

FDA Pediatric Oncology Drug Approvals

Updated 12/31/2024

[A] Drugs Approved		[B] Indications	[C] Pediatric	
Generic Name	(Brand Name)		Approval Date	Notes
Mercaptopurine [Purinethol tablet]		Treatment of adult and pediatric patients with acute lymphoblastic leukemia (ALL) as part of a combination chemotherapy maintenance regimen	09/11/53	
Methotrexate		Treatment of adult and pediatric patients with acute lymphoblastic leukemia (ALL) as part of a combination chemotherapy regimen	12/07/53	
		Prophylaxis and treatment of meningeal leukemia in adult and pediatric patients	12/07/53	
		Treatment of adults and pediatric patients with Non-Hodgkin lymphoma	12/07/53	
		Treatment of adults and pediatric patients with osteosarcoma as part of a combination chemotherapy regimen	12/07/53	
Cyclophosphamide [Cytosan]		MULTIPLE	11/16/59	♥
Vincristine Sulfate		Acute leukemia; MULTIPLE (in combination)	7/10/63	
		Palliative treatment of malignancies including generalized Hodgkin's disease, histiocytosis X and testicular germ cell carcinoma	11/25/65	
Dactinomycin [Cosmegen]		Treatment of adult and pediatric patients with Wilms tumor, as part of a multi-phase, combination chemotherapy regimen	12/10/64	
		Treatment of adult and pediatric patients with rhabdomyosarcoma, as part of a multi-phase, combination chemotherapy regimen	12/10/64	
		Treatment of adult and pediatric patients with Ewing sarcoma, as part of a multi-phase, combination chemotherapy regimen	12/10/64	
		Treatment of adult and pediatric patients with metastatic, nonseminomatous testicular cancer, as part of a multi-phase, combination chemotherapy regimen	12/10/64	
Cytarabine		In combination with other approved anti-cancer drugs, for remission induction in acute non-lymphocytic leukemia of adults and pediatric patients	06/17/69	
Procarbazine Hydrochloride [Matulane]		In combination with other anticancer drugs for the treatment of Stage III and IV Hodgkin's disease	07/22/69	
Doxorubicin Hydrochloride		MULTIPLE	08/07/74	♥
Lomustine [Gleostine]		Treatment of patients with primary and metastatic brain tumors following appropriate surgical and/or radiotherapeutic procedures	08/04/76	
		As a component of combination chemotherapy for the treatment of patients with Hodgkin's lymphoma whose disease has progressed following initial chemotherapy	08/04/76	
Asparaginase [Elspar]		As a component of a multi-agent chemotherapeutic regimen for the treatment of patients with acute lymphoblastic leukemia (ALL)	01/10/78	
Daunorubicin Hydrochloride		In combination with other approved anticancer drugs, for remission induction in acute lymphocytic leukemia of children and adults	12/19/79	♥
Pegaspargase [Oncaspar]		As a component of a multi-agent chemotherapeutic regimen for the treatment of pediatric and adult patients with ALL and hypersensitivity to native forms of L-asparaginase	02/01/94	

	As a component of a multi-agent chemotherapeutic regimen for the first-line treatment of pediatric and adult patients with acute lymphoblastic leukemia (ALL)	07/24/06	
Tretinoin [Vesanoid]	Induction of remission in adults and pediatric patients 1 year of age and older with acute promyelocytic leukemia (APL) characterized by the presence of the t(15;17) translocation or PML/RAR α gene expression, and who are refractory to or who have relapsed from anthracycline chemotherapy or for whom anthracycline-based chemotherapy is contraindicated	11/22/95	#
Arsenic trioxide [Trisenox]	Induction of remission and consolidation in patients with acute promyelocytic leukemia (APL) who are refractory to, or have relapsed from, retinoid and anthracycline chemotherapy, and whose APL is characterized by the presence of the t(15;17) translocation or PML/RAR-alpha gene expression	09/25/00	
Busulfan [Busulfex]	For use in combination with cyclophosphamide as a conditioning regimen prior to allogeneic hematopoietic progenitor cell transplantation for chronic myelogenous leukemia	01/13/03	
Clofarabine [Clolar]	Treatment of pediatric patients 1 to 21 years old with relapsed or refractory acute lymphoblastic leukemia after at least two prior regimens	12/28/04	Peds 1st
Nelarabine [Arranon]	Treatment of T-cell acute lymphoblastic leukemia (T-ALL) and T-cell lymphoblastic lymphoma (T-LBL) in adult and pediatric patients age 1 year and older whose disease has not responded to or has relapsed following treatment with at least 2 chemotherapy regimens	10/28/05	
Imatinib Mesylate [Gleevec]	Newly diagnosed adult and pediatric patients with Philadelphia chromosome positive chronic myeloid leukemia (Ph+ CML) in chronic phase	09/27/06	
	Pediatric patients with newly diagnosed Philadelphia chromosome positive acute lymphoblastic leukemia (Ph+ ALL) in combination with chemotherapy	1/25/13	
Everolimus [Afinitor]	Adult and pediatric patients aged 1 year and older with tuberous sclerosis complex (TSC) for the treatment of subependymal giant cell astrocytoma (SEGA) that requires therapeutic intervention but cannot be curatively resected	10/29/10	
Asparaginase Erwinia Chrysanthemi [Erwinaze] [currently in shortage]	A component of a multi-agent chemotherapeutic regimen for the treatment of patients with acute lymphoblastic leukemia (ALL) who have developed hypersensitivity to E. coli-derived asparaginase	11/18/11	Combo
Denosumab [Xgeva]	Treatment of adults and skeletally mature adolescents with giant cell tumor of bone that is unresectable or where surgical resection is likely to result in severe morbidity	6/13/13	
Dinutuximab [Unituxin]	In combination with granulocyte-macrophage colony-stimulating factor (GM-CSF), interleukin-2 (IL-2), and 13-cis-retinoic acid (RA), for the treatment of pediatric patients with high-risk neuroblastoma who achieve at least a partial response to prior first-line multiagent, multimodality therapy	3/10/15	Peds 1st *PRV Combo
Thioguanine [Tabloid]	Remission induction and remission consolidation treatment of acute nonlymphocytic leukemias	12/21/16	
Avelumab [Bavencio]	Treatment of adults and pediatric patients 12 years and older with metastatic Merkel cell carcinoma (MCC)	3/23/17	
Pembrolizumab [Keytruda]	Treatment of adult and pediatric patients with unresectable or metastatic, microsatellite instability-high (MSI-H) or mismatch repair deficient (dMMR) solid tumors that have progressed following prior treatment and who have no satisfactory alternative treatment options	5/23/17	
	Treatment of adult and pediatric patients with refractory primary mediastinal large B-Cell lymphoma (PMBCL), or who have relapsed after 2 or more prior lines of therapy	6/13/18	

	Treatment of adult and pediatric patients with recurrent locally advanced or metastatic Merkel cell carcinoma (MCC)	12/19/18	
	Treatment of adult and pediatric patients with unresectable or metastatic tumor mutational burden-high (TMB-H) [≥10 mutations/megabase (mut/Mb)] solid tumors, as determined by an FDA-approved test, that have progressed following prior treatment and who have no satisfactory alternative treatment options	10/14/20	
	Treatment of pediatric patients with refractory classical Hodgkin lymphoma (cHL), or cHL that has relapsed after 2 or more lines of therapy	10/14/20	
	Adjuvant treatment of adult and pediatric (12 years and older) patients with Stage IIB, IIC, or III melanoma following complete resection	12/3/21	
Blinatumomab [Blincyto]	Treatment of relapsed or refractory CD19-positive B-cell precursor acute lymphoblastic leukemia (ALL) in adult and pediatric patients one month and older	7/11/17	
	Treatment of CD19-positive B-cell precursor acute lymphoblastic leukemia (ALL) in first or second complete remission with minimal residual disease (MRD) greater than or equal to 0.1% in adult and pediatric patients one month and older	3/29/18	
	Treatment of CD19-positive Philadelphia chromosome-negative B-cell precursor acute lymphoblastic leukemia (ALL) in the consolidation phase of multiphase chemotherapy in adult and pediatric patients one month and older	6/14/24	
Tisagenlecleucel [Kymriah]	Treatment of patients up to 25 years of age with B-cell precursor acute lymphoblastic leukemia (ALL) that is refractory or in second or later relapse	8/30/17	Peds 1st *PRV
Gemtuzumab ozogamicin [Mylotarg]	Treatment of relapsed or refractory CD33-positive acute myeloid leukemia in adults and pediatric patients 2 years and older.	9/1/17	
Dasatinib [Sprycel]	Treatment of pediatric patients 1 year of age and older with Philadelphia chromosome-positive (Ph+) chronic myeloid leukemia (CML) in chronic phase	11/9/17	
	Treatment of pediatric patients 1 year of age and older with newly diagnosed Philadelphia chromosome-positive acute lymphoblastic leukemia (Ph+ ALL) in combination with chemotherapy	12/21/18	
Nilotinib [Tasigna]	Treatment of adult and pediatric patients greater than or equal to 1 year of age with newly diagnosed Philadelphia chromosome positive chronic myeloid leukemia (Ph+ CML) in chronic phase	3/22/18	
	Treatment of pediatric patients greater than or equal to 1 year of age with chronic phase and accelerated phase Philadelphia chromosome positive chronic myeloid leukemia (Ph+ CML) with resistance or intolerance to prior tyrosine-kinase inhibitor (TKI) therapy	3/22/18	
Ipilimumab [Yervoy]	In combination with nivolumab, for the treatment of adult and pediatric patients 12 years and older with microsatellite instability-high (MSI-H) or mismatch repair deficient (dMMR) metastatic colorectal cancer (mCRC) that has progressed following treatment with a fluoropyrimidine, oxaliplatin, and irinotecan	7/10/18	
	As a single agent or in combination with nivolumab, for the treatment of unresectable or metastatic melanoma in adult and pediatric patients 12 years and older	2/15/23	Combo
Nivolumab [Opdivo]	As a single agent or in combination with ipilimumab, for the treatment of adult and pediatric patients 12 years and older with microsatellite instability-high (MSI-H) or mismatch repair deficient (dMMR) metastatic colorectal cancer (CRC) that has progressed following treatment with a fluoropyrimidine, oxaliplatin, and irinotecan	7/10/18	Combo
	As a single agent or in combination with ipilimumab, for the treatment of adult and pediatric patients 12 years and older with unresectable or metastatic melanoma	2/15/23	Combo

	Adjuvant treatment of adult and pediatric patients 12 years and older with completely resected Stage IIB, Stage IIC, Stage III, or Stage IV melanoma	10/13/23	
Iobenguane I-131 [Azedra]	Treatment of adult and pediatric patients 12 years and older with iobenguane scan positive, unresectable, locally advanced or metastatic pheochromocytoma or paraganglioma who require systemic anticancer therapy	7/30/18	
Emapalumab-lzsg [Gamifant]	Treatment of adult and pediatric (newborn and older) patients with primary hemophagocytic lymphohistiocytosis (HLH) with refractory, recurrent or progressive disease or intolerance with conventional HLH therapy	11/20/18	
Larotrectinib [Vitrakvi]	Treatment of adult and pediatric patients with solid tumors that have a neurotrophic receptor tyrosine kinase (NTRK) gene fusion without a known acquired resistance mutation, are metastatic or where surgical resection is likely to result in severe morbidity, and have no satisfactory alternative treatments or that have progressed following treatment	11/26/18	
Calaspargase pegol - mkn1 [Asparlas]	As a component of a multi-agent chemotherapeutic regimen for the treatment of acute lymphoblastic leukemia in pediatric and young adult patients age 1 month to 21 years	12/20/18	Peds 1st.
Tagraxofusp-erzs [Elzonris]	Treatment of blastic plasmacytoid dendritic cell neoplasm (BPDCN) in adults and in pediatric patients 2 years and older	12/21/18	
Tazemetostat [Tazverik]	Treatment of adults and pediatric patients aged 16 years and older with metastatic or locally advanced epithelioid sarcoma not eligible for complete resection	1/23/20	
Selumetinib [Koselugo]	Treatment of pediatric patients 2 years of age and older with neurofibromatosis type 1 (NF1) who have symptomatic, inoperable plexiform neurofibromas (PN)	4/10/20	Peds 1st *PRV
Gemtuzumab ozogamicin [Mylotarg]	Treatment of newly-diagnosed CD33-positive acute myeloid leukemia in adults and pediatric patients 1 month and older	6/16/20	
Naxitamab-gqgk [Danyelza]	In combination with granulocyte-macrophage colony-stimulating factor (GM-CSF), for the treatment of pediatric patients 1 year of age and older and adult patients with relapsed or refractory high-risk neuroblastoma in the bone or bone marrow who have demonstrated a partial response, minor response, or stable disease to prior therapy	11/25/20	Peds 1st *PRV Combo
Pralsetinib [Gavreto]	Treatment of adult and pediatric patients 12 years of age and older with advanced or metastatic RET-mutant medullary thyroid cancer (MTC) who require systemic therapy	12/1/20	
Pralsetinib [Gavreto]	Treatment of adult and pediatric patients 12 years of age and older with advanced or metastatic RET fusion-positive thyroid cancer who require systemic therapy and who are radioactive iodine-refractory (if radioactive iodine is appropriate)	12/1/20	
Crizotinib [Xalkori]	Treatment of pediatric patients 1 year of age and older and young adults with relapsed or refractory, systemic anaplastic large cell lymphoma (ALCL) that is ALK-positive	1/14/21	
	Treatment of adult and pediatric patients 1 year of age and older with unresectable, recurrent, or refractory inflammatory myofibroblastic tumor (IMT) that is ALK-positive	7/14/22	
Daunorubicin and cytarabine liposome [Vyxeos]	Treatment of newly-diagnosed therapy-related acute myeloid leukemia (t-AML) or AML with myelodysplasia-related changes (AML-MRC) in adults and pediatric patients 1 year and older	3/30/21	❤️
Asparaginase Erwinia chrysanthemi (recombinant)-rywn [Rylaze]	As a component of a multi-agent chemotherapeutic regimen for the treatment of acute lymphoblastic leukemia (ALL) and lymphoblastic lymphoma (LBL) in adult and pediatric patients 1 month or older who have developed hypersensitivity to E. coli-derived asparaginase	6/30/21	

Cabozantinib [Cabometyx]	Treatment of adult and pediatric patients 12 years of age and older with locally advanced or metastatic differentiated thyroid cancer (DTC) that has progressed following prior VEGFR-targeted therapy and who are radioactive iodine-refractory or ineligible	9/17/21	
Rituximab [Rituxan]	Treatment of pediatric patients aged 6 months and older with previously untreated, advanced stage, CD20-positive diffuse large B-cell lymphoma (DLBCL), Burkitt lymphoma (BL), Burkitt-like lymphoma (BLL) or mature B-cell acute leukemia (B-AL) in combination with chemotherapy	12/2/21	
Nivolumab and Relatlimab-rmbw) [Opdualag]	Treatment of adult and pediatric patients 12 years of age or older with unresectable or metastatic melanoma	3/18/22	
Azacitidine [Vidaza]	Treatment of pediatric patients aged 1 month and older with newly diagnosed Juvenile Myelomonocytic Leukemia (JMML)	5/20/22	
Brentuximab vedotin [Adcetris]	Treatment of pediatric patients 2 years and older with previously untreated high risk classical Hodgkin lymphoma (cHL), in combination with doxorubicin, vincristine, etoposide, prednisone, and cyclophosphamide	11/10/22	
Atezolizumab [Tecentriq]	As a single agent, for the treatment of adult and pediatric patients 2 years of age and older with unresectable or metastatic alveolar soft part sarcoma (ASPS)	12/9/22	
Dabrafenib [Tafinlar]	In combination with trametinib, for the treatment of pediatric patients 1 year of age and older with low-grade glioma (LGG) with a BRAF V600E mutation who require systemic therapy	3/16/23	
	In combination with trametinib, for the treatment of adult and pediatric patients 1 year of age and older with unresectable or metastatic solid tumors with BRAF V600E mutation who have progressed following prior treatment and have no satisfactory alternative treatment options	8/31/23	
Trametinib [Mekinist]	In combination with dabrafenib, for the treatment of pediatric patients 1 year of age and older with low-grade glioma (LGG) with a BRAF V600E mutation who require systemic therapy	3/16/23	
	In combination with dabrafenib, for the treatment of adult and pediatric patients 1 year of age and older with unresectable or metastatic solid tumors with BRAF V600E mutation who have progressed following prior treatment and have no satisfactory alternative treatment options	8/31/23	
Bosutinib [Bosulif]	Treatment of adult and pediatric patients 1 year of age and older with chronic phase (CP) Philadelphia chromosome-positive chronic myelogenous leukemia (Ph+ CML), newly-diagnosed or resistant or intolerant to prior therapy	9/26/23	
Entrectinib [Rozlytrek]	Treatment of adult and pediatric patients older than 1 month with solid tumors that have a neurotrophic tyrosine receptor kinase (NTRK) gene fusion without a known acquired resistance mutation, are metastatic or where surgical resection is likely to result in severe morbidity, and have progressed following treatment or have no satisfactory standard therapy	10/20/23	
Eflornithine [Iwiflin]	To reduce the risk of relapse in adult and pediatric patients with high-risk neuroblastoma (HRNB) who have demonstrated at least a partial response to prior multiagent, multimodality therapy including anti-GD2 immunotherapy	12/13/23	Peds 1st
Inotuzumab ozogamicin [Besponsa]	Treatment of relapsed or refractory CD22-positive B-cell precursor acute lymphoblastic leukemia (ALL) in adult and pediatric patients 1 year and older	3/6/24	
Lutetium Lu 177 dotatate [Lutathera]	Treatment of adult and pediatric patients 12 years and older with somatostatin receptor-positive gastroenteropancreatic neuroendocrine tumors (GEP-NETs), including foregut, midgut, and hindgut neuroendocrine tumors	4/23/24	
Tovorafenib [Ojemda]	Treatment of patients 6 months of age and older with relapsed or refractory pediatric low-grade glioma (LGG) harboring a BRAF fusion or rearrangement, or BRAF V600 mutation	4/23/24	

Selpercatinib [Retevmo]	Treatment of adult and pediatric patients 2 years of age and older with locally advanced or metastatic solid tumors with a RET gene fusion, as detected by an FDA-approved test, that have progressed on or following prior systemic treatment or who have no satisfactory alternative treatment options	5/29/24
	Treatment of adult and pediatric patients 2 years of age and older with advanced or metastatic thyroid cancer with a RET gene fusion, as detected by an FDA-approved test, who require systemic therapy and who are radioactive iodine-refractory (if radioactive iodine is appropriate)	5/29/24
	Treatment of adult and pediatric patients 2 years of age and older with advanced or metastatic medullary thyroid cancer (MTC) with a RET mutation, as detected by an FDA-approved test, who require systemic therapy	5/29/24
Repotrectinib [Augtyro]	Treatment of adult and pediatric patients 12 years of age and older with solid tumors that have a neurotrophic tyrosine receptor kinase (NTRK) gene fusion, are locally advanced or metastatic or where surgical resection is likely to result in severe morbidity, and have progressed following treatment or have no satisfactory alternative therapy	6/13/24
Vorasidenib [Voranigo]	Treatment of adult and pediatric patients 12 years and older with Grade 2 astrocytoma or oligodendroglioma with a susceptible isocitrate dehydrogenase-1 (IDH1) or isocitrate dehydrogenase-2 (IDH2) mutation following surgery including biopsy, sub-total resection, or gross total resection	8/6/24
Revumenib [Revuforj]	Treatment of relapsed or refractory acute leukemia with a lysine methyltransferase 2A gene (KMT2A) translocation in adult and pediatric patients 1 year and older	11/15/24

Notes

Column A [DRUGS APPROVED FOR PEDIATRIC CANCERS [BRAND NAME]: Reference products may have been discontinued. Links to generic products were selected based on extent of information and do not represent any specific product preference.

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

* The listed pediatric approval date reflects the most recent approval for the pediatric cancer(s) listed in the indication.

Peds 1st. Drugs approved for pediatrics, then later approved for adults


Column B: [INDICATION]: This table reflects current data as of November 2024. To view additional information, please check the Drugs@FDA listing.

02-21-2023: label updated to Physician Labeling Rule (PLR) format; indication statement updated to specify “adults and pediatric patients 1 year of age and older” and remove the FAB classification

Column C: [PEDIATRIC APPROVAL DATE]: Missing dates: Not available in digital records.

Combo: drug used in combination with another

***PRV** = Awarded Priority Review Voucher



Source:: <https://www.fda.gov/about-fda/oncology-center-excellence/pediatric-oncology-drug-approvals>