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MDB-83. OUTCOMES OF INFANTS AND YOUNG CHILDREN WITH NEWLY DIAGNOSED SHH MEDULLOBLASTOMA TREATED ON THE NEXT CONSORTIUM “HEAD START” 4 PROTOCOL
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BACKGROUND: Infants and young children with SHH medulloblastoma were demonstrated to have a favorable outcome on “Head Start” III clinical trial utilizing five cycles of induction followed by one myeloablative high-dose chemotherapy (HDCt) cycle. We present the results of “Head Start” 4 (HS-4) trial where SHH subgroup patients received either three or five cycles of induction based on response followed by one HDCt cycle (similar to HS III). METHODS: Eligibility included children <6 years of age at diagnosis of localized medulloblastoma and <10 years for patients with disseminated disease. Eligible patients with SHH medulloblastoma were considered “low-risk” and non-randomly assigned to receive three cycles (five cycles if complete response of induction chemotherapy [vinristine, cisplatin, cyclophosphamide, etoposide, and high-dose methotrexate] followed
by consolidation with single HDCT cycle (thiotepa, carboplatin, etoposide) and autologous hematopoietic stem-cell rescue. RESULTS: Thirty-nine children with SHH medulloblastoma were enrolled on HS-4 with median age of 2.18 years (range: 0.28-6.88 years). Median follow-up for this cohort is 41 months (range: 18-67 months). Patients with localized SHH medulloblastoma (n=28) had significantly better 3-year event-free (EFS) compared to disseminated patients (n=11): 96.4% (95% CI: 90-100%) and 36.4% (95% CI: 16.6-79.5%), respectively (p=0.0001); however, there was no significant difference in 3-year overall survival (OS) between the two groups: 100% and 90.0% (95% CI: 73.2-100%), respectively (p=0.10). The estimated 3-year EFS for localized SHH subtype 1 and 2 patients was 100% and 95%, respectively (p=0.63). None of trial patients received irradiation prior to progression. All patients, except for four, underwent three cycles of induction. Germline variants were detected in 27% of patients tested (8/30).

CONCLUSION: We report excellent results for young children with localized SHH medulloblastoma when treated with only three cycles (reduced) of induction and single HDCT cycle on HS-4 trial without irradiation. Molecular data will be presented.