	Peds/AYA	Targeted	3/11/2016	1/14/2021	1 yr. & Young Adults ALK-positive systemic anaplastic large cell lymphoma

* Source: https://www.cancer.gov/research/areas/childhood/fda-approved-drugs-childhood-cancers?cid=eb_govdel

*ct = Data from NCI-sponsored clinical trials were used to support the approval

*PRV = Priority Review Voucher issued

** NME = New Molecular Entities

*** MAB= Monoclonal Antibody

****Exact pediatric-specific approval date is unknown.

 Possible late-onset cardiotoxicity <https://www.uspharmacist.com/article/chemotherapy-agents-that-cause-cardiotoxicity>

*NB Dinutuximab - NCI basic research 1960-2015 <https://www.cancer.gov/research/areas/childhood/childhood-cancer-basic-cancer-research>

Information Below Supplied by the FDA:

Supportive Care Oncology Drugs to treat pediatric patients with toxicity associated with cancer treatment

Drug	Approved for	Type	Original Approval	Pediatric Approval	Indication
Pegfilgrastim	Adults/Peds		1/31/2002	11/13/2015	Decrease incidence of infection,increases survival in patients acutely exposed to myelosuppressive doses of radiation
Rasburicase	Adults/Peds	NME*	7/12/2002	7/12/2002	Management of plasma uric acid levels in patients at risk for tumor lysis syndrome
Palifermin	Adults/Peds		12/15/2004		Decreased incidence and duration of severe oral mucositis
Levolucucovorin	Adults/Peds		3/7/2008	3/7/2008	Rescue after HD-MTX
Tocilizumab	Adults/Peds	MAB***	1/8/2010	8/30/2017	Treatment of chimeric antigen receptor (CAR) T cell-induced cytokine release syndrome
Voraxaze	Adults/Peds		1/17/2012	1/17/2012	Treatment of toxic plasma methotrexate concentration based on delayed MTX clearance