

# Developmental Therapies Protocol List



Check with the lead coordinator for up-to-date information about study enrollment and stratum availability.

CNS developmental therapy treatment protocols					
Brain Tumors	Study	Clinical trial name	Phase	Age	Local PI
	<a href="#">AflacST1901 – Peds WP1066</a>	AflacST1901: A Phase I Study of WP1066 in Children with Refractory and Progressive or Recurrent Malignant Brain Tumors	I	≥3 to 25 yr	Tobey MacDonald, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#">UCSD Cabozantinib cisRA</a>	A National Phase I Study of Cabozantinib in Combination with 13-cis-Retinoic Acid in Children with Relapsed or Refractory Solid Tumors	I	≥2 to ≤26 yr	Dolly Aguilera, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#">PBTC-045</a>	PBTC-045: A Safety and Preliminary Efficacy trial of MK-3475 (pembrolizumab; anti-PD-1) in Children with Recurrent, Progressive or Refractory Diffuse Intrinsic Pontine Glioma (DIPG), Non-brainstem High-grade Gliomas (NB-HGG), Ependymoma, Medullo-blastoma and Hypermutated Brain Tumors	I	≥1 to ≤18 yr	Jason Fangusaro, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#">PBTC-048</a>	PBTC-048: Feasibility Trial of Optune for Children with Recurrent or Progressive Supratentorial High-grade Glioma and Ependymoma	I	≥5 to ≤21 yr	Jason Fangusaro, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>

	<a href="#"><u>PBTC-051</u></a>	Phase I Study to Evaluate the Safety and Tolerability of the CD40 Agonistic Monoclonal Antibody APX005M in Pediatric Subjects with Recurrent/ Refractory Brain Tumors and Newly Diagnosed Brain Stem Glioma	I	≥1 to ≤21 yr	Jason Fangusaro, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>PBTC-053</u></a>	A Pediatric Brain Tumor Consortium Phase I/II and Surgical Study of CX-4945 in Patients with Recurrent SHH Medulloblastoma	I/II	≥3 to ≤18 yr	Jason Fangusaro, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>PBTC-055</u></a>	PBTC-055: Phase I/II Trial of Dabrafenib, Trametinib, and Hydroxychloroquine (HCQ) for BRAF V600E-mutant or Trametinib and HCQ for BRAF Fusion/Duplication Positive or NF1-associated Recurrent or Refractory Gliomas in Children and Young Adults	I/II	≥1 to ≤30 yr	Jason Fangusaro, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>PBTC-056</u></a>	Phase 1 trial of INCB7839 for children with recurrent or progressive high-grade gliomas, including, but not limited to, diffuse intrinsic pontine glioma (DIPG) and other diffuse midline gliomas (DMGs), after upfront therapy.	I	<u>3 Years to 21 Years</u>	Jason Fangusaro, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>ONC014</u></a>	ONC201 in Newly Diagnosed Diffuse Intrinsic Pontine Glioma and Recurrent Pediatric H3 K27M Gliomas	I	≥2 to <19 yr	Tobey MacDonald, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>COG APEC1621D<sup>REQ</sup></u></a>	APEC1621D NCI-COG Pediatric MATCH (Molecular Analysis for Therapy Choice)—Phase 2 Subprotocol of LY3023414 in Patients with Solid Tumors	II	≥12 mo to ≤21 yr	Himalee Sabnis, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>COG APEC1621K<sup>REQ</sup></u></a>	APEC1621K NCI-COG Pediatric MATCH (Molecular Analysis for Therapy Choice)—Phase 2 Subprotocol of AG-120 (Ivosidenib) in Patients with Tumors Harboring IDH1 Mutations	II	≥12 mo to ≤21 yr	Tobey MacDonald, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>

	<a href="#"><u>COG ADVL1823</u></a>	ADVL1823, Larotrectinib (LOXO-101, NSC# 788607, IND# 141824) for Previously Untreated TRK Fusion Pediatric Solid Tumors and TRK Fusion Relapsed Pediatric Acute Leukemias	II	≤30 yr	Jason Fangusaro, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>Aflac ST1502 CHOANOME II</u></a>	AflacST1502: A Phase II Study of Sirolimus in Combination with Metronomic Chemotherapy in Children with Recurrent and/or Refractory Solid and CNS Tumors	II	>12 mo to ≤30 yr	Thomas Cash, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>SJATRT</u></a>	Phase 2 Study of Alisertib as a Single Agent in Recurrent or Progressive Central Nervous System (CNS) Atypical Teratoid Rhabdoid Tumors (AT/RT) and Extra-CNS Malignant Rhabdoid Tumors (MRT) and in Combination Therapy in Newly Diagnosed AT/RT (SJATRT)	II	<22 yr	Dolly Aguilera, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>

### Leukemia developmental therapy treatment protocols

Disease type	Study	Clinical trial name	Phase	Age	Local PI
AML	<a href="#"><u>PEPN1812</u></a>	PEPN1812: A Phase 1 Trial of the CD123 X CD3 Dual Affinity Re-targeting Antibody Flotetuzumab (NSC#808294, IND#145986) in Children, Adolescents, and Young Adults with Relapsed or Refractory Acute Myeloid Leukemia	I	<21 yr	Jason Fangusaro, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
ALL	<a href="#"><u>AflacLL1602 ENCERT</u></a>	ENCERT: A Phase 1 Trial using Everolimus in combination with Nelarabine, Cyclophosphamide and Etoposide in Relapsed T cell Lymphoblastic Leukemia/ Lymphoma	I	>1 to <30 yr	Himalee Sabnis, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
ALL	<a href="#"><u>TACL 2017-002</u></a>	A TACL Phase 1/2 Study of PO Ixazomib in Combination with Chemotherapy for Childhood Relapsed or Refractory Acute Lymphoblastic Leukemia and Lymphoblastic Lymphoma	I/II	≤21 yr	Himalee Sabnis, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
ALL	<a href="#"><u>20140106 (formerly ONYX CFZ008)</u></a>	Phase 1b Study of Carfilzomib in Combination with Induction Chemotherapy in Children with Relapsed or Refractory Acute Lymphoblastic Leukemia	Ib/II	≤18 yr	Himalee Sabnis, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
AML	<a href="#"><u>AC220-A-U202 (ADVL1822)</u></a>	A Phase 1/2, Quizartinib Administered in Combination with Re-induction Chemotherapy, and as a Single-agent Continuation Therapy, in Pediatric Relapsed/Refractory AML Subjects with FLT3-ITD Mutations	I/II	≥1 mo to <21 yr	Himalee Sabnis, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>

AML	<a href="#">TACL 2016-002</a>	TACL2016-002: A TACL Phase 1/2 Study of Nivolumab in Combination with 5-azacytidine in pediatric patients with relapsed/refractory acute myeloid leukemia (BMS reference CA209-9JY)	I/II	>1 to ≤30 yr	Himalee Sabnis, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
ALL	<a href="#">COG AALL1521 [Incyte]</a>	INCB 18424-269: A Phase 2 Study of JAK1/JAK2 Inhibitor Ruxolitinib with Chemotherapy in Children with <i>De Novo</i> High-Risk CRLF2-rearranged and/or JAK Pathway-mutant Acute Lymphoblastic Leukemia	II	>1 to ≤21 yr	Melinda Pauly, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
JMML	<a href="#">COG ADVL1521</a>	ADVL1521: A Phase 2 Study of the MEK inhibitor Trametinib (IND #119346, NSC# 763093) in Children with Relapsed or Refractory Juvenile Myelomonocytic Leukemia	II	≥2 to <22 yr	Himalee Sabnis, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
ALL/AML	<a href="#">COG ADVL1823</a>	ADVL1823, Larotrectinib (LOXO-101, NSC# 788607, IND# 141824) for Previously Untreated TRK Fusion Pediatric Solid Tumors and TRK Fusion Relapsed Pediatric Acute Leukemias	II	≤30 yr	Jason Fangusaro, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
ALL/CML	<a href="#">DFCI 18-328</a>	Phase I Trial of Ribociclib in Combination with Everolimus and Dexamethasone in Children and Young Adults with Relapsed Acute Lymphoblastic Leukemia	I	>12 mo to ≤30 yr	Himalee Sabnis, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>

**Lymphoma developmental therapy treatment protocols**

Disease type	Study	Clinical trial name	Phase	Age	Local PI
Lymphoma	<a href="#">AflacLL1602 ENCERT</a>	ENCERT: A Phase 1 Trial using Everolimus in combination with Nelarabine, Cyclophosphamide and Etoposide in Relapsed T cell Lymphoblastic Leukemia/ Lymphoma	I	>1 to <30 yr	Himalee Sabnis, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
Lymphoma	<a href="#">COG ADVL1721</a>	A Non-randomized, Open-label, Multi-center, Phase I/II Study of PI3K Inhibitor Copanlisib in Pediatric Patients with Relapsed/Refractory Solid Tumors or Lymphoma	I/II	≥6 mo to ≤21 yr	Thomas Cash, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
NHL	<a href="#">COG APEC1621D<sup>REQ</sup></a>	APEC1621D NCI-COG Pediatric MATCH (Molecular Analysis for Therapy Choice) – Phase 2 Subprotocol of LY3023414 in Patients with Solid Tumors	II	≥12 mo to ≤21 yr	Himalee Sabnis, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>

NHL	<a href="#">COG APEC1621K<sup>REQ</sup></a>	APEC1621K NCI-COG Pediatric MATCH (Molecular Analysis for Therapy Choice)—Phase 2 Subprotocol of AG-120 (Ivosidenib) in Patients with Tumors Harboring IDH1 Mutations	II	≥12 mo to ≤21 yr	Tobey MacDonald, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
NHL	<a href="#">AbbVie M13-833</a>	A Phase 1 Study of the Safety and Pharmacokinetics of Venetoclax in Pediatric and Young Adult Patients with Relapsed or Refractory Malignancies	I	<25 yr	Kelly Goldsmith, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
ALL	<a href="#">TACL 2017-002</a>	A TACL Phase 1/2 Study of PO Ixazomib in Combination with Chemotherapy for Childhood Relapsed or Refractory Acute Lymphoblastic Leukemia and Lymphoblastic Lymphoma	I/II	≤21 yr	Himalee Sabnis, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>

**Neuroblastoma developmental therapy treatment protocols**

	Study	Clinical trial name	Phase	Age	Local PI
	<a href="#">AflacST1603 GemAbrax</a>	AflacST1603: A Phase 1 Study Using Nab-paclitaxel (Abraxane®) in Combination with Gemcitabine for Pediatric Relapsed and Refractory Solid Tumors	I	≥6 mo to ≤30 yr	Thomas Cash, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#">AbbVie M13-833</a>	A Phase 1 Study of the Safety and Pharmacokinetics of Venetoclax in Pediatric and Young Adult Patients with Relapsed or Refractory Malignancies	I	<25 yr	Kelly Goldsmith, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#">UCSD Cabozantinib cisRA</a>	A National Phase I Study of Cabozantinib in Combination with 13-cis-Retinoic Acid in Children with Relapsed or Refractory Solid Tumors	I	≥2 to ≤26 yr	Dolly Aguilera, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#">COG ADVL1721</a>	A Non-randomized, Open-label, Multi-center, Phase I/II Study of PI3K Inhibitor Copanlisib in Pediatric Patients with Relapsed/Refractory Solid Tumors or Lymphoma	I/II	≥ 6 mo to ≤21 yr	Thomas Cash, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#">COG APEC1621D<sup>REQ</sup></a>	APEC1621D NCI-COG Pediatric MATCH (Molecular Analysis for Therapy Choice) – Phase 2 Subprotocol of LY3023414 in Patients with Solid Tumors	II	≥12 mo to ≤21 yr	Himalee Sabnis, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>

	<a href="#"><u>COG APEC1621K<sup>REQ</sup></u></a>	APEC1621K NCI-COG Pediatric MATCH (Molecular Analysis for Therapy Choice)—Phase 2 Subprotocol of AG-120 (Ivosidenib) in Patients with Tumors Harboring IDH1 Mutations	II	≥12 mo to ≤21 yr	Tobey MacDonald, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>Aflac ST1502 CHOANOME II</u></a>	AflacST1502: A Phase II Study of Sirolimus in Combination with Metronomic Chemotherapy in Children with Recurrent and/or Refractory Solid and CNS Tumors	II	>12 mo to ≤30 yr	Thomas Cash, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>COG ADVL1823</u></a>	ADVL1823, Larotrectinib (LOXO-101, NSC# 788607, IND# 141824) for Previously Untreated TRK Fusion Pediatric Solid Tumors and TRK Fusion Relapsed Pediatric Acute Leukemias	II	≤30 yr	Jason Fangusaro, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>NANT 2015-02<sup>REQ</sup></u></a>	NANT 2015-02: Phase 1 Study of Lorlatinib (PF-06463922), an Oral Small Molecule Inhibitor of ALK/ROS1, for Patients with ALK-driven Relapsed or Refractory Neuroblastoma	I	≥12 mo	Kelly Goldsmith, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>NANT 2017-01<sup>REQ</sup></u></a>	NANT 2017-01: A Phase I Study of <sup>131</sup> I-MIBG with Dinutuximab for Relapsed/Refractory Neuroblastoma (IND# 137554)	I	≥1 to <30 yr	Kelly Goldsmith, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>MIBG Access</u></a>	An Open Label, Expanded Access Protocol Using <sup>131</sup> I-Metaiodobenzylguanidine ( <sup>131</sup> I-MIBG) Therapy in Patients with Refractory Neuroblastoma, Pheochromocytoma, or Paraganglioma	Access to MIBG therapy	≥12 mo	Kelly Goldsmith, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>

**Solid tumor developmental therapy treatment protocols**

Disease type	Study	Clinical trial name	Phase	Age	Local PI
	<a href="#"><u>AflacST1603 GemAbrax</u></a>	AflacST1603: A Phase 1 Study Using Nab-paclitaxel (Abraxane®) in Combination with Gemcitabine for Pediatric Relapsed and Refractory Solid Tumors	I	≥6 mo to ≤30 yr	Thomas Cash, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>

<a href="#"><u>AbbVie M13-833</u></a>	A Phase 1 Study of the Safety and Pharmacokinetics of Venetoclax in Pediatric and Young Adult Patients with Relapsed or Refractory Malignancies	I	<25 yr	Kelly Goldsmith, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
<a href="#"><u>Campfire</u></a>	A Phase 1 study of the safety and efficacy of ramucirumab in combination with other chemotherapy in the treatment of relapsed, recurrent, or refractory desmoplastic small round cell tumor (DSRCT) in children and young adults.	I	12 Months to 29 Years	Thomas Cash, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
<a href="#"><u>UCSD Cabozantinib cisRA</u></a>	A National Phase I Study of Cabozantinib in Combination with 13-cis-Retinoic Acid in Children with Relapsed or Refractory Solid Tumors	I	≥2 to ≤26 yr	Dolly Aguilera, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
<a href="#"><u>Colorado 18-2740</u></a>	A Phase I/Ib Study of Losartan in Combination with Sunitinib in the Treatment of Pediatric and Adult Patients with Relapsed or Refractory Osteosarcoma	I/Ib	10 to 40 yr	Thomas Cash, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
<a href="#"><u>COG ADV1721</u></a>	A Non-randomized, Open-label, Multi-center, Phase I/II Study of PI3K Inhibitor Copanlisib in Pediatric Patients with Relapsed/Refractory Solid Tumors or Lymphoma	I/II	≥ 6 mo to ≤21 yr	Thomas Cash, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
<a href="#"><u>COG ADV1 1921 Pfizer A5481092</u></a>	A study to learn about safety and find out maximum tolerable dose of palbociclib given in combination with chemotherapy in children, adolescents and young adults with recurrent or refractory solid tumors	I/II	2 Years to 20 Years	Thomas Cash, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
<a href="#"><u>COG APEC1621D<sup>REQ</sup></u></a>	APEC1621D NCI-COG Pediatric MATCH (Molecular Analysis for Therapy Choice) – Phase 2 Subprotocol of LY3023414 in Patients with Solid Tumors	II	≥12 mo to ≤21 yr	Himalee Sabnis, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
<a href="#"><u>COG APEC1621K<sup>REQ</sup></u></a>	APEC1621K NCI-COG Pediatric MATCH (Molecular Analysis for Therapy Choice)—Phase 2 Subprotocol of AG-120 (Ivosidenib) in Patients with Tumors Harboring IDH1 Mutations	II	≥12 mo to ≤21 yr	Tobey MacDonald, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>

	<a href="#"><u>Aflac ST1502 CHOANOME II</u></a>	AflacST1502: A Phase II Study of Sirolimus in Combination with Metronomic Chemotherapy in Children with Recurrent and/or Refractory Solid and CNS Tumors	II	>12 mo to ≤30 yr	Thomas Cash, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>COG ADVL1823</u></a>	ADVL1823, Larotrectinib (LOXO-101, NSC# 788607, IND# 141824) for Previously Untreated TRK Fusion Pediatric Solid Tumors and TRK Fusion Relapsed Pediatric Acute Leukemias	II	≤30 yr	Jason Fangusaro, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>COG ARST1921</u></a>	ARST1921, A Safety, Pharmacokinetic and Efficacy Study of a γ-Secretase Inhibitor, Nirogacestat (PF-03084014; IND# 146375), in Children and Adolescents with Progressive, Surgically Unresectable Desmoid Tumors	II	>12 mo to <18 yr	Thomas Cash, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>PEPN1924</u></a>	Trastuzumab Deruxtecan for the Treatment of HER2+ Newly Diagnosed or Recurrent Osteosarcoma	II	12 Years to 39 Years	Thomas Cash, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>
	<a href="#"><u>SJATRT</u></a>	Phase 2 Study of Alisertib as a Single Agent in Recurrent or Progressive Central Nervous System (CNS) Atypical Teratoid Rhabdoid Tumors (AT/RT) and Extra-CNS Malignant Rhabdoid Tumors (MRT) and in Combination Therapy in Newly Diagnosed AT/RT (SJATRT)	II	<22 yr	Dolly Aguilera, MD <a href="mailto:AflacDevTReferral@choa.org">AflacDevTReferral@choa.org</a>