ences between children with NF1 and controls were greater at younger than older ages. CONCLUSION: Microstructural differences were observed in WMTs in children with NF1 compared to controls. These differences were not explained by intracranial volume and were most pronounced in younger children with NF1 compared to controls. These findings have implications for understanding neurocognitive deficits and gliomagenesis observed in children with NF1.

NFB-12. TRAMETINIB THERAPY FOR PEDIATRIC PATIENTS WITH REFRACTORY LOW GRADE GLIOMA OR EXTENSIVE SYMPTOMATIC PLEXIFORM NEUROFIBROMA

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OBJECTIVE: Refractory symptomatic plexiform neurofibromas (PNF) and inoperable refractory low grade gliomas (LGG) pose a clinical challenge that may be life threatening. Phase 1 and 2 clinical trials of MEK inhibition with selumetinib in inoperable PNF and LGG have demonstrated promising results in pediatrics, however access has been limited to enrollment on clinical trial. Phase 1 clinical trial for trametinib a MEK 1 and 2 inhibitor has been completed, publication is pending. Thus we have treated a series of children on a compassionate basis with extensive PN or LGG refractory disease with trametinib, as this is available in Canada. METHODS: We have treated children with trametinib on a compassionate basis in our province since 2017. Review of the clinical data regarding this therapy has been IRB approved. RESULTS: Two young patients were treated for indication of life threatening extensive PNF and have had tumor shrinkage and improvement of clinical status. Treatment has been complicated by paronychiae, eczema exacerbation, chondrodermatitis nodularis helicis, RSV and influenza B infection and CTCAE grade 2 pneumonia. In spite of the side effects these two patients remain on treatment due to clear benefit from therapy including: improved respiratory compromise, hearing and dysphagia. We will present the data of additional patients treated with trametinib. CONCLUSION: Trametinib is an effective therapy for life threatening PNF by changing the natural history of tumor growth in young children. Further data is required in terms of tolerance, efficacy and durability of response in such patients in the setting of clinical trials.

NFB-13. TRAMETINIB FOR PLEXIFORM NEUROFIBROMA AND RECURRENT LOW-GRADE GLIOMA

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BACKGROUND: Based on early clinical efficacy data, Seattle Children's established a standard clinical practice for MEK inhibitor therapy for children with plexiform neurofibroma (PN) or recurrent low-grade glioma (LGG). METHODS: Data were collected under an IRB-approved retrospective chart review. Trametinib was prescribed off-label at 0.025 mg/kg daily for up to two years. Physical exam and laboratory monitoring were monthly for 3 months, then every 3 months. Retinal examination, ECHO/ECG were every 3 months. Tumor response was evaluated by MRI every 3 months for LGG; imaging for PN was dependent on tumor location. RESULTS: 30 patients received trametinib; 17 LGG, 16 PN (3 both); 22 with Neurofibromatosis, Type-1 (NF1); 16 female/15 male; median age 11 (range 4.1–22.6). Most common tumor location was optic pathway (n=11) and face/neck (n=10). Most common adverse events (AE) were dermatologic and gastrointestinal. Ten had dose interruption/reduction, only one discontinued therapy for AE. Six received dermatology specialty care for AE. With median follow-up of 12 months, only 3 patients had progression, one with NF1. One-year EFS was 100% for PN and 88%+7 for LGG. Driver mutations were identified in 9 of 10 tumors tested (5 BRAF fusion, 1 BRAFV600E, 1 FGFR1+NF1, 1 FGFR1+PTPN11, 1 NF1). Radiology review of response will be presented. CONCLUSIONS: This real-world pediatric cohort supports efficacy and tolerability of MEK inhibitor therapy for short-term control of plexiform neurofibroma and low-grade glioma with and without NF1. Further studies are warranted to evaluate comparative efficacy, combination therapy and duration of therapy.

NFB-14. PSYCHOSOCIAL OUTCOMES IN CHILDREN WITH NEUROFIBROMATOSIS TYPE 1 AND PLEXIFORM NEUROFIBROMAS

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OBJECTIVE: This case series seeks to examine neurocognitive outcomes, social-emotional functioning, and family burden in young children diagnosed with Neurofibromatosis, type 1 (NF1) with early growing plexiform neurofibromas (PNFs). BACKGROUND: Neurofibromatosis, type 1 (NF1) is a common predisposing chronic disease arising in early childhood, with an incidence of approximately 1:3000. Though NF1 displays a wide range of phenotypic variability, the primary feature of the disease is peripheral nerve sheath tumors called neurofibromas. Less is well known regarding the broader neurocognitive and social-emotional profile in presentations with more complex tumor growths, namely PNFs, which are present in at least half of the NF1-affected population. METHODS: Participants with NF1 and PNFs (n=2) aged 6-7years completed comprehensive neuropsychological evaluations and parents completed measures of quality of life, social-emotional/behavioral functioning of child, parental stress, family adaptability, and family cohesion. RESULTS: Outcomes suggest broad neurocognitive dysfunction (e.g., executive functioning deficits, attention problems, visual-motor delays, and poor motor coordination), social-emotional challenges (e.g., symptoms of anxiety and depression, and poor social skills), and familial distress. CONCLUSIONS: Findings indicate the value of early and frequent monitoring of children with PNFs in medical systems and multi-disciplinary teams, and the importance of early intervention for both children and families.

NFB-16. MTOROPATHIES AND SUBEPENDYMAL GIANT CELL ASTROCYTOMAS: PREDICTIVE VALUE OF GERMINAL TSC1/2 MUTATIONS SCREENING IN FAMILIAL CASES

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mTOR controls several important aspects of cell function particularly in the nervous system. Its hyperactivation has been involved in tuberous sclerosis complex (TSC) and other mTORopathies as well as drug-resistant epilepsy. Mutations in TSC1 and TSC2 genes cause loss of normal inhibition of mTORC1 complex, leading to cell overgrowth and disruptions in synaptogenesis. Many children and adults with TSC harbour neurologic defects especially subependymal giant cell astrocytomas (SEGAs) in the brain. Here, we have performed mutational analysis followed by a genetic counselling for a Tunisian family from Sfax town harboring epileptic seizures associated to a neurocutaneous disorder. Index cases were referred for renal angiolipomas (RAL) associated to seizures crisis and were diagnosed as having TSC. The first 26-year-old patient complained of epilepsy since the age of 22 with left temporal crisis related to cortical tubers near the Heschl's gyrus. His brother, a 36-year-old man presented more severe epileptic crisis (since 15 years-old), multiples RAL, subependymal nodules, and a rapid evolution of his mTORopathy with tumoral progression of his renal and central nerve lesions: renal cell carcinoma and SEGAs. TSC1 gene mutation screening showed heterozygous two bp deletion at codons 213 and 214 of exon 5. SEGAs are rare, low-grade glioneuronal brain tumors that occur almost exclusively in TSC patients but can lead to nervous complications. We showed through this report, the predictive value of germinal TSC mutations screening in familial cases, because early recognition of the molecular defect may lead to appropriate management of the tumoral progression.

NFB-17. MEK INHIBITOR BINIMETINIB SHOWS CLINICAL ACTIVITY IN CHILDREN WITH NEUROFIBROMATOSIS TYPE 1-ASSOCIATED PLEXIFORM NEUROFIBROMAS: A REPORT FROM PNOC AND THE NF CLINICAL TRIALS CONSORTIUM

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BACKGROUND: Plexiform neurofibromas (PNs) can cause significant morbidity. In this phase 2 study, we assessed imaging and functional outcomes to the MEK-inhibitor Binimetinib in pediatric patients with PNs. METHODS: Children (age 1–17 years) with PN that were progressive or causing significant morbidity were eligible. Binimetinib is dosed twice-daily (starting dose of 32mg/m^2) for maximum of 24 four-week courses. Participants with partial response (PR; >20% decrease in PN volume on central MRI review) at cycle 12 may stay on therapy. Participants undergo MRI and functional assessments at baseline and after courses 4, 8, 12, 18 and 24. Functional assessments are based on PN location. RESULTS: Here we present 1-year response data. Twenty participants (55% male) with median age 12 years (range 2–16 years) enrolled; 19 are evaluable for response. Median baseline tumor volume was 326 ml (range, 8-6661 ml). Fourteen participants (74%) met criteria for PR, with 11 achieving PR by course 5. Median maximal PN volume reduction was 25.5% (range, 9–54%). As of August 2020, 14 participants received at least 12 cycles of Binimetinib; 10 remain on therapy. Off study reasons include treatment associated toxicities (n=2), subject withdrawal (n=2), non-compliance (n=2), prolonged treatment delay (n=1), and lack of response (n=3). Thirteen participants underwent dose reduction. Institution-reported related grade 3 toxicities included dry skin, weight gain, muscle weakness, rash, paronychia, cellulitis, diarrhea, gastric hemorrhage and CPK increase. CONCLUSIONS: Binimetinib appears reasonably well-tolerated and shows promising activity in children with NF1-associated PNs. Outcomes on functional improvement will be reported at the meeting.

NFB-18. IMMUNE FUNCTION IN CHILDREN TREATED WITH TRAMETINIB

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BACKGROUND: Trametinib (Tr) has been applied in the treatment of children with various tumor types, often for prolonged periods. Little is known regarding immune function (IF) following prolonged Tr in this age group. OBJECTIVE: Describe laboratory measures of IF in children on Tr. METHOD: Patients receiving Tr had low grade glioma with BRAF anomalies (6), or neurofibromatosis-1 (16) with glioma or plexiform neurofibroma. IF was evaluated using leukocyte/lymphocyte counts, immunoglobulin levels, and antibody titres. RESULTS: 22 patients received Tr. 2 also received Dabrafenib. Median age at Tr initiation of Tr was 7.75 years. As of June 2020, 7 patients have had IFT; results are pending on 15. Median duration of Tr therapy at time of IF was 3.5 years (0.8 – 4). In these 7 patients, median white cell count was 6.9 x 10^9/L (4.1 – 12.6), neutrophils 4.2 x 10^9/L (1.8 – 6.8) and lymphocytes 3.2 x 10^9/L (1.4 – 7). IgG levels, B cells and CD8 cytotoxic T cells were normal across 7/7 patients:medians 9.47 g/L (8.62 – 17), 0.51 x 10^9/L (0.2 – 1.26) and 0.58 x 10^9/L (0.25 – 2.03) respectively. CD3 and CD4 T cells: median 2.08 x 10^9/L (0.67 – 4.62) and 1.34 x 10^9/L (0.35 – 2.31), borderline low in 1 heavily pre-treated patient. An adequate immune response was present in all 4 vaccine antigens tested in 5/5 patients. CONCLUSION: IF appears relatively intact, relevant for immunisation and infection precautions in children on Tr. Data on the complete cohort will be presented.

NURSING/PATIENT CARE

NURS-01. INTRACEREBROVENTRICULAR DRUG ADMINISTRATION FOR TREATMENT OF PEDIATRIC BRAIN TUMORS

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Intrathecal (IT) chemotherapy, given via lumbar puncture (LP) or an intracerebroventricular (ICV) device has become a safe and effective way to deliver chemotherapy into the cerebrospinal fluid (CSF) space. The blood brain barrier makes treating tumors with CSF dissemination difficult with systemic chemotherapy alone. IT chemotherapy is often necessary for tumors which disseminate into the CSF space including embryonal tumors and choroid plexus carcinomas. It is also used for relapsed or recurrent tumors. Giving IT chemotherapy via an ICV device instead of via an LP can be preferable as it requires no deep sedation and allows for more uniform drug distribution. Drugs given IT include methotrexate, cytarabine, hydrocortisone, etoposide, and topotecan. ICV devices can be placed in patients with adequate CSF flow and a flow study can be done if needed to confirm. Accessing the ICV device for administration of chemotherapy is typically done by a physician or nurse practitioner using sterile technique. Our institution has had success using music therapy and child life specialists for assistance with coping during the procedure as patients are awake. The procedure has few complications the most common being infection usually with skin flora. It can also cause nausea and headache. There are few long term risks.

NURS-02. CLINICAL MANAGEMENT OF PATIENTS RECEIVING CAR T CELL THERAPY FOR CNS TUMORS

Susan Holtzclaw, and Corrine Hoeppner; Seattle Children's Hospital, Seattle, WA, USA

Chimeric antigen receptor (CAR) T cells are an innovative new therapy with proven efficacy in some pediatric cancers such as leukemia and lymphoma, but much less experience in solid tumors, especially tumors of the central nervous system (CNS). Seattle Children's has three open Phase 1 CAR T cell studies (BrainChild-01, -02, and -03 targeting HER2, EGFR, and B7-H3, respectively) for recurrent/refractory CNS tumors and DIPG (BrainChild-03 only). As of December 2019, four patients have been treated at Seattle Children's Hospital with CAR T cells infused on a weekly schedule through indwelling catheters into the tumor resection cavity or ventricular system. Given the scrutiny of clinical care needed for Phase 1 studies, we are now able to report detailed clinical information that we have learned during the treatment of these patients. Clinical care includes the judicious use of steroids, the clinical support of patient's symptoms pre- and post-infusion, and the management of peritumoral edema. We will also discuss the psychosocial support needed for families who travel long distances to receive this therapy compounded by the many emotional components of being enrolled on any Phase 1 trial. Case studies and experience from a Nurse Practitioner role will be provided and discussed.

NURS-03. DEVELOPMENT OF A PATIENT-HELD TREATMENT SUMMARY FOR PAEDIATRIC CNS TUMOUR PATIENTS

Rachel McAndrew, Bernadine Wilkie, Mark Brougham, and Jo Phillips; NHS Lothian, Edinburgh, Scotland, United Kingdom

BACKGROUND: Following the Scottish Government Cancer Plan 2012–15*(1)* 'End of Treatment' summaries for paediatric oncology patients treated in SE Scotland have been successfully implemented. However, it became evident that the particular needs of patients with CNS tumours were not adequately captured on the standardised documentation. METHODS: In view of these difficulties an alternative document was prepared specifically for this patient cohort by the multi-disciplinary team, including Nurse Specialists, Paediatric Neuro-oncology and Neuro-psychology. This was designed to be a flexible, fluid summary to be used for all such patients regardless of tumour grade or treatment modality and included those undergoing surveillance only. OUTCOMES: The document is primarily completed by the Neuro-Oncology Nurse Specialist alongside the patient and family, usually following initial treatment and is used alongside their holistic needs assessment. The document is circulated to all involved professionals, including Primary Care, and a copy is retained by the patient. This then provides a concise source of information detailing diagnosis and treatment, any specific ongoing sequelae and details of red flag symptoms to alert patients and health professionals to the potential of relapse or other associated significant health problems. These treatment summaries are currently being piloted and have been well received thus far. They will be formally audited in due course with the aim to use nationally throughout Scotland in future.

NURS-04. COMBINATION OF NEURO-ONCOLOGY AND DERMATOLOGY CLINICS IMPROVE THE MANAGEMENT AND KNOWLEDGE OF SKIN-RELATED TOXICITIES WITH MEK AND BRAF TARGETED THERAPY

Tara McKeown, Irene Lara-Corrales, and Andrea Cote; Hospital for Sick Children, Toronto, ON, Canada

BACKGROUND: The recent advancement in treating pediatric low grade glioma has led to upfront use of MEK and BRAF (MAPK) inhibitor therapy. At the Hospital for Sick Children we are the National leaders in treating pediatric oncology diagnosis with MAPK therapies. DESIGN: After treating several patients on MAPK inhibitors with various degrees of skin toxicity, we found we had poor and inconsistent access to dermatology services and as oncology practitioners had limited front-line knowledge about skin management. It was determined that a more formalized expertise and time with dermatology was needed. In 2018, in combination with the derma-