

Pediatric CIRB Meeting Agenda

October 14, 2021

I. Continuing Review

ACNS1831, A Phase 3 Randomized Study of Selumetinib (IND # 77782) versus Carboplatin/Vincristine in Newly Diagnosed or Previously Untreated Neurofibromatosis Type 1 (NF1) Associated Low-Grade Glioma (LGG) (Protocol Version Date 04/07/21)

II. Continuing Review

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 (Protocol Version Date 11/10/20)

III. Continuing Review

ANBL1232, Utilizing Response- and Biology-Based Risk Factors to Guide Therapy in Patients with Non-High-Risk Neuroblastoma. A Groupwide Historically Controlled Phase III Study (Protocol Version Date 11/13/17)

IV. Continuing Review

ANBL1531, A Phase 3 Study of ¹³¹I-Metaiodobenzylguanidine (¹³¹I-MIBG) or ALK Inhibitor Therapy Added to Intensive Therapy for Children with Newly Diagnosed High-Risk Neuroblastoma (NBL) (IND# 134379) (Protocol Version Date 09/22/21)

V. Continuing Review

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 (Protocol Version Date 06/29/20)

VI. Continuing Review

PBTC-056, A Phase I Study of the ADAM-10 inhibitor, INCB007839 in Children with Recurrent/Progressive High-Grade Gliomas to Target Microenvironmental Neuroligin-3 (Protocol Version Date 05/27/21)

VII. Continuing Review

PEPN1924, A Phase 2 Study of DS-8201A (NSC# 807708, IND# 153036) in Adolescents, or Young Adults with Recurrent HER2+ Osteosarcoma (Protocol Version Date 02/12/21)

VIII. New Study - Initial Review

PEPN2112, A Phase 1/ 2 Study of BAY 1895344 (elimusertib, IND#152153, NSC#810486) in Pediatric Patients with Relapsed or Refractory Solid Tumors (Protocol Version Date 08/27/21)

IX. New Study - Initial Review

PEPN2113-APAL2020C, A Phase 1 and pharmacokinetic study of Uproleselan (GMI-1271, IND #117870, NSC #801708) in combination with fludarabine and cytarabine for patients with acute myeloid leukemia, myelodysplastic syndrome or mixed phenotype acute leukemia that expresses E-selectin ligand on the cell membrane and is in second or greater relapse or that is refractory to relapse therapy (Protocol Version Date 08/27/21)

X. Amendment

AALL1731, A Phase 3 Trial Investigating Blinatumomab (IND# 117467, NSC# 765986) in Combination with Chemotherapy in Patients with Newly Diagnosed Standard Risk or Down syndrome B-Lymphoblastic Leukemia (B-ALL) and the Treatment of Patients with Localized B-Lymphoblastic Lymphoma (B-LLy) (Protocol Version Date 09/03/21)

XI. Memos

AALL1821, A Phase 2 Study of Blinatumomab (NSC# 765986, IND #125462) in Combination with Nivolumab (NSC# 748726, IND# 125462), a Checkpoint Inhibitor of PD-1, in B-ALL Patients Aged ≥ 1 to < 31 Years with First Relapse (Protocol Version Date 02/10/21)
