risk of developing intraperitoneal spread of melanoma. BACKGROUND: Neurocutaneous melanocytosis, a rare condition characterized by excessive proliferation and deposition of melanocytes in the leptomeninges and brain parenchyma of children with large, facial or orbital melanocytic nevi and multiple smaller congenital nevi. These patients are at heightened risk for developing NRAS+ melanomas in the central nervous system, which in turn may lead to symptomatic hydrocephalus requiring cerebrospinal fluid shunting (VP shunt). METHODS: Retrospective single-institution study of patients with histologically or radiographically confirmed NCM evaluated at Memorial Sloan Kettering Cancer Center (MSKCC) from 2001-2022. RESULTS: Of the 47 patients with a diagnosis of NCM, 44 patients had symptomatic neurological complications. Eleven patients developed hydrocephalus. 10 had CNS melanoma, and required ventriculoperitoneal shunt placement. Nine of the 10 patients ultimately died of their disease. Three patients were diagnosed with intraperitoneal melanoma, though data are unavailable for the remaining eight. CONCLUSIONS: All (n=11) patients with CNS melanoma received b-4 therapy for symptomatic relief. Ten of these patients died within 4.3 years of VP shunt placement, with a range of 1 month to 13.5 years prior to succumbing to their disease. While the intratumoral pathology remains unknown for 7 of the cases, 3 had confirmed intraperitoneal melanoma, suggesting that VP shunts provided the conduit to CNS melanoma seeding of the peritoneum. Obtaining baseline abdominal imaging studies prior to VP shunt placement may be helpful in the follow-up of these patients.

RARE-10. TREATMENT OF CHILDHOOD-ONSET CRANIOPHYNGIOMA PATIENTS USING PROTON BEAM THERAPY VERSUS PHOTON-BASED RADIATION THERAPY IN THE PROSPECTIVE KRANIOPHYNGEOM 2007 TRIAL

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BACKGROUND: Proton beam therapy (PBT) compared to photon-based radiotherapy (XRT) offers the benefit to administer lower radiation doses to critical organs thereby possibly minimizing the risk of sequelae in patients with residual craniopharyngiomas (CP) after hypothalamo-spurring surgery. The validation in large CP patient cohorts is still pending. PATIENTS AND METHODS: Of 290 childhood-onset CP patients included 2007-2019 in the prospective multicenter trial KRANIOPHYNGEOM 2007, 99 (34%) received external RT (65% PBT, 35% XRT). Outcome was compared between the different groups in terms of clinical recovery and event-free survival. Total 25% patients completed one, three and five years after irradiation/CP diagnosis. RESULTS: PBT as well as XRT were associated with high (p<0.001) EFS (PBT: 0.917 ± 0.040; XRT: 0.940 ± 0.041) compared to non-RT (EFS: 0.669 ± 0.044). OS was similar in all groups. No differences between PBT, XRT and non-RT CP patients concerning functional capacity and auxological data (BMI and height SDS) one, three and five years after irradiation/CP diagnosis. PROTON BEAM THERAPY: Age at diagnosis was 13.1 ± 5 years. 12% patients received radiotherapy within the first and second half of the enrollment period, respectively. PBT as well as XRT were associated with high (p<0.001) EFS (PBT: 0.917 ± 0.040; XRT: 0.940 ± 0.041) compared to non-RT (EFS: 0.669 ± 0.044). OS was similar in all groups. No differences between PBT, XRT and non-RT CP patients concerning functional capacity and auxological data (BMI and height SDS) one, three and five years after irradiation/CP diagnosis. RARE-11. 60 YEARS SINGLE CENTRE EXPERIENCE OF CRANIOPHYNGIOMA MANAGEMENT

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Adamantinomatous craniopharyngiomas are challenging intracranial tumours associated with significant morbidity. Management includes surgery (and/or radiotherapy) with or without endocrine and radiotherapy, with a shift towards more conservative surgery in recent years, aimed at preserving hypothalamic function. The West Midlands Regional Children’s Tumour Registry collects detailed clinical, pathological and follow up information on patients treated within the region from 1957. 52 cases (26 male, 26 female) of craniopharyngioma treated at Birmingham Children’s Hospital 1957-2018, were identified, with further clinical details obtained from patient records, where available. Clinical data were available at last follow up, where available.

Of 52 children (median age 11 years, range 4-17 years), 17 cases (33%) had endocrine signs and symptoms at diagnosis, with 70% having growth hormone deficiency and 55% diabetes insipidus. In total, 16 patients had at least one endocrinopathy, with 38/45 patients having diabetes insipidus. 10 patients developed neurovascular complications and three fatty liver disease. Where data was available at follow up, all patients had gained weight. Where data was available 9/27 (33%) patients had developed hypothyroidism and 4/27 (15%) patients had developed diabetes insipidus. Of 15 patients followed for at least one year after surgery, 4/15 (27%) had developed significant radiological recurrence. 10 patients had undergone a second surgery. Two patients had undergone a neuroendocrine lead (one case with posterior fossa recurrence and one case with hypothalamic dysfunction). Radiotherapy was used in 14/15 patients who had not undergone surgery. 4/15 patients (27%) had developed a second recurrence after radiotherapy.

Of the 52 cases, 36 were treated with surgery and radiotherapy (33 patients) and 16 with surgery alone (9 patients). The most common primary treatment modality was surgery (31 patients), followed by surgery and radiotherapy (27 patients) and 2 patients treated with radiotherapy alone. Of the 10 patients who had developed a recurrence after primary treatment, 6 patients were treated with additional radiotherapy and 4 patients were treated with additional surgery. Of the patients treated with radiotherapy, 1 patient had developed a second recurrence and was treated with additional surgery.

Of the 36 patients with primary treatment modality surgery and radiotherapy, 27 patients had developed a recurrence after primary treatment and were treated with additional surgery (27 patients) and 6 patients with additional radiotherapy (6 patients). Of the 16 patients with surgery alone, 6 patients had developed a recurrence after primary treatment and were treated with additional radiotherapy (6 patients) and 3 patients with additional surgery (3 patients). Of the 4 patients who had initially undergone surgery and then radiotherapy, 3 patients had developed a recurrence after primary treatment and were treated with additional surgery (3 patients) and 1 patient with additional radiotherapy (1 patient). Of the 4 patients who had initially undergone radiotherapy, 3 patients had developed a recurrence after primary treatment and were treated with additional surgery (3 patients) and 1 patient with additional radiotherapy (1 patient).

Of the 52 cases, 27 cases (52%) had developed a recurrence after primary treatment and were treated with additional surgery (27 patients) and 6 patients with additional radiotherapy (6 patients). Of the 16 patients with surgery alone, 6 patients had developed a recurrence after primary treatment and were treated with additional radiotherapy (6 patients) and 3 patients with additional surgery (3 patients). Of the 4 patients who had initially undergone surgery and then radiotherapy, 3 patients had developed a recurrence after primary treatment and were treated with additional surgery (3 patients) and 1 patient with additional radiotherapy (1 patient). Of the 4 patients who had initially undergone radiotherapy, 3 patients had developed a recurrence after primary treatment and were treated with additional surgery (3 patients) and 1 patient with additional radiotherapy (1 patient).
Abstracts

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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 review between January 2001–2021 and included into the HIT2000 trial, HIT2000 interim- or HIT-MED-registry were eligible. RESULTS: 88 patients were identified. Age at diagnosis was 0.01–20.71 y (median 9.34 y), median follow-up was 6.54 y (IQR 1.78–12.41 y) in 48 patients alive at last follow-up. 20 patients were <4 y and received chemotherapy, with the aim of avoiding 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 <4 y differed according to metastatic status (M0 (n=40): 72.7±0.3%/75.6±0.3% vs. M+ (n=28): 28.7±10.3%/40.8±10.9%, p=0.001/0.001). Therapy escalation in M0 patients by giving SSK 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%/79.9±9.4%, p=0.61/0.73). Applied CSI doses were 24–50 Gy (mean 35.66 Gy) with no prognostic value of specific dosages being observed. Similarly, in M0 patients hyperfractionated radiotherapy (2×1.0 Gy/d, total dose (TD) 36 Gy, n=23) was not superior to conventional radiotherapy (TD 30 Gy, n=21). In all patients, prognostically significant age >4 y (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±6.3%/61.4±6.4% vs. 14.4±9.4%/21.4±11.0%, p=0.001/0.003), whereas unfavorable prognostic factor was associated with metastatic disease at diagnosis (5-y-PFS/OS 35.6±9.0%/45.9±9.3% vs. 58.8±6.7%/59.3±7.7%, p=0.028/0.086). CONCLUSION: Survival is poor in pineoblastoma patients <4 y treated without radiotherapy. Unfavorable prognosis was associated with metastatic disease at diagnosis especially in older children. Children younger than 4 y are still at risk for non-metastatic patients at age >4 y. Further research will consider biological subgroups to inform 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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PURPOSE/BACKGROUND: Children diagnosed with craniopharyngioma represent a high-risk group, with possible functional and academic impairments, predominantly treated with surgery and radiation initially and with recurrence. The most common deficits for all patients were neuro-endocrine followed by motor and cognitive impairments. The aim of this study was to evaluate the long-term outcomes for children with craniopharyngioma.

METHODS/MATERIALS: Patients diagnosed with craniopharyngioma from 1999–2020 were included. The most common treatments were surgery and radiation, and progression to chemotherapy if patients progressed to radiation. The patients examined were affected by the disease for at least 3 y, with a range of 3–24 y (mean 9.64 y). The outcomes assessed were academic, functional, and QOL. These data were collected from 2020–2021.

RESULTS: Ninety of 127 children were included. The median follow-up time was 10.97 y (range 4–24 y). Eighty-five patients were diagnosed with a non-recurrent craniopharyngioma, maximal safe resection and radiation were the most frequent treatments (n=84), followed by partial resection (n=40), radiation (n=8), biopsy (n=3), and chemotherapy (n=3). Most patients (n=120) 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. Responses to treatment improvements.

CONCLUSION: The long-term management of pediatric craniopharyngioma is characterized by a high rate of recurrence due to the tumor's invasive nature. Despite initial treatment, the recurrence rate is high, and long-term outcomes are often compromised. The results of this study highlight the need for continued research into more effective treatment strategies, with a focus on preserving quality of life (QOL) and minimizing long-term adverse effects. Further research is needed to develop novel therapeutic approaches that can improve outcomes and enhance QOL for children affected by craniopharyngioma.