OTHR-06. KNOWLEDGE GAP AMONG HEALTH CARE PROVIDERS IN CHILDHOOD CENTRAL NERVOUS SYSTEM TUMORS: RESULT FROM AN INTERNATIONAL MULTICENTER SURVEY

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BACKGROUND: Central nervous system (CNS) tumors in children are associated with a longer delay in diagnosis. One of the contributing factors is the lack of awareness regarding childhood CNS tumors presentation among health care providers. To evaluate the knowledge gap among health care providers, we conducted a cross-sectional survey that was distributed globally. METHODS: The survey was disseminated to health care practitioners via electronic mail in November 2018 and it was closed in March 2020. The participants were asked to complete a pre-test survey, requested to view a CNS tumor education seminar, and subsequently complete a post-test survey. The survey had nine questions focusing on CNS tumor symptoms, pre-diagnosis symptom interval (PSI), and imaging indication. The knowledge gap was evaluated with pre-test and post-test scores. RESULTS: 889 pre-test and 392 post-test responses were received. The majority of the respondents were from Asia, with a percentage of 73.1% and 87.5% in pre-test and post-test respectively. For the pre-test, the median score for accurate answers was 40.0% (range:13.1-92.9%). Interestingly, a high rate of correctness was achieved in the post-test with a median score of 77.1% (14.9-98.2%). In the pre-test, only 18.7% of the participants responded precisely that Cushing's triad is a less common symptom and just 15.0% recognized that older children >10 years old are at risk for late diagnosis. Surprisingly, 21.9% falsely reported that patients with malignant tumors experience the longest PSI, and 54.5% of the respondents wrongly selected medulloblastoma as the commonest CNS tumor. Overall, the pre-test scores among pediatricians and professionals with >10 years of experience did not demonstrate improved knowledge when compared to other specialties. CONCLUSIONS: The survey analysis showed a significant knowledge gap regarding childhood CNS tumors among health care providers. Therefore, raising professional awareness is very important and can be achieved through targeted educational strategies.

OTHR-07. A NEW FRAMEWORK FOR MISSING VALUE TOLERANT DATA INTEGRATION

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Dataset integration is common practice to overcome limitations, e.g., in statistically underpowered omics datasets. This is of particular importance when analyzing rare tumor entities. However, combining datasets leads to the introduction of biases, so called 'batch effects', which are due to differences in quantification techniques, laboratory equipment or used tissue type. A common problem is the missing quantification for features like gene transcripts or proteins within a dataset. These missing values can appear at random in a given dataset and also get introduced by combination of multiple datasets. Currently, strategies beyond common normalization for batch effect reduction are either missing entirely or are unable to handle absence of data points and therefore rely on error-prone data imputation. We introduce a framework that enables batch effect adjustments for combined datasets while avoiding data loss by appropriately handling missing values without imputation. The underlying idea is based on a matrix dissection approach, adjusting common information from the integrated dataset under guarantee of sufficient data presence. The strategy is implemented within the R environment and linked with popular software stacks that are built on top of R. Successful data adjustment is exemplarily shown for proteomic data generated by different quantification approaches and LC-MS/MS instrumentation setups.

OTHR-08. PEDIATRIC NEUROLOGIC ASSESSMENT IN NEURO-ONCOLOGY (PNANO) SCALE: A TOOL TO ASSESS NEUROLOGIC FUNCTION FOR RESPONSE ASSESSMENT IN NEURO-ONCOLOGY (RAPNO)

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BACKGROUND: The Neurologic Assessment in Neuro-Oncology (NANO) scale, a standardized metric to objectively measure neurologic function in adult brain tumor patients, complements radiographic assessment in evaluating outcomes of neuro-oncology patients in clinical trials and clinical practice. Currently, there is no standardized measure for neurologic function in pediatric neuro-oncology patients despite their distinct clinical presentations and tumor locations. Therefore, we developed a dedicated pediatric NANO (pNANO) scale. METHODS: An international group of pediatric neurologists and adult and pediatric neuro-oncologists convened bi-weekly over 5 months to draft the pNANO scale as an objective and quantifiable measure of neurologic function in children that can be administered during routine examination by pediatric-trained providers of any subspecialty and be utilized together with other indicators to assess response in clinical trials. RESULTS: Ten relevant domains of neurologic function were identified based on common pediatric brain tumor locations: gait, strength, cerebellar function, visual fields, visual acuity, facial strength, level of consciousness, extraocular movements, dysarthria and dysphagia. For each domain, developmental age appropriate levels of function were defined and categorized. Each domain, based on direct observation and testing during any routine neurological exam, can be used for longitudinal monitoring. CONCLUSIONS: The pNANO scale has been developed and aims to provide an objective metric of neurologic function for pediatric brain tumor patients. This scale will be tested for reliability, feasibility and inter-observer variability. Consistent evaluation of neurologic function using pNANO along with radiographic assessment will enable more comprehensive and standardized response assessment in pediatric neuro-oncology patients enrolled in clinical trials.

OTHR-09. THE PREVALENCE AND COMPLEX MANAGEMENT OF MEK INHIBITOR INDUCED CUTANEOUS SIDE EFFECTS

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BACKGROUND: Cutaneous side effects commonly occur with MEK inhibitor (MEKi) therapy and can be challenging to manage. METHODS: A retrospective chart review was performed on sixteen pediatric patients treated with MEKi therapy for at least three months. These patients were diagnosed with either a brain tumor, plexiform neurofibroma, Langerhans Cell Histiocytosis, or Parkes Weber Syndrome (PWS). OBJECTIVES: To describe cutaneous side effects from MEKi therapy, compare the side effect profiles of patients treated for different diagnoses, and compare the side effect profiles between different MEKi agents. RESULTS: The most prevalent cutaneous toxicities for all eligible patients were acneiform rash (81%, most common), paronychia (50%, second most common), hair changes and xerosis. Mean number of cutaneous skin toxicities were 3.2 (trametinib group: 3.4, selumetinib group: 2.8). Only one patient, treated with trametinib for PWS, did not experience a cutaneous side effect. The average number of interventions (new medication or over-the-counter product) recommended to prevent or treat cutaneous-related toxicities was 6.8 per person, the majority of those were for acne and paronychia. Over 75% of the interventions were topical. Of those who experienced an acneiform rash, the average number of recommended interventions were 3.1. Sixty two percent of these patients developed acne within the first cycle; average time to development was 2.6 months into therapy. Most exhibited improvement overtime following intervention. Those patients who experienced paronychia were treated with a mean of 3.1 interventions – 37.5% developed paronychia within the first cycle of therapy; average time to development was 3.9 months into therapy. CONCLUSIONS: Cutaneous side effects are common, occur early in therapy, and require multiple interventions. The number and complexity of these interventions may further