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THIRTY YEARS OF EXPERIENCE IN THE TREATMENT OF CHILDHOOD APLASTIC ANEMIA IN LITHUANIA

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Background and aims: Aplastic anemia (AA) is a rare heterogeneous disorder of hematopoietic stem cells causing pancytopenia and marrow hypoplasia. We aimed to review the epidemiology, diagnostics and treatment results of childhood AA (acquired) in Lithuania.

Methods: We performed a retrospective analysis of pediatric AA cases diagnosed and treated at the Center for Pediatric Oncology and Hematology, VULSK, in Lithuania from 1992 to 2021. Patients with AA are centralized at our institution. Data was obtained from medical records. The study period was split in two groups: 1992-2001 and 2002-2021. The reason for this distribution was the initiation of pediatric hematopoietic stem cell transplantation (HSCT) in 2002 in Lithuania.

Results: From 1992 to 2021 49 children were diagnosed and treated for acquired AA (idiopathic n = 40, following viral hepatitis n = 8 and toxic n = 1). 21 cases (43.0%) in the first period (1992-2001), and 28 cases (57.0%) in the second period (2002-2021) were included into the study. The median age at diagnosis was 9.02 years (1.9-17.8 years). 71.4% of patients died within the first period (1992-2001). They were treated with prednisolone/cyclosporin/ATG/granulocyte-colony stimulating factor/androgen. There was no opportunity to perform HSCT. The results of the second period: 71.4% of patients survived in this period. HSCT was performed on 19 patients (67.9%): 10 patients from HLA identical sibling donors; for 9 patients from HLA unrelated donors, but 4 of whom died after HSTC (2 due to GVHD, 1 – CMV infection, 1 – Zygomycosis). HSCT was performed after median 2.17 months (1-5 months) after AA diagnosis.

Conclusions: Patients' survival before treatment with HSCT era was very low – 28,6% compared with treatment with HSCT (71,4%), p = 0.02. Our results show that allogeneic HSCT is the first-line treatment for children with AA. Aplastic anemia requires bone marrow transplantation as soon as possible

Keywords: Children, Aplastic anemia, HSCT

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P15
HOSPITAL ADMISSIONS OF APLASTIC ANAEMIA: REAL WORLD EVIDENCE FROM UK CHILDREN ADMITTED FROM 2017 – 2022

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Background and aims: Aplastic anaemia is a rare, heterogenous, life-threatening haematological disorder. There is limited contemporary data on the burden of the disease within the paediatric population in the UK. Though there is no national AA registry there are datasets that capture isolated parts of the patient journey i.e. hospital admissions. We wanted to determine the hospital activity of children with aplastic anaemia.

Methods: We performed a retrospective health service evaluation to determine the demographics and number of new patients admitted to hospital each year with a diagnosis of aplastic anaemia and their treatment. The primary source for this analysis was the Hospital Episode Statistics (HES) database and patients were identified using ICD-10 coding.

Results: For the period 1st April 2017-31st March 2022, there were 125 new patients under the age of 18 years of age diagnosed with aplastic anaemia, an average of 25 new cases per year. Most patients received a diagnosis of 'AA, unspecified' – 56%. 48% of patients were female. 60% of patients were white. The ethnicity with the highest patients per 100,000 population was 'Any other Asian background' followed by 'Pakistani (Asian or Asian British)'. Twenty-five patients (20% of the whole cohort) subsequently underwent a stem cell transplant during the analysis period. 80% of transplants were allogenic peripheral blood stem cell transplant and 20% were bone marrow transplants. The mean time from diagnosis to transplant was 183 days.

Conclusions: To our knowledge, this is the first epidemiological data on AA in UK children. There are geographic variations in the way data is classified making the analysis difficult and supporting the idea that a national registry to determine incidence, prevalence and survival of children with aplastic anaemia in the UK is needed as is seen in other countries.

Keywords: Administrative database, Electronic health records, Epidemiology, Stem cell transplant, Constitutional aplastic anaemia

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P16
REFRACTORY CYTOPENIA OF CHILDHOOD: CLINICAL FEATURES, PATHOLOGIC FINDINGS AND EXPERIENCE OF BRAZILIAN COOPERATIVE GROUP OF PEDIATRIC MYELODYSPLASTIC SYNDROME

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Background and aims: Myelodysplastic Syndromes (MDS) estimated incidence is 1 to 4 cases per million, with Refractory Cytopenia of Childhood (RCC) being the most common subtype of pediatric MDS. However, it can be a challenging diagnosis. In these cases, cytogenetic alterations as well as immunophenotyping by flow cytometry contribute to diagnostic workup. Knowing the profile of these children can improve clinical reasoning, suspicion, and identification of patients. OBJECTIVE: To describe the clinical and pathological profile of patients referred to the BCG-MDS-PED with refractory cytopenia of childhood diagnosis.

Methods: Descriptive cross-sectional study with retrospective data collection from 91 children (0 to 18 years old) referred to the group from 1997 to May 2022 who were diagnosed with MDS - RCC. Statistical analyzes were performed using SPSS Software for Windows® version 21.

Results: RESULTS: We had 56% of female cases and the Caucasian ethnicity was the most prevalent (65.9%). Age ranged from 1 to 17 years, with a median of 8.9 years. Regarding clinical profile, pallor was the most common alteration, affecting 64.8% of patients and skin/mucosal bleeding was observed in 39.6%. In