specification by altering the components of the PRC1 complex. These studies identify the mechanistic basis of BMI1 co-operation with SMARCB1 loss in ATRT and establish BMI1 inhibition as a novel therapeutic approach in ATRT.

ATRT-07. HIGH-DOSE CHEMOTHERAPY AND AUTOLOGOUS STEM CELL TRANSPLANTATION FOR AN ADULT PRESENTATION OF THE ATYPICAL TERATOID-RHABDOID TUMOR (ATRT)

Maciej Mrugala[1], Aditya Raghunathan[2], and Jose Leis[1]; [1]Mayo Clinic, Phoenix, AZ, USA, [2]Mayo Clinic, Rochester, MN, USA

BACKGROUND: ATRT is a rare primary CNS tumor occurring predominantly in children with the peak age of onset at less than 3 years old. Adult presentations are exceedingly rare, associated with poor prognosis and no standard therapies exist. METHODS: Case presentation. RESULTS: 61 y old woman presented with headaches, sinus pressure, and cognitive decline. She was found to have a pineal tumor causing obstructive hydrocephalus. The patient underwent gross total resection of the tumor with pathology reported as ATRT. Her CNS staging, including CSF, was negative. She subsequently received radiotherapy to the resection bed. There was no consensus on what should be the next step in her therapy given lack of data in adults. Ultimately, we adopted a pediatric regimen and treated the patient with a combination of high-dose chemotherapy with cisplatin, cyclophosphamide, and vincristine followed by autologous stem cell transplantation (ASCT). This regimen called for up to 4 cycles of chemotherapy with ASCT and we had collected enough cells to complete 3 cycles. The patient completed 2 cycles of therapy with moderate toxicity. Her CNS imaging remained stable with no evidence of recurrence 14-months from the original diagnosis. CONCLUSIONS: ATRT continues to be an exceedingly rare diagnosis in adults. No standard therapies exist and treatment decisions are challenging given lack of data and lack of prospective clinical trials. Pediatric regimens can frequently be adopted for adults although high-dose chemotherapy with ASCT can be challenging. Our case exemplifies the feasibility of treating ATRT in an adult in the most aggressive fashion.

ATRT-08. A PHASE II STUDY OF CONTINUOUS LOW DOSE PANOBINOSTAT IN PAEDIATRIC PATIENTS WITH MALIGNANT RHABDOID TUMORS/ATYPICAL TERATOID RHABDOID TUMORS

Paul Wood[1,2], Jayesh Desai[3,4], Kelly Waldeck[3], Jason Cain[5], Nick Gottardo[6], Robyn Strong[7,8], Kathryn Kinross[7,8], Michelle Carr[7,8], Janelle Jones[7,8], Lily Wong[9], David Ziegler[10], Jordan Hansford[11,12], Michael Michael[3], and David Ashley[13]; [1]Monash Health, Melbourne, VIC, Australia, [2]Monash University, Melbourne, VIC, Australia, [3]Peter MacCallum Cancer Centre, Melbourne, VIC, Australia, [4]Australia and New Zealand Sarcoma Association, Melbourne, VIC, Australia, [5]Hudson Institute of Medical Research, Melbourne, VIC, Australia, [6]Princess Margaret Hospital for Children, Perth, WA, [7]Australian and New Zealand Children's Haematology/Oncology Group, Melbourne, VIC, Australia, [8]Australasian Children's Cancer Trials, Melbourne, VIC, Australia, [9]Kids Oncology and Leukaemia Trials (KOALA), Sydney, NSW, Australia, [10]Sydney Children's Hospital, Sydney, NSW, Australia, [11]Royal Children's Hospital, Melbourne, VVIC, Australia, [12]University of Melbourne, Melbourne, VIC, Australia, [13]The Preston Robert Tisch Brain Tumor Centre, Durham, NC, USA

BACKGROUND: Panobinostat treatment has been shown to terminally differentiate malignant rhabdoid tumor (MRT)/atypical teratoid rhabdoid tumors (ATRT) in pre-clinical models. This is an open label, phase II study of panobinostat in patients with newly diagnosed or relapsed MRT/ATRT. AIMS: To assess the anti-tumor activity of low dose, continuous panobinostat, its associated toxicities, the biological activity of low dose panobinostat by measuring histone acetylation status in peripheral mononuclear cells (PMNC), and markers of differentiation in fresh tumor tissue specimens. METHODS: Following cycles of induction and consolidation chemotherapy and/or radiation treatment, patients were enrolled and commenced on panobinostat as a continuous daily oral dose starting at 10mg/m^2 following a three-week wash out period between therapies. Real-time acetylation status, measuring acetylated H4 on PMNC, was performed to determine the pharmacodynamics of panobinostat. Patients were monitored for drug toxicities with the possibility of dose reductions in decrements of 2mg/m^2. RESULTS: Six patients with newly diagnosed ATRT/MRT and one patient with relapsed MRT have been enrolled to date. The average age at enrollment was 2.5 years. Currently, six patients (85.7%) remain on study with a mean treatment duration of 170 days (range 44–327 days). One patient was removed from study at day 44 due to disease progression. The main dose-limiting toxicity observed to date has been myelosuppression. Panobinostat, at a dose of 10mg/m^2, caused significant acetylation of H4 in PMNC. CONCLUSIONS: Treatment with panobinostat appears to be well tolerated in infants with MRT/ATRT, with successful real-time pharmacodynamic assessment of H4 acetylation.

ATRT-09. IDENTIFICATION OF HUB GENES IN ATYPICAL TERATOID/RHABDOID TUMORS BY MULTIPLE-MICROARRAY ANALYSIS

Wei Liu[1,2], Yi Chai[1,2], Junhua Wang[1,2], and Yuqi Zhang[1,2]; [1]School of Clinical Medicine, Tsinghua University, Beijing, China, [2]Department of Neurosurgery, Yuquan Hospital, School of Clinical Medicine, Tsinghua University, Beijing, China

BACKGROUND: Atypical teratoid/rhabdoid tumors (ATRT) are rare, highly malignant neoplasms arising in infants and young children. However, the biological basis of ATRTs remains poorly understood. In the present study, we employed integrated bioinformatics to investigate the hub genes and potential molecular mechanism in ATRT. METHODS: Three microarray datasets, GSE35943, GSE6635 and GSE86574, were downloaded from Gene Expression Omnibus (GEO) which contained a total of 79 samples including 32 normal brain tissue samples and 47 ATRT samples. The RobustRankAggreg method was employed to integrate the results of these gene expression datasets to obtain differentially expressed genes (DEGs). The GO function and KEGG pathway enrichment analysis were conducted at the Enrichr database. The hub genes were screened according to the degree using Cytoscape software. Finally, transcription factor (TF) of hub genes were obtained by the NetworkAnalyst algorithm. RESULTS: A total of 297 DEGs, consisting of 94 downregulated DEGs and 103 upregulated DEGs were identified. Functional enrichment analysis revealed that these genes were associated with cell cycle, p53 signaling pathway and DNA replication. Protein-protein interaction (PPI) network analysis revealed that CDK1, CCNA2, BUB1B, CDC20, KIF11, KIF20A, KIF2C, NCAPG, NDC80, NUSAP1, PBK, RRM2, TPX2, TOP2A and TTK were hub genes and these genes could be regulated by MYC, SOX2 and KDM5B according to the results of TF analysis. CONCLUSIONS: Our study will improve the understanding of the molecular mechanisms and provide novel therapeutic targets for ATRT.

ATRT-10. ATYPICAL TERATOID/RHABDOID TUMOR OF THE PINEAL REGION IN A PEDIATRIC PATIENT

Carlos Almeida Jr[1], Bruna Minniti Mançano[1], Marcus Matsushita[1], Stephanie Previdelli[2], Marina Lopes Lamim[2], Fabio Costa Faustino[1], Fernanda Magalhaes Souza[1], and Lucas Dias Lourenço[1]; [1]Barretos´s Children and Young Adults Cancer Hospital, Barretos, Sao Paulo, Brazil, [2]School of Health and Science Dr Paulo Prata, Barretos, Sao Paulo, Brazil

BACKGROUND: Atypical teratoid/rhabdoid tumor (ATRT) is a malignant neoplasm of the central nervous system and corresponds to 1.5% of all intracranial tumors. Mainly affects children under three years of age and shows aggressive behavior (most pediatric patients succumb to their disease within a year after the initial diagnosis, despite the treatment performed). Its place of occurrence in children is preferably in the posterior fossa, and it is rare to appear in other regions. There are only seven patients with ATRT reported on literature; all of them are adults. We present the case of a pediatric patient with a tumor in the pineal region diagnosed as ATRT. CASE REPORT: Three-year-old female patient admitted with occipital headache, vomiting, and seizure. Magnetic resonance imaging (MRI) showed obstructive hydrocephalus secondary to a solid-cystic lesion located at the pineal region that was 3.0 x 3.0 x 3.5 cm in size. Spine MRI did not reveal leptomeningeal spreading. We performed an occipital transtentorial approach to achieve the best safe resection possible, and a ventriculoperitoneal shunt. Histological examination revealed ATRT. The patient received adjuvant treatment with radiotherapy and chemotherapy according to the "Head Start" protocol. One year after the surgery, MRI did not identify any remaining lesion. CONCLUSION: ATRT is an aggressive and rare neoplasm whose clinical picture depends on the location of the tumor; however, it must be considered in the differential diagnosis of tumors of the pineal region in the pediatric population.

ATRT-11. PREVALENCE OF GERMLINE VARIANTS IN SMARCB1 INCLUDING SOMATIC MOSAICISM IN AT/RT AND OTHER RHABDOID TUMORS

Ryota Shirai[1,2], Tomoo Osumi[1,3], Keita Terashima[3], Chikako Kiyotani[3], Meri Uchiyama[1,3], Shinichi Tsujimoto[1,3], Masanori Yoshida[1,2], Kaoru Yoshida[1], Toru Uchiyama[4], Daisuke Tomizawa[3], Yoko Shioda[3], Masahiro Sekiguchi[5], Kenichiro Watanabe[6], Dai Keino[7], Hitomi Ueno-Yokohata[1], Kentaro Ohki[1], Junko Takita[5], Shuichi Ito[2], Takao Deguchi[3], Nobutaka Kiyokawa[1], Hideki Ogiwara[8], Tomoro Hishiki[9], Seishi Ogawa[10], Hajime Okita[11,12], Kimikazu Matsumoto[3], Takako Yoshioka[11], and Motohiro Kato[1,3]; [1]Department of Pediatric Hematology and Oncology Research, National Research Institute for Child Health and Development, Tokyo, Japan, [2]Department of Pediatrics, Yokohama City University, Yokohama, Japan, [3]Children's Cancer Center, National Center for Child Health and Development, Tokyo, Japan, [4]Department of Human Genetics, National Research Institute for Child Health and Development, Tokyo, Japan,