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BACKGROUND: Pineoblastoma is a malignant tumor of the pineal gland and accounts for <1% of all pediatric brain tumors. **PURPOSE/** METHODS: Patients <21 years (y) with pineoblastoma confirmed by central neuropathology review between 2001–2021 and included into the HIT2000 trial, HIT2000interim- or I-HIT-MED-registries were eligible. RESULTS: 88 patients were identified. Age at diagnosis was 0.01–20.71y (median 9.34y), median follow-up was 6.54y (IQR 1.78–12.41y) in 48 patients alive at last follow-up. 20 patients were <4y and received chemotherapy with intent to avoid radiotherapy. Of these, 7 patients were alive at last follow-up, two patients were radiotherapy-naïve and 5 patients had undergone CSI + boost (4 after incomplete response and one after progression). 5-y-PFS/OS in 68 patients >4y differed according to metastatic status (M0 (n=40) 72.7±8.3%/75.0±8.3%; M+ (n=28) 28.7±10.3%/40.8±10.9%, p=0.001/0.001). Therapy escalation in M0 patients by giving SKK chemotherapy before radiotherapy did not improve PFS/OS compared to upfront radiotherapy (5-y-PFS/OS 70.7±14.3%/70.0±14.5% vs 74.2±10.1%/78.9±9.4%, p=0.61/0.73). Applied CSI dosages were 24–50Gy (mean 35.6Gy) with no prognostic value of specific dosages being observed. Similarly, in M0 patients hyperfractionated radiotherapy (2x1.0Gy/d, total dose (TD) 36Gy, n=23) was not superior to conventional radiotherapy (1.6Gy/d, TD 35.2Gy, n=7). In all patients, favorable prognostic factors were age >4y (5-y-PFS/OS 54.1±7.0%/60.0±7.0% vs 30.0 ±10.2%/35.0±10.7%, p=0.012/0.053) and radiotherapy in primary therapy (5-y-PFS/OS 55.8±6.5%/61.4±6.4% vs 14.4±9.4%/21.4±11.0%, p<0.001/=0.003), whereas unfavorable prognosis was associated with metastatic disease (5-y-PFS/OS 33.6±9.0%/45.9±9.3% vs 58.8±7.6%/59.3±7.7%, p=0.028/0.086). **CONCLUSION:** Survival is poor in pineoblastoma patients <4y treated without radiotherapy. Unfavorable prognosis was associated with metastatic disease, especially in older children. Chemotherapy combined with CSI is effective for non-metastatic patients at age >4y. Further research will consider biological subgroups to enhance risk stratification and identify approaches for therapy improvements.

RARE-13. CLINICAL MANAGEMENT AND FUNCTIONAL AND SURVIVAL OUTCOMES IN PEDIATRIC CRANIOPHARYNGIOMA, A PATIENT AND FAMILY PERSPECTIVE

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Craniopharyngiomas are rare, histologically benign, sellar/parasellar tumors with significant tumor and therapy related morbidity and impairment in quality of life (QOL). We report survey results from patients/families affected by childhood-onset craniopharyngioma to identify opportunities for improvement in management. An anonymous REDCap survey was distributed via social media and clinic visits to patients/families of craniopharyngioma survivors. Survey questions investigated perspectives on clinical management and functional and survival outcomes at initial diagnosis and recurrence. A total of 159 patients/families completed the survey, 40% (n=64) reported craniopharyngioma recurrence. For primary craniopharyngioma, maximal safe resection was the most frequent treatment reported (n=84), followed by partial resection (n=40), radiation (n=8), biopsy (n=5), and chemotherapy (n=3). Most patients (n=120) decided on a treatment plan within one week, 63 (40%) decided in one day. For recurrent craniopharyngioma, maximal safe resection and radiation were the most frequent interventions (n=33 each), followed by partial resection (n=13), chemotherapy (n=4) and biopsy (n=2). Multiple treatment options and/or participation in a clinical trial were offered to similar numbers of patients across primary and recurrent diagnoses (~21% for each). Most recurrent craniopharyngioma patients decided on management within one week (n=43). Long term effects related to tumor and treatment were identified as the primary concern in all respondents. The most common deficits for all patients were neuro-endocrine followed by vision and neurocognition problems. Neuro-endocrine complications were self-reported as the biggest impact on QOL. Families reported that they would prefer treatment options with the potential for improved QOL, even if these options also carried an increased risk of recurrence. Craniopharyngioma continues to be predominantly treated with surgery and radiation initially and with recurrence. Survivors have multiple comorbidities, with an interest in targeted therapies that preserve QOL. Novel therapies to prevent co-morbidities and provide long term benefits are necessary and upcoming.

RARE-14. NEWBORN WITH HYPOTHALAMIC HAMARTOMA AND PALLISTER-HALL SYNDROME

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A female full-term newborn of 41 + 2 weeks gestational age with a respiratory adaptation disorder and hypercapnia was transferred from an external maternity clinic to our pediatric intensive care unit. The child is the second child of healthy, non-consanguineous parents. Multiple dysmorphias were noticed at arrival. We identified a choanal atresia/stenosis on both sides in the respiratory tract, a high palate, a submucous cleft palate, a bifid uvula, a laryngeal cleft and a bronchus suis. The child required intubation and ventilation. In addition, we recognized brachydactyly of the hands and feet. The phalanges were not visibly separable. There was nail hypoplasia and rocker bottom feet on both sides. Furthermore, we saw an anal atresia. In routine laboratory work-up, a hypoglycemia and not measurable low TSH serum concentration was noticed. Extended endocrinological laboratory diagnostics revealed a complete pituitary insufficiency. On cranial MRI, a large, iso- to slightly hyperintense space-occupying mass (3.8x3.7x2.5 cm^3), originating from the hypothalamus was observed. The brainstem was displaced posteriorly by the mass. The imaging is consistent with a hypothalamic hamartoma. With regard to the present findings, we assumed an underlying genetic cause of the congenital malformations. As a clinical diagnosis, a Pallister-Hall syndrome was suspected. As described in our case, we saw the characteristic features: dysmorphia of the hands and feet, upper respiratory tract, anal atresia, and hypothalamic hamartomas. The Pallister-Hall syndrome is caused by mutations in the GLI3 gene on the 7p13 chromosome. It is inherited in an autosomal dominant manner and its prevalence is unknown. In our patient, a heterozygous, probably pathogenic variant in the GLI3-Gene was proven by Next Generation Sequencing (NGS).

RARE-15. ASTROBLASTOMA, *MN1* ALTERED COMPRISES TWO MOLECULARLY AND CLINICALLY DISTINCT SUBGROUPS DEFINED BY THE FUSION PARTNERS *BEND2* AND *CXXC5*

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