29TH EAHP CONGRESS

CASE SERIES: REAL WORLD EFFECTIVENESS AND SAFETY OF TISAGENLECLEUCEL FOR THE TREATMENT OF B-CELL PRECURSOR ACUTE LYMPHOBLASTIC LEUKEMIA IN PEDIATRICS

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BACKGROUND AND IMPORTANCE

Tisagenlecleucel is a **CD19**-directed genetically modified autologous **T-cell immunotherapy** indicated for the treatment of B-cell precursor **acute lymphoblastic leukemia** (ALL) that is refractory or in relapse.

AIM AND OBJECTIVES

To analyze the **effectiveness** and **safety** of tisagenlecleucel in **pediatric patients** with B-ALL.

MATERIALS AND METHODS

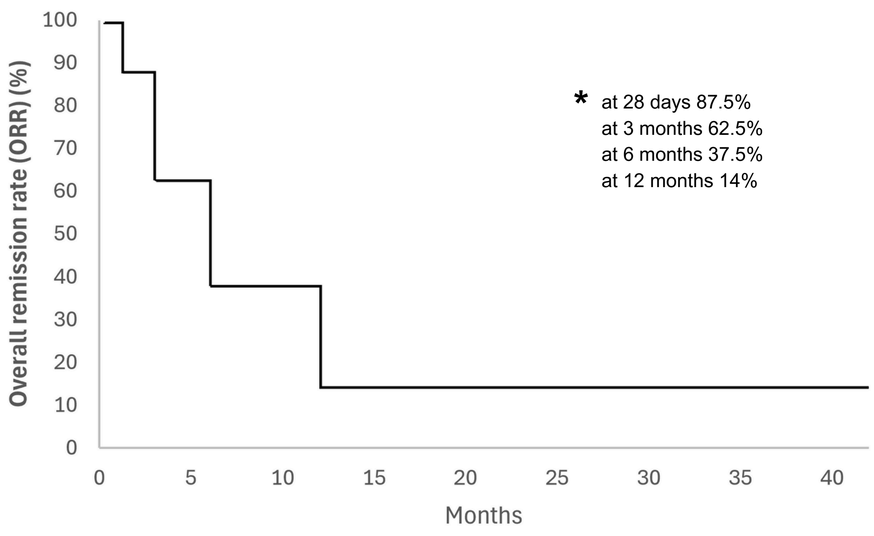
Retrospective observational study of all patients with B-ALL treated with tisagenlecleucel in our center.
Study period: 08/2019-09/2024

Variables collected
Demographic: Age, sex
Clinical: Diagnosis
Therapeutic: previous treatments
Effectiveness: overall remission rate (ORR): proportion of patients in complete remission(CR) or complete remission with incomplete blood count recovery(CRi) with minimal residual disease (MRD)<0.01% at 28 days, 3, 6 and 12 months, and median overall survival (mOS)
Safety: cytokine release syndrome (CLS), immune effector cell-associated neurotoxicity syndrome (ICANS), cytopenias, infections, macrophage activation syndrome (MAS) and hypogammaglobulinemia.

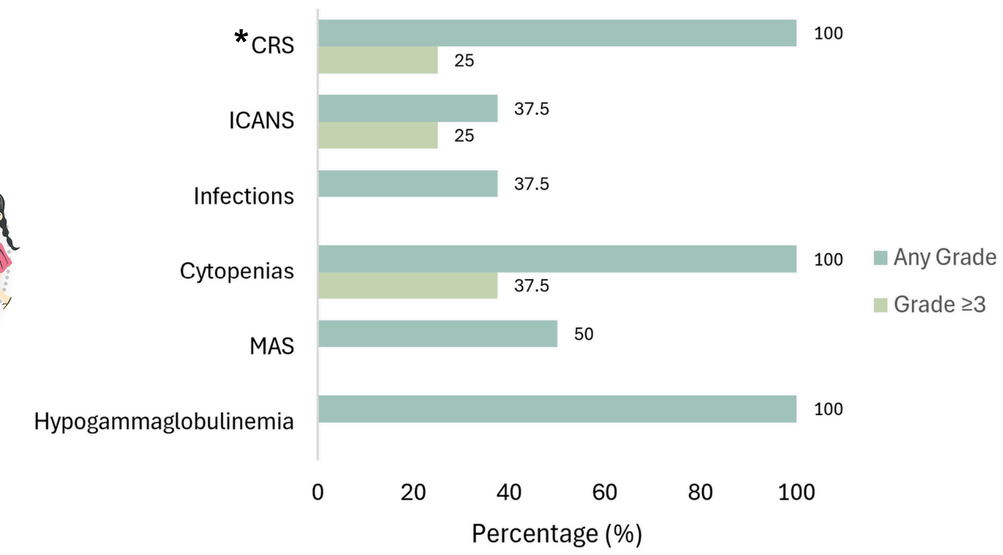
RESULTS

Eight patients, 62.5% male, median age **6 years(**3-15). **75%** with previous **allogeneic hematological transplant** and one received blinatumomab. **100% were in relapse pre-CART** receiving bridging therapy. **Pre-CART tumor load: 49.40%**(0.01-96.00). Median **follow-up: 12.36 months(**3.0-41.4).

EFFECTIVENESS



SAFETY



mOS = 11.5 months (3.0-14.7)
83.3% relapses CD19-
3 patients were still alive, one after 42 months in CR.
The rest died due to progression B-ALL.

*** Needed tocilizumab** **4 patients (50%)**
Needed tocilizumab+corticosteroids **2 patients (25%)**
Admitted to ICU **5 patients (62.5%)**

CONCLUSIONS

Tisagenlecleucel **effectiveness** in our cohort was **lower** than that observed in the pivotal clinical trial, while maintaining a **similar safety** level. The majority of **relapses are CD19 negative.** More detailed analysis of factors that could influence response is needed, as well as a larger sample size.