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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https://doi.org/10.1016/j.htct.2025.105506

ID - 3374

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.

https://doi.org/10.1016/j.htct.2025.105507

ID - 2229

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

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