

BLINATUMOMAB: ANALYSIS OF OVERALL SURVIVAL AND DISEASE-FREE SURVIVAL IN PEDIATRIC PATIENTS DIAGNOSED WITH RELAPSED/REFRACTORY PH-NEGATIVE B-ALL

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Objectives: To analyze the overall survival (OS) and disease-free survival (DFS) in pediatric patients diagnosed with Ph-negative B-cell Acute Lymphoblastic leukemia (B-ALL) who underwent treatment with Blinatumomab for relapse/refractory disease. **Materials and Methods:** This is a retrospective cohort study that included pediatric patients aged ≤ 18 years with Ph-negative B-ALL in first hematological remission with relapsed/refractory, treated at the Hospital da Criança de Brasília José Alencar (HCB) between 2017 and 2024. Clinical, pathological, and therapeutic data were collected. The analysis of OS and DFS was performed using the Kaplan-Meier method, considering the period until death as OS or until relapse/death as DFS, from the diagnosis or initiation of treatment with Blinatumomab. Fisher's exact test was used to evaluate the association between general data and outcomes, considering the small sample size. The study was approved by the hospital's Research Ethics Committee [4.825.280]. **Results:** 16 patients with B-ALL were included in the study, with 10 (62.5%) were male, with a mean diagnosis age of 6.81 years. The probability of overall survival was 105.20 months (median = 118.00). The average time to death from the use of Blinatumomab was 55.80 months, with a final cumulative survival of 0.63. For patients with pre-Blinatumomab positive minimal residual disease (MRD), the average time to death was 34.54 months. For patients with negative MRD, it was not possible to calculate survival due to the absence of deaths. The final cumulative survival for pre-Blinatumomab MRD was 0.53. After treatment, the average time to death was 54.00 months for positive MRD and 32.50 months for negative MRD, with cumulative survivals of 0.87 (positive) and 0.57 (negative), respectively. The estimated probability of DFS, from diagnosis to relapse/death, was 72.86 months (median = 75.00). The estimated mean duration from the start of Blinatumomab was 22.28 months (median = 25.00), with a final cumulative survival of 0.25 for pre-Blinatumomab MRD, the mean time to death was 25.84 months, and for negative MRD it was 70 months. After treatment, the mean duration was 54.00 months for positive MRD and 32.50 months for negative MRD, with cumulative survivals of 0.87 (positive) and 0.57 (negative). The Log Rank (Mantel-Cox) test did not find a significant difference between pre ($p = 0.12$) and post ($p = 0.51$) Blinatumomab survival curves. The Fisher's Exact test was used to evaluate the association between general data and outcomes; considering the small sample size, no quantitative variable showed significant differences. At the end of the study, 7 patients (43.75%) were in remission, 6 (37.5%) were in relapse, and 3 (18.75%) died. The treatment of one patient was

discontinued due to neurotoxicity. **Discussion:** Other studies have already demonstrated promising results for pediatric patients with relapsed/refractory B-ALL, such as long-term OS improvement and better response rates for negative MRD, supporting the findings of this study. **Conclusion:** The inclusion of Blinatumomab in new therapeutic regimens for Ph-negative B-ALL is a promising approach to achieving better outcomes in patients with poor prognoses. However, it is important to note that neurotoxicity may be a significant adverse event associated with the use of Blinatumomab.

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QUALIDADE DE VIDA DE CRIANÇAS E ADOLESCENTES COM HEMOFILIA EM TRATAMENTO DE PROFILAXIA, ATENDIDAS NUM CENTRO DE REFERÊNCIA DE HEMATOLOGIA DO NORDESTE BRASILEIRO

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Introdução: A hemofilia é uma doença hereditária ligada ao cromossomo X, caracterizada pela deficiência da atividade coagulante do fator VIII (hemofilia A) ou do fator IX (hemofilia B). Essa deficiência predispõe aos sangramentos espontâneos ou a consequências mais graves em caso de lesões. O tratamento profilático tem objetivo de prevenir hemartroses de repetição, que podem ocasionar deformidades funcionais permanentes, sendo o tratamento de escolha para hemofilia grave, segundo a Organização Mundial de Saúde (OMS), pois garante a integridade física, psicológica e social, melhorando a qualidade de vida (QV), que é definida pela OMS como “a percepção do indivíduo de sua posição na vida, no contexto da cultura e sistema de valores nos quais ele vive e em relação aos seus objetivos, expectativas, padrões e preocupações”. As doenças crônicas são as que mais afetam a QV porque promovem uma mudança na forma como o indivíduo se relaciona consigo e com o mundo. No caso de crianças e adolescentes com hemofilia (CACh), que adaptam sua vida em torno do tratamento, que consiste em infusões intravenosas do fator deficiente de forma frequente, por vezes diárias; atendimentos de emergência por traumas; e consultas mensais com equipe multidisciplinar, repercutindo no seu estado físico, psicossocial e cultural. **Objetivo:** Avaliar a qualidade de vida de crianças e adolescentes com hemofilia em tratamento profilático atendidas no hospital do Hemope, no período de maio a outubro de 2023. **Métodos:** : Estudo transversal, analítico e quantitativo realizado com CACh, sexo masculino, faixa etária de 8 a 16 anos, e seus genitores. A coleta de dados foi realizada com o questionário Haem-QoL, que possui doze domínios: saúde física, sentimentos, autopercepção, família, amigos, apoio, outras pessoas, esportes/escola, enfrentamento, tratamento, futuro e relacionamentos; cuja pontuação varia de zero a 100, sendo que pontuações mais altas indicam