Abstracts

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 related to baseline intravoxel volume and were prominent 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 symptomaticplexiform 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. Here we report our experience and list of patients referred to enrolment 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 who met criteria, 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: improvement in respiratory complications, hearing and dysphagia. We will present the data of additional patients treated with trametinib. CONCLUSION: Trametinib is an effective therapy for life threatening PN 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 (LGG). 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 growingplexiform neurofibromas (PNFs). BACKGROUND: Neurofibromatosis type 1 (NF1) is a common predisposing chronic disease arising in early child-life, with devastating consequences for patients, families, and society. METHODS: Participants with NF1 and PNFs (n=2) aged 6–7 years completed comprehensive neuropsychological evaluations and parents completed measures of quality of life, social-emotional functioning, 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 multidisciplinary teams, and the importance of early intervention for both children and families.

NFB-16. MITOPATHIES 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 mitogen-activated protein kinase (MEK) pathways. Screening showed through this report, the predictive value of germinal TSC1/2 mutations to identify patients at risk (e.g., penetrance, clinical status, and familial distress. Additional studies are warranted to evaluate the comparative efficacy, tolerability, and long-term outcomes of MEK inhibitor therapy for children with NF1 with extensive tumor burden.

NFB-17. MEK INHIBITOR BINMETINIB 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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We identified 9 in 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.
and choroid plexus carcinomas. It is also used for relapsed or recurrent tumors. Giving IT chemotherapy via an IVC 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. IVC devices can be placed in patients with adequate CSF flow and a flow study can be done if needed to confirm. Accessing the IVC device for administration of chemotherapy is typically done by dermatology or nurse practitioners, but with training and experience, 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-03. DEVELOPMENT OF A PATIENT-HELD TREATMENT SUMMARY FOR PAEDIATRIC CNS TUMOUR PATIENTS

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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 these themes: absence of treatment, treatment delay (n=1), and lack of response (n=3). Thirteen participants underwent dose reduction. Institution-reported related grade 3 toxicities included dexamethasone, muscle weakness (n=1), change in mental state (n=1), diarrhea, gastric hemorrhage and CPK increase. CONCLUSIONS: Binimetinib appears reasonably well-tolerated and shows promising activity in children with NFI-associated PNs. Outcomes on functional improvement will be reported at the meeting.

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

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BACKGROUND: The recent advancement in treating pediatric low grade gloma 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 inhibitors. 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-