BACKGROUND: Household material hardship (HMH) is defined as holds material hardship. Furthermore, targeted interventions exist to modify targeted molecular alteration, who received compassionate pre.

METHODS: We aimed to enroll 150 patient family chooses. Through GFAC, children can pass at home, hospital, or hospice situation where the desire to donate comes at the cost of patient care and patient families. Through establishing standard operating procedures and medically limited to basic socioeconomic factors such as race and ethnicity. The often limited to basic socioeconomic factors such as race and ethnicity. The

RESULTS: Fifty-eight evaluable patients received MEKi for PN treatment-limiting toxicities by race and ethnicity. METHODS: This is a cation. Urgent need of targeted and novel therapies is warranted for pedi.

ABSTRACT CITATION ID: NOAE064.154 TRLS-01. INTRAVENTRICULAR B7-H3 CAR T CELLS FOR DIFFUSE INTRINSIC PONTINE GLIOMA: SAFETY AND EFFICACY REPORT FROM THE COMPLETED PHASE 1 TRIAL BRAINCHILD-03 Nicholas A Vitanza1,2, Rebecca Komsley2,3, Wenjuan Huang4, Krishy Seidel5, Alison Thomsen6, Juliane Gust4,5, Jason Hauptman6, Michelle Choe7, Erin Crotty5, Sarah Leary4,7, Joshua A Gustafson6, Rebecca A Gardner6, Sowmya Pattabhi8, Jason Wendler1, On Hu9, Colleen Annesley10, Julie R Park11, Michael C. Jensen12; 1Ben Towne Center for Childhood Cancer Research, Seattle Children’s Research Institute, Seattle, WA, USA, 2Department of Pediatrics, Seattle Children’s Hospital, University of Washington, Seattle, WA, USA, 3Seattle Children’s Therapeutics, Seattle, WA, USA, 4Department of Neurology, University of Washington, Seattle, WA, USA, 5Department of Radiation Oncology, University of Washington, Seattle, WA, USA, 6Division of Biomedical Informatics, Cincinnati Children’s Hospital Medical Center, Cincinnati, OH, USA, 7Department of Biostatistics & Health Data Sciences, Indiana University School of Medicine, Indianapolis, IN, USA, 8Department of Health Policy & Management, Columbia University, New York, NY, USA, 9Department of Pediatrics, University of Washington, Seattle, WA, USA, 10Department of Biostatistics, Stanford University/Lucile Packard Children’s Hospital Stanford, Stanford, CA, USA, 11Department of Medicine, Stanford University/Lucile Packard Children’s Hospital Stanford, Stanford, CA, USA, 12Department of Biostatistics & Health Data Sciences, Indiana University School of Medicine, Indianapolis, IN, USA.
BACKGROUND: BrainChild-03 is a first-in-human clinical trial that completed the phase 1 component of repeatedly dosed intraventricular B7-H3 CAR T cells to children with diffuse intrinsic pontine glioma (DIPG) without lymphodepleting chemotherapy. METHODS: We report on BrainChild-03 Arm C, patients with DIPG receiving up to 10x10⁶? B7-H3CAR T cells/dose. Primary endpoints were feasibility and safety. Secondary endpoints were disease response and correlates of CAR T activity. RESULTS: 23 patients enrolled. All had successful CAR T manufacturing. 21 patients were treated; 17 received sufficient doses for safety evaluation (4 doses/8 weeks) without a dose limiting toxicity (DLT). 4 other treated patients include: 1 patient DLT (pontine hemorrhage 8 days post-initial dose) and 3 unevaluable patients (2 discontinued protocol therapy for rapid progression; 1 did not meet eligibility to begin treatment). Overall, patients received 224 doses (median: 7, range: 1-60). Common adverse events included headache, nausea/vomiting, and fever. Of 21 treated patients, 11 enrolled after progression and survived 9.7 months (range: 6-16.9) after initial CAR T cell infusion. Of the 10 patients enrolled prior to progression, the overall survival is 16.9 months (range: 7.6-40.5) with 5/10 patients still alive. Three patients are alive 3 years from diagnosis, including 2 patients still on protocol therapy. Patients were not allowed to receive other anti-tumor agents, radiation, or surgical debulking while on protocol therapy. Circulating CSF CAR T cells were identified in 18/21 (85.7%) of patients and elevations in chemokine/cytokines associated with T cell activation and migration, including CXCL10 and IFNγ. CONCLUSIONS: Repeated intraventricular B7-H3 CAR T cell dosing is feasible and tolerable for children with DIPG, even after progression, with evidence of local immune activation. Early signals of survival extension underscore the potential for clinical benefit and the consideration to advance to phase 2 multi-site clinical trials.