

Development Therapeutics

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Rare Tumors	
ADVL1622	<p>Phase 2 Trial of XL184 (Cabozantinib) an Oral Small-Molecule Inhibitor of Multiple Kinases, in Children and Young Adults with Refractory Sarcomas, Wilms Tumor, and Other Rare Tumors</p> <ul style="list-style-type: none"> • AYA age 2-30 (except MTC, RCC, HCC upper age 18)
	<p>Relapsed/refractory or newly diagnosed with no known curative therapy:</p> <ul style="list-style-type: none"> o Medullary thyroid carcinoma (MTC) o Renal cell carcinoma (RCC) o Hepatocellular carcinoma (HCC) o Hepatoblastoma o Adrenocortical carcinoma o Pediatric solid tumors (including CNS tumors) with known molecular alterations in the targets of XL184 (i.e., MET amplification, overexpression, activating mutation, MET translocation, MET exon skipping mutations, activating RET mutations, RET rearrangement, overexpression or activation of AXL).
Relapsed/refractory Juvenile Myelomonocytic Leukemia	
ADVL1521	A Phase 2 Study of the MEK inhibitor Trametinib (IND#119346, NSC#763093) in Children with Relapsed or Refractory Juvenile Myelomonocytic Leukemia (ages ≥ 2 years to < 22)

Upfront Therapy Neuro-oncology	
Head Start 4	Newly Diagnosed Children (< 10 Years Old) With Medulloblastoma And Other Central Nervous System Embryonal Tumors . Clinical And Molecular Risk-Tailored Intensive And Compressed Induction Chemotherapy Followed By Consolidation With Randomization To Either Single-Cycle Or To Three Tandem Cycles Of Marrow-Ablative Chemotherapy With Autologous Hematopoietic Progenitor Cell Rescue (less than 10 years of age)
ADVL1217	A Phase 1 Study of AZD1775 (MK-1775, IND#116459) Concurrent with Local Radiation Therapy for the Treatment of Newly Diagnosed Children with Diffuse Intrinsic Pontine Gliomas ($>$ than 36 months to ≤ 21 years)
Relapsed/Refractory Neuro-oncology	
UAB1472	Phase I Clinical Trial of HSV G207 Alone or with a Single Radiation Dose in Children with Recurrent Supratentorial Brain Tumors (≥ 36 months to < 19 years)
ADVL1414	A Phase 1 Study of Selinexor (KPT-330, IND #125052), a Selective XPO1 Inhibitor, in Recurrent and Refractory Pediatric Solid Tumors, including CNS Tumors (\geq than 12 months to ≤ 21 years)

ADV1416	A Phase 1 study of Ramucirumab , a Human Monoclonal Antibody Against the Vascular Endothelial Growth Factor-2 (VEGFR-2) Receptor in Children with Refractory Solid tumors, including CNS Tumors (≥ 12 months to ≤ 21 years)
ADV1513	A Phase 1 Study of entinostat , an oral histone deacetylase inhibitor, in Pediatric Patients with Recurrent or Refractory Solid Tumors, including CNS Tumors and Lymphoma (\geq than 12 months to ≤ 21 years)
ADV1514	A Phase 1 Study of ABI-009 (nab-Rapamycin) in Pediatric Patients with Recurrent or Refractory Solid Tumors, Including CNS Tumors as a Single Agent and in Combination with Temozolomide and Irinotecan (≥ 12 months to ≤ 21 years)
ADV1515	A Phase 1 Study of LY2606368, a CHK1/2 inhibitor, in Pediatric Patients with Recurrent or Refractory Solid Tumors, including CNS Tumors (\geq than 12 months to ≤ 21 years)
ADV1615	A Phase 1 Study of Pevonedistat (MLN4924, IND# 136078), a NEDD8 Activating Enzyme (NAE) Inhibitor, in Combination with Temozolomide and Irinotecan in Pediatric Patients with Recurrent or Refractory Solid Tumors (≥ 6 months to < 21 years)
ADV1711	A Phase 1/2 Study of Lenvatinib in Combination With Everolimus in Recurrent and Refractory Pediatric Solid Tumors, Including CNS Tumors (≥ 2 years to < 18 years for phase 1; ≥ 2 years to ≤ 21 years for phase 2)
MEK162	Phase I-II Study of MEK162 for Children with Low Grade Gliomas and Other Ras/Raf/ERK Pathway Activated Tumors (> 1 year to < 18 years)

Relapsed/Refractory Solid tumors

ADV1412	A Phase 1/2 Study of Nivolumab (IND#124729) in Children, Adolescents, and Young Adults with Recurrent or Refractory Solid Tumors as a Single Agent and in Combination with Ipilimumab (≥ 12 months to ≤ 30 years depending on strata)
ADV1414	A Phase 1 Study of Selinexor (KPT-330, IND #125052), a Selective XPO1 Inhibitor, in Recurrent and Refractory Pediatric Solid Tumors, including CNS Tumors (\geq than 12 months to ≤ 21 years)
ADV1416	A Phase 1 study of Ramucirumab , a Human Monoclonal Antibody Against the Vascular Endothelial Growth Factor-2 (VEGFR-2) Receptor in Children with Refractory Solid tumors, including CNS Tumors (≥ 12 months to ≤ 21 years)
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ADV1515	A Phase 1 Study of LY2606368, a CHK1/2 inhibitor, in Pediatric Patients with Recurrent or Refractory Solid Tumors, including CNS Tumors (\geq than 12 months to ≤ 21 years)
ADV1614	A Phase 1/2 Study of VX15/2503 (IND# 136181) in Children, Adolescents, or Young Adults with Recurrent or Relapsed Solid Tumors (≥ 12 months to ≤ 30 years depending on strata)
ADV1615	A Phase 1 Study of Pevonedistat (MLN4924, IND# 136078), a NEDD8 Activating Enzyme (NAE) Inhibitor, in Combination with Temozolomide and Irinotecan in Pediatric Patients with Recurrent or Refractory Solid Tumors (≥ 6 months to < 21 years)
ADV1622	Phase 2 Trial of XL184 (Cabozantinib) an Oral Small-Molecule Inhibitor of Multiple Kinases, in Children and Young Adults with Refractory Sarcomas, Wilms Tumor, and Other Rare Tumors (≥ 2 to ≤ 30 years depending on strata)

ADVL1711	A Phase 1/2 Study of Lenvatinib in Combination With Everolimus in Recurrent and Refractory Pediatric Solid Tumors, Including CNS Tumors (≥ 2 years to < 18 years for phase 1; ≥ 2 years to ≤ 21 years for phase 2)
ADVL1722	A Phase 2, multicenter, open-label study to assess safety and preliminary activity of eribulin mesylate in pediatric subjects with relapsed/refractory rhabdomyosarcoma (RMS), non-rhabdomyosarcoma soft tissue sarcoma (NRSTS) and Ewing sarcoma (EWS) (≥ 12 months to < 18 years)
ADVL1721	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 (6 months to ≤ 21 years)

Neuroblastoma	
NMTRC012	A Study Using Molecular Guided Therapy with Induction Chemotherapy followed by a Randomized Controlled Trial of standard immunotherapy with or without DFMO followed by DFMO maintenance for Subjects with Newly Diagnosed High-Risk Neuroblastoma (≤ 21 years at initial diagnosis)
NMTRC014	Neuroblastoma Maintenance Therapy Trial Using Difluoromethylornithine (DFMO) (≤ 30.99 years of age) <ul style="list-style-type: none"> • Stratum 1: Subjects who are in remission at the completion of standard upfront COG induction, consolidation and maintenance therapy. • Stratum 2: Neuroblastoma that is in first complete remission following standard upfront therapy different from that described for Stratum 1. • Stratum 3: Subjects who are in first remission after receiving any additional therapy for neuroblastoma that was refractory to standard induction/consolidation therapy. • Stratum 4: Subjects who have achieved a second or subsequent CR following relapse(s).
ADVL1312	A Phase 1/2 Study of AZD1775 (MK-1775, IND# 121422) in Combination with Oral Irinotecan in Children, Adolescents, and Young Adults with Relapsed or Refractory Solid Tumors ($>$ than 12 months to ≤ 21 years)

Lymphoma	
ADVL1513	A Phase 1 Study of entinostat , an oral histone deacetylase inhibitor, in Pediatric Patients with Recurrent or Refractory Solid Tumors, including CNS Tumors and Lymphoma (\geq than 12 months to ≤ 21 years)
ADVL1615	A Phase 1 Study of Pevonedistat (MLN4924, IND# 136078), a NEDD8 Activating Enzyme (NAE) Inhibitor, in Combination with Temozolomide and Irinotecan in Pediatric Patients with Recurrent or Refractory Solid Tumors (≥ 6 months to < 21 years)
ADVL1721	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 (6 months to ≤ 21 years)

Molecularly Targeted Agents	
APEC1621A pan-TRK inhibitor	Phase 2 study of LOXO-101 (Larotrectinib) in patients with tumors harboring NTRK fusions.
APEC1621B FGFR inhibitor	Phase 2 study of JNJ-42756493 (Erdafitinib) in patients with tumors harboring FGFR alterations
APEC1621C EZH2 inhibitor	Phase 2 study of Tazemetostat in patients with tumors harboring alterations in EZH2 or members of the SWI/SNF complex
APEC1621D	Phase 2 study of LY3023414 in patients with solid tumors

PI3K/mTOR	
APEC1621E MEK inhibitor	Phase 2 study of Selumetinib in patients with tumors harboring activating MAPK pathway mutations
APEC1621F ALK inhibitor	Phase 2 study of Ensartinib in patients with tumors harboring ALK or ROS1 genomic alterations
APEC1621G BRAF inhibitor	Phase 2 study of Vemurafenib in patients with tumors harboring BRAF V600 mutations
APEC1621H PARP inhibitor	Phase 2 study of Olaparib in patients with tumors harboring defects in DNA damage repair genes
APEC1621I CDK 4/6 inhibitor	Phase 2 subprotocol of Palbociclib in Patients with Tumors Harboring Activating Alterations in Cell Cycle Genes
APEC1621J ERK 1/2 Inhibitor	Phase 2 Subprotocol of BVD-523FB (ulixertinib) in Patients with Tumors Harboring Activating MAPK Pathway Mutations
MEK162 MEK inhibitor	Phase I-II Study of MEK162 for Children with Low Grade Gliomas and Other Ras/Raf/ERK Pathway Activated Tumors
NMTRC012	A Study Using Molecular Guided Therapy with Induction Chemotherapy followed by a Randomized Controlled Trial of standard immunotherapy with or without DFMO followed by DFMO maintenance for Subjects with Newly Diagnosed High-Risk Neuroblastoma