(such as histone modification, including PHF6, CTCF, EED, EZH2, SUZ12, and SETD2), and signaling genes (such as activation JAK-STAT, IL-7R and RAS signaling pathway, including JAK1, JAK3, IL7R, SH2B3, NRAS, KRAS, FLT3, NF1, and PTPN11). ETP-ALL has a lower frequency of classical T-ALL genetic alterations such as NOTCH1/FBXW7/CDKN2A mutations and a higher prevalence of FLT3, NRAS/KRAS, DNMT3A, IDH1, IDH2, JAK3, and ETV6 mutations (1,2,5). ETP-ALL was initially thought to have a poor prognosis, but the opinions on it vary (2,3,7). ETP ALL is often corticosteroid resistant and a high percentage of ETP ALL patients have detectable MRD at day 29 including many induction failures (1,2,7,8). No difference in OS was observed in the COG AALL0434 study and UKALL 2003 trial between the patients with ETP-ALL and typical T-ALL (4,9). Therefore, it is important to continue with conventional therapy in ETP-ALL patients who have poor end-induction response; MRD based therapeutic approach is recommanded (7,10). Patients with ETP-ALL had high risk of hematological relapse treated at St Jude Children's Research Hospital (3). For relapsed and refractory patients, the use of acute myeloid leukemia-oriented therapies such as FLAG-IDA regimen or targeted agents may be of benefit for some patients, including FLT3 inhibitors, tyrosine kinase inhibitors, BCL-2 inhibitors such as venetoclax, or JAK/STAT inhibitors in patients with JAK mutations or fusions (1,2,4,5,7).

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Sp03

SUBCUTANEOUS TREATMENT MODALITIES IN HEMOPHILIA CARE IN 2022

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Hemophilia is a rare hereditary, recessive X-linked, hemorrhagic disorder characterized by deficiency of coagulation factor VIII (hemophilia A) or IX (hemophilia B). A typical presentation of this disease is spontaneous or traumatic bleeding. Although bleeding can occur in any part of the body, the most frequently affected parts are the joints and muscles. Bleeding into the joints (hemarthrosis) can lead to stiffness, pain, swelling and severe joint damage which can cause the patient severe long-term disability and potentially death if untreated. A while ago, **prophylaxis** with **factor concentrates** started at an early age in children with severe or moderate hemophilia, has proven its efficacy over **on demand treatment** in minimizing the hemorrhagic risk and so the long-term sequelae. Subsequently, after the introduction of **extended half-life** factor concentrates, patients are living longer and ''better'' as a result of safer factor concentrates, and less treatment burden on young patients. Despite the great efforts of clinical research, until recently there were no treatments other than replacement factors. Lately, **"non-factor therapies"** gained their place in the treatment armamentarium of hemophilia. Those are medications that improve hemostasis without replacing the missing factor. These therapies are all designed to be given **subcutaneously** and at relatively **infrequent intervals** and thus reducing the treatment burden. The aim of our presentation is to shed the light on different families of "non-factor therapies": **bispecific monoclonal antibody** like **emicizumab** (approved and available to clinicians for the subcutaneous treatment of hemophilia A) and **MIM8** (under investigation), **rebalancing agents** like **fitusiran (an antithrombin inhibitor)** and the **anti-TFPI** (Tissue Factor Pathway Inhibitor) antibodies, as **marstacimab or concizumab**.

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Sp04

STEM CELL TRANSPLANTATION IN BRAIN TUMORS

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Central nervous system (CNS) tumors are the second most common pediatric malignancies after acute leukemias and are the most common pediatric solid tumors. Although cure rates have improved with numerous technical advances in multimodal therapy, the prognosis remains poor for some high-risk histological type and for patients with residual, recurrent or disseminated disease. Radiotherapy (RT) remains an integral part of treatment for childhood brain tumors; however, the profound and irreversible sequelae of brain irradiation in the younger children are now well documented. In an effort to decrease irradiation toxicity while improving survival and quality of life in these patients, high-dose chemotherapy with autologous hematopoietic stem cell transplantation (HD-CT&autoHSCT) has been incorporated in both up-front as well as recurrent therapies. In up-front treatment, it is used in patients under the age of 3 years to delay RT or not to use RT at all. It can be used tandem non-myeloablatively in patients older than 3 years of age, after dose-intensive chemotherapy, both to shorten the neutropenic period and to give more intense chemotherapy in a shorter time when compared to conventional chemotherapy treatment approaches. AutoHSCT may also be considered after a myeloablative conditioning regimen for relapsed embryonal brain tumors, as either once or tandem, in cases with good response to salvage therapy as consolidation. In this talk, the role of autoHSCT in childhood brain tumors will be discussed by giving the results from international studies.

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Sp05

TREATMENT/MANAGEMENT OF OTHER HEPATIC TUMORS

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Pediatric liver neoplasias are rare, comprising 1–4% of all solid childhood tumors. More than half of these masses are due to hepatoblastoma and hepatocellular carcinomas, making remaining various liver tumors much more rarer. *Hepatic mesenchymal hamartoma (HMH)* is a benign tumor observed in children less than five- year-old. It is located in the right lobe of the liver more often. It has a cystic structure, and AFP is generally normal or slightly elevated. The treatment is complete resection. In rare cases, HMH can transform into undifferentiated embryonal sarcoma. *Focal nodular hyperplasia* is a well-circumscribed benign lesion, which is usually discovered incidentally as well as with oral contraceptive use, postchemotherapy status, and hereditary hemorrhagic telengiectasia. Typical imaging finding is a central scar in the mass lesion, which might overlap with fibrolamellar hepatocellular carcinoma. Asymptomatic cases might be managed with an expectant approach by careful monitoring whereas symptomatic cases might be treated with surgery or ablation therapy. *Infantile hemangioma* is the most common benign hepatic tumor in infancy. Symptoms at admission may include abdominal distention due to hepatomegaly, congestive heart failure, feeding problems, anemia, thrombocytopenia and consumptive coagulopathy, jaundice. Many of these hemangiomas are discovered incidentally and are localized. In most cases, these lesions are small enough to be of no clinical significance. In severe cases, propranolol, corticosteroids, mTOR inhibitors or cytotoxic agents can be used for treatment. *Undifferentiated embryonal sarcoma of the liver (UESL)* is the third most common malignant liver tumor in children. The median age of diagnosis of UESL is 10,5 years, in comparison to that of liver rhabdomyosarcoma which is 3.6 years. UESL is an aggressive malignancy that should be treated with multimodal therapy. Most important prognostic factor is the completeness of the resection. The survival is reported to range between 70% and 90% in various case series. *Rhabdomyosarcomas* are observed in the biliary tract. The usual sign in admission is obstructive jaundice with or without abdominal mass. Treatment is multimodal therapy. With CWS protocols, the 5-year overall (OS) and event free survival (EFS) rates were 58% and 47%, respectively. *Hepatic angiosarcoma* is a rare but highly aggressive malignancy of endothelial cells, previously known as infantile hemangioendothelioma type 2. There is a girl predominance. Despite multiagent aggressive treatment approaches including liver transplantation, the overall prognosis is still poor.

Table-1-Distribution of primary liver tumors of children*

Age group	Malignant	Benign
Infants, toddlers	Hepatoblastoma (43%)	Hemangioma/vascular (14%)
	Rhabdoid tumor (<1%)	Mesenchymal hamartoma (6%)
	Malignant germ cell (<1%)	Teratoma (<1%)
School age, adolescents	Hepatocellular and transitional cell tumors (23%)	Hepatic adenoma (2%)
	Sarcomas (7%)	Focal nodular hyperplasia (2%)

* Von Schweinitz D.: Management of liver tumors in childhood. Seminar in Pediatric Surgery 2006; 15: pp. 17-24.

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