

Pediatric CIRB Meeting Agenda

January 11, 2024

I. Initial Review

PEPN2312, A Phase 1 Study of GRN163L (Imetelstat) in Combination with Fludarabine and Cytarabine for Patients with Acute Myeloid Leukemia That Is in Second or Greater Relapse or That Is Refractory to Relapse Therapy; Myelodysplastic Syndrome or Juvenile Myelomonocytic Leukemia in First or Greater Relapse or Is Refractory to Relapse Therapy (Version Date 11/28/23)

II. Continuing Review

AALL1631, International Phase 3 trial in Philadelphia chromosome-positive acute lymphoblastic leukemia (Ph+ALL) testing imatinib in combination with two different cytotoxic chemotherapy backbones (Version Date 09/16/22)

III. Continuing Review

AAML18P1, Stopping Tyrosine Kinase Inhibitors (TKI) to Assess Treatment-Free Remission (TFR) in Pediatric Chronic Myeloid Leukemia - Chronic Phase (CML-CP) (Version Date 10/06/20)

IV. Continuing Review

ACNS1833, A Phase 3 Randomized Non-Inferiority Study of Carboplatin and Vincristine versus Selumetinib (NSC# 748727, IND# 77782) in Newly Diagnosed or Previously Untreated Low-Grade Glioma (LGG) not associated with BRAFV600E Mutations or Systemic Neurofibromatosis Type 1 (NF1) (Version Date 09/16/22)

V. Continuing Review

ARST2031, A Randomized Phase 3 Trial of Vinorelbine, Dactinomycin, and Cyclophosphamide (VINO-AC) Plus Maintenance Chemotherapy with Vinorelbine and Oral Cyclophosphamide (VINO-CPO) vs Vincristine, Dactinomycin and Cyclophosphamide (VAC) plus VINO-CPO Maintenance in Patients with High Risk Rhabdomyosarcoma (HR-RMS) (Version Date 06/10/22)

VI. Continuing Review

PBTC-059, Phase 1 Trial of Autologous HER2-specific CAR T cells in Pediatric Patients with Refractory or Recurrent Ependymoma (Version Date 03/17/23)

VII. Continuing Review

PEPN2011, A Phase 1/2 Study of Tegavivint (IND#156033, NSC#826393) in Children, Adolescents, and Young Adults with Recurrent or Refractory Solid Tumors, Including Lymphomas and Desmoid Tumors (Version Date 02/13/23)

VIII. Continuing Review

PEPN2121, A Phase 1/2 Study of Tiragolumab (NSC# 827799, IND# 161266) and Atezolizumab (NSC# 783608, IND# 161266) in Patients with Relapsed or Refractory SMARCB1 or SMARCA4 Deficient Tumors (Version Date 10/19/23)

IX. Unanticipated Problem Review

PBTC-051, 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 (Version Date 09/23/22)

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