Introduction: Acute lymphoblastic leukemia (ALL) is the most common pediatric malignancy, and although survival rates in children are high, adult B-lineage ALL (B-ALL) presents with high rates of relapse and refractoriness.[1,2] Chimeric antigen receptor (CAR)-T cell therapy, a form of personalized immunotherapy, has emerged as a revolutionary strategy for hematological cancers, showing transformative results where conventional treatments have failed.[3] This therapy involves genetically modifying a patient's T-cells to recognize and eliminate cancer cells.[3] Given its potential, this study aims to synthesize the current evidence on the efficacy and safety of CAR-T therapy in patients with relapsed or refractory (R/R) B-ALL. **Aim:** To evaluate and synthesize the results on the efficacy and safety of different CAR-T treatments in patients with refractory B-ALL through a systematic review and meta-analysis of published clinical trials. **Material and methods:** A systematic review and meta-analysis were conducted following PRISMA guidelines. A search of PubMed, SciELO, BVS, Cochrane Library, and Web of Science databases was performed for clinical trials published between 2014 and 2025 that evaluated CAR-T therapy for R/R B-ALL. A total of 27 single-arm, non-randomized clinical trials were included. Data on measurable residual disease negativity, cytokine-release syndrome and neurotoxicity were extracted. A meta-analysis of proportions was performed using a random-effects model to calculate pooled estimates and 95% confidence intervals. **Discussion and conclusion:** The pooled analysis showed an overall MRD-negativity rate of 78.89% (95% CI: 73.17%–84.16%). Subgroup analysis showed rates of 79.70% for anti-CD19 constructs, 83.17% for dual anti-CD19/CD22, and 56.45% for anti-CD22 alone. The incidence of CRS of any grade was 80.91% (95% CI: 74.38%–86.79%), while severe CRS (Grade ≥ 3) occurred in 14.62% (95% CI: 9.35%–20.66%) of patients. Neurotoxicity of any grade was observed in 22.66% (95% CI: 14.17%–32.27%) of patients. Heterogeneity was observed across the analyses (I^2 from 66%–87%). CAR-T cell therapy induces high rates of complete remission and MRD negativity in adult patients with R/R B-ALL, representing a major advance for this historically poor-prognosis population. However, adverse events such as CRS and neurotoxicity are frequent. The significant heterogeneity among studies highlights the influence of factors like patient characteristics, CAR-T construct, and treatment protocols on outcomes. Future research should focus on optimizing CAR-T cell persistence, overcoming resistance, and refining patient selection to maximize the curative potential of this therapy.

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ERROS INATOS DA IMUNIDADE E TCTH: URGÊNCIA, BARREIRAS E OPORTUNIDADES

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Introdução: Os erros inatos da imunidade (EII) constituem um grupo heterogêneo de doenças genéticas caracterizadas por disfunções no sistema imunológico. Em determinadas formas, especialmente nas imunodeficiências combinadas graves, o transplante de células-tronco hematopoéticas (TCTH) é a única intervenção potencialmente curativa, decisiva para a sobrevida e a qualidade de vida. **Objetivos:** Este estudo teve como objetivo descrever o perfil epidemiológico de pacientes com EII e indicação de TCTH acompanhados em um centro de referência. **Material e métodos:** Estudo retrospectivo, observacional, baseado em registros eletrônicos hospitalares e no banco nacional de receptores de medula óssea (REREME), envolvendo pacientes acompanhados entre 2011 e 2025 (14 anos) com indicação formal de TCTH. **Resultados:** Foram incluídos 42 pacientes, 76% do sexo masculino, mediana de idade de 1 ano (0–16). Desses, 57% (24) realizaram TCTH, sendo 71% (17) meninos, com mediana de idade ao procedimento de 0,7 anos (0–11) e tempo mediano de espera de 135 dias (22–1.571). As principais indicações foram SCID (17) — mutações ADA, JAK3, NOD2, ILR7 —, Síndrome de Wiskott-Aldrich (3), doença granulomatosa crônica (1), deficiência de receptor de IL-10 (1), deficiência de NFKB (1) e outras imunodeficiências combinadas (1). A mortalidade foi de 26% (6), principalmente por VOD e infecção por CMV. Entre os 43% (18) não transplantados, 5 perderam seguimento e 5 evoluíram a óbito antes da realização. Oito permaneciam em busca de doador compatível ou fora da faixa etária, com mediana de espera de 1.769 dias (67–1.862), período anterior à ampla adoção dos transplantes haploidênticos (2018) e à implantação da triagem neonatal (2021) no país. **Discussão e conclusão:** O TCTH é pilar terapêutico nos EII graves. O atraso diagnóstico e a escassez de doadores compatíveis comprometem a sobrevida. Medidas para detecção precoce e ampliação do acesso a centros especializados são cruciais para melhores desfechos.

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ESTADO NUTRICIONAL DE CRIANÇAS SUBMETIDAS A TRANSPLANTE DE CÉLULAS-TRONCO HEMATOPOIÉTICAS (TCTH)

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Introdução: O estado nutricional do paciente está diretamente ligado no tratamento proposto, dessa forma a avaliação nutricional pré e acompanhamento nutricional pós-transplante de células-tronco hematopoiéticas é essencial e influencia diretamente na qualidade de vida do paciente. **Objetivos:** Descrever o estado nutricional pré e pós TCTH prevalente de crianças submetidas a TCTH. **Material e métodos:** Incluiu-se no estudo pacientes pediátricos submetidos a transplante de células-tronco hematopoiéticas internados em um hospital particular de São Paulo. Trata-se de um estudo retrospectivo com 13 pacientes que realizaram TCTH entre janeiro a julho de 2025 com tempo médio de internação de 49 dias. Os dados foram obtidos através de prontuário eletrônico. O estado nutricional foi classificado conforme a circunferência do braço (CB) e avaliado no período pré TCTH e pós TCTH. **Resultados:** A média de idade foi 8 anos, sendo prevalente pacientes com diagnóstico prévio de meduloblastoma (30,7%), seguido por leucemia mieloide aguda (23,1%), Anemia falciforme (15,4%), anemia de Fanconi (7,7%), síndrome de Wiskott-Aldrich (7,7%), linfo-histiocitose hemofagocítica (7,7%) e mielofibrose (7,7%). Predominou-se TCTH do tipo alogênico não aparentado (46,1%) em detrimento ao alogênico aparentado (23,2%) e autólogo (30,7%). Predominou-se no período pré TMO o estado nutricional de pacientes com eutrofia (61,5%), desnutrição moderada (23,1%) e seguido por desnutrição grave (15,4%). No período pós TMO predominou-se pacientes com eutrofia (54%), desnutrição moderada (38%) e desnutrição grave (8%). Apenas 2 pacientes modificaram a classificação do estado nutricional durante o período avaliado e evoluiu com mudança de estado nutricional. Desses 2 pacientes, 1 passou do quadro de eutrofia para o quadro de desnutrição grave e 1 passou do quadro de desnutrição grave para o quadro de desnutrição moderada. **Discussão e conclusão:** A maioria dos pacientes não modificaram o estado nutricional, devido triagem nutricional precoce, acompanhamento nutricional diário durante a internação, avaliação e aconselhamento nutricional ambulatorial pré TCTH com intervenção nutricional precocemente. A maioria dos pacientes que fizeram TCTH apresentavam estado nutricional de eutrofia tanto no período pré TCTH quanto pós TCTH, sem alterações de classificação nesse período. Sugere-se uma avaliação mais ampla para detecção do real estado nutricional destes pacientes. **Palavras-chave:** Transplante de Células-Tronco Hematopoéticas; Estado nutricional; Terapia nutricional.

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EXPERIENCE WITH AUTOLOGOUS PERIPHERAL BLOOD STEM CELL (PBSC) COLLECTION AND TRANSPLANTATION (HSCT) IN CHILDREN WEIGHTING 25 KG OR LESS

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Introduction: The collection of autologous PBSC from small children may be challenging, especially to professionals proficient in adult Hematology and Hemotherapy. The reason for these autologous collections, now go beyond HSCT, also including therapy with CAR-T cells. Over 26 years of activity and over 1,000 transplants in children and adolescents, approximately 28% were performed in children weighting 25 kg or less. **Aim:** The objective of this study is to report the experience of autologous HSCT in patients weighing 25 kg or less. **Material and methods:** This is a review of the Laboratory forms and electronic medical records of all transplants performed between Dec,99 and July,25. The PBSC were collected by apheresis through a central venous catheter using the COBE Spectra® with ACD-A, 1:15 ratio, processing around 6 blood volumes. The procedures were repeated until 5×10^6 CD34+ cells/kg were collected. As of 2012, the target dose was tripled for patients undergoing tandem HSCT. The priming used irradiated and leukocyte poor red blood cells, filtered and resuspended in Normal Saline. During the apheresis, the patients received IV Na, K, Ca, Mg. As of 2016, the PBSC were collected by Spectra Optia®, ACD-A 1:12-1:15 depending on the duration of the procedure, platelet count, and outlet flow. The number of blood volumes was calculated based on the targeted CD34+ and the number on the peripheral blood on the day of the collection. The cells were cryopreserved with a final solution HAES 6%, albumin 4%, and DMSO 5%, and stored in an ultrafreezer. On the day of the infusion, a water bath 37° C was used for thawing the cells in the laboratory, where the quality control samples were drawn. DMSO was initially removed in children < 25 kg, with renal or heart failure, and infusions with > 1g/DMSO/kg using Rubinstein's protocol with a 1:1 dilution in a dextran-albumin solution 5% at 4°C. Quality control samples were obtained and it was centrifuged at 400 g, 4°C for 20 min. The buffy coat was resuspended in the same solution and sent to the floor for infusion. In 2018, patient had DMSO removed if < 15 kg and as of October 2021 weight was no longer considered a criterion, only the presence of comorbidities. **Results:** A total of 171 transplants were performed in 129 patients weighting < 25 kg: simple thawing in 65 transplants and 106 with DMSO removal. The conditioning regimens were used according to the underlying diseases: 24 Bu-Mel, 21 Carbo-Thiotepa (TT), 7 Carbo-VP-TT, 12 CEM, 62 CEM-TT, 2 CTX-Mel, 1 Temodal-TT-Carbo. No patient had graft failure and 72% are alive. **Discussion and conclusion:** Leukoaphereses in our service were performed safely, even large volume procedures. The protocol we propose for children < 25 kg include priming with packed red blood cells, infusion of electrolytes during the procedure and continuous monitoring of the patients. Autologous transplantation is safe and feasible in these children.

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