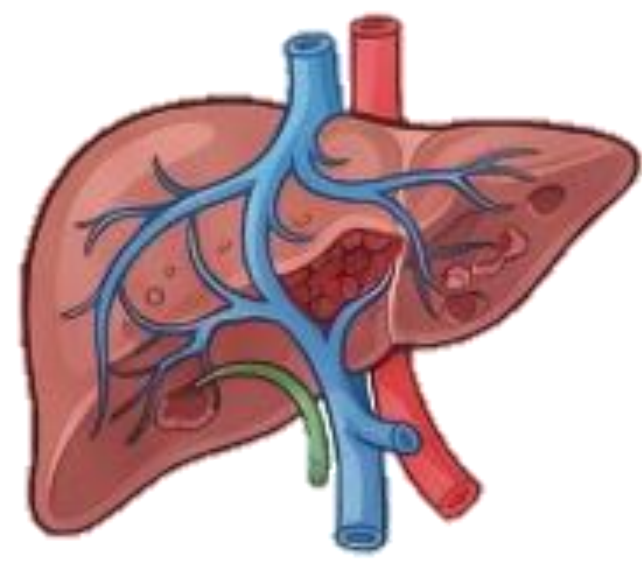


# REAL-WORLD PEDIATRIC EXPERIENCE WITH DEFIBROTIDE PROPHYLAXIS AFTER HEMATOPOIETIC CELL TRANSPLANTATION

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## BACKGROUND AND IMPORTANCE



**Sinusoidal obstruction syndrome (SOS)**  
 Potentially life-threatening complication after hematopoietic cell transplantation (HCT)



### Defibrotide

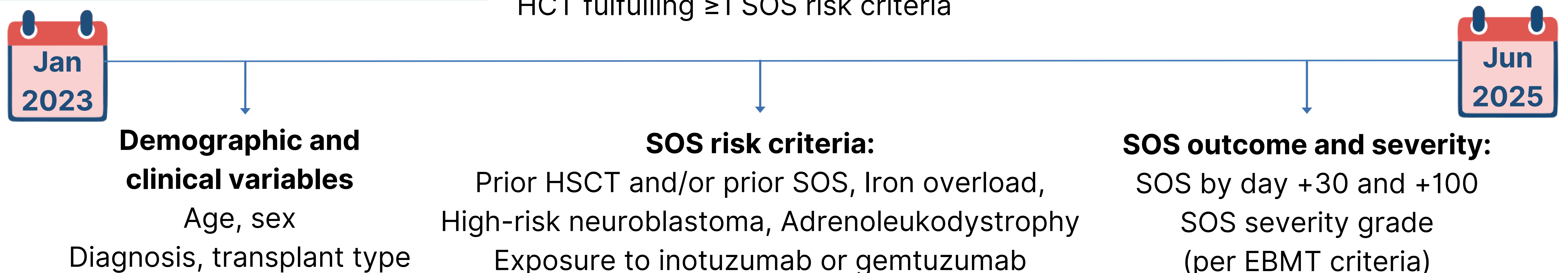
An endothelial-protective drug established for the treatment of SOS. It has been used for prophylaxis in high-risk subgroups, but its evidence remain limited and poorly defined.

## AIM AND OBJECTIVES

Describe patient characteristics and SOS outcomes by day +30 and day +100 from the date of transplantation; in a pediatric cohort meeting prespecified SOS risk criteria and receiving defibrotide prophylaxis

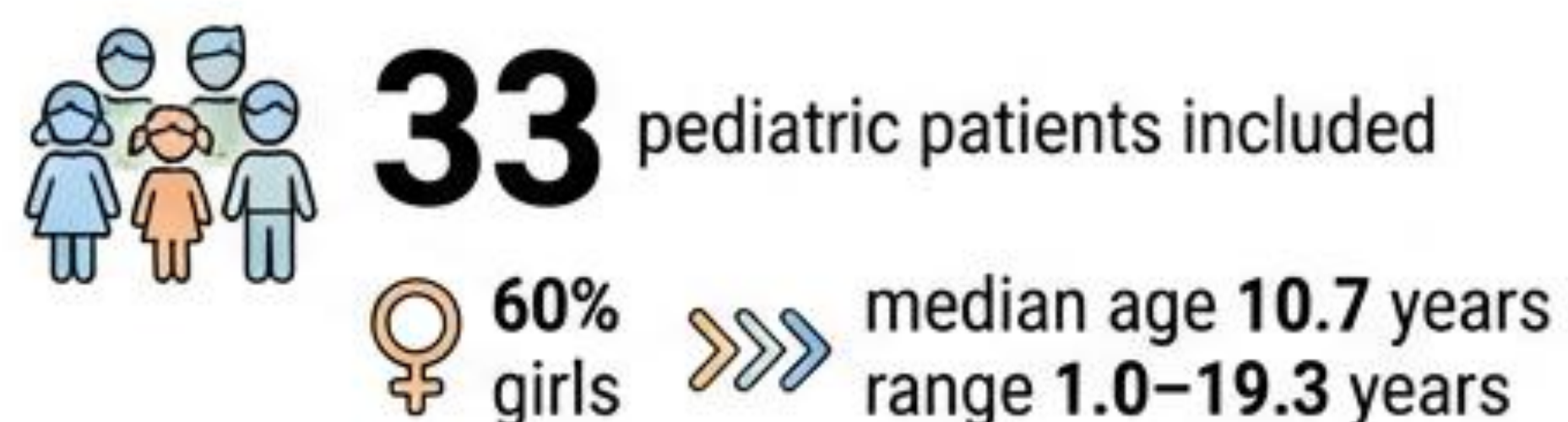
## MATERIAL AND METHODS

Retrospective, observacional study of patients undergoing HCT fulfilling  $\geq 1$  SOS risk criteria

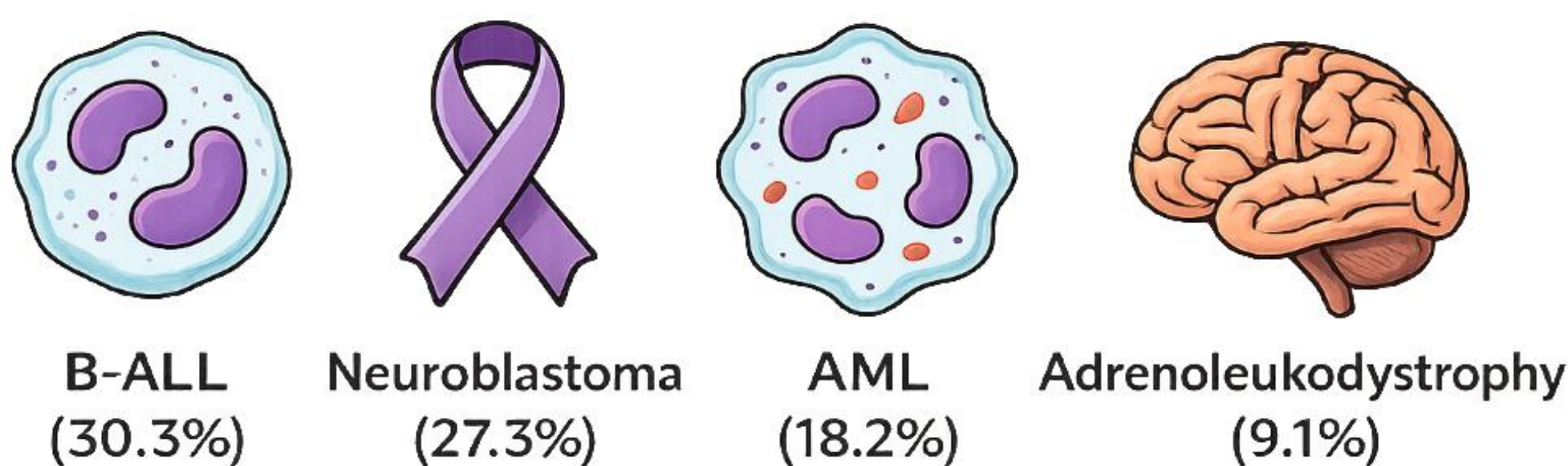


## RESULTS

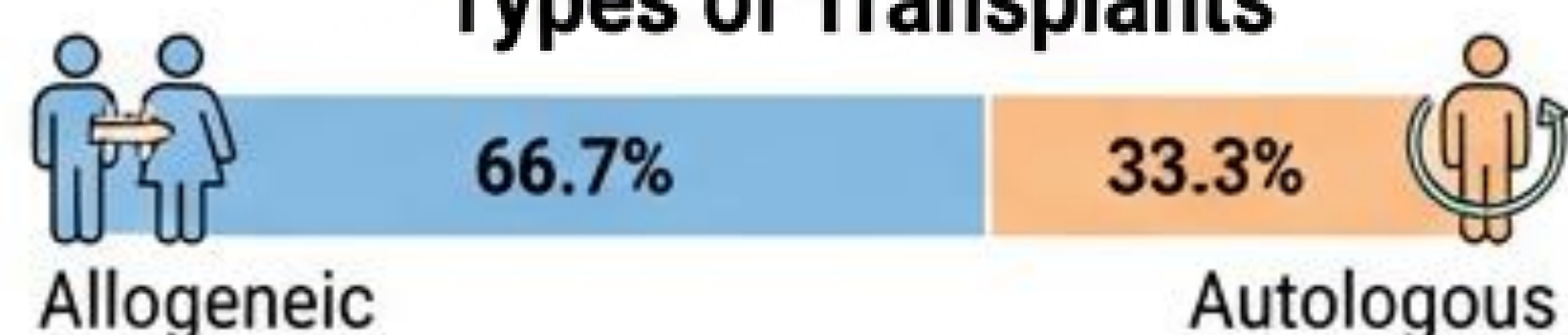
### Cohort Profile and Diagnoses



### Main Diagnoses



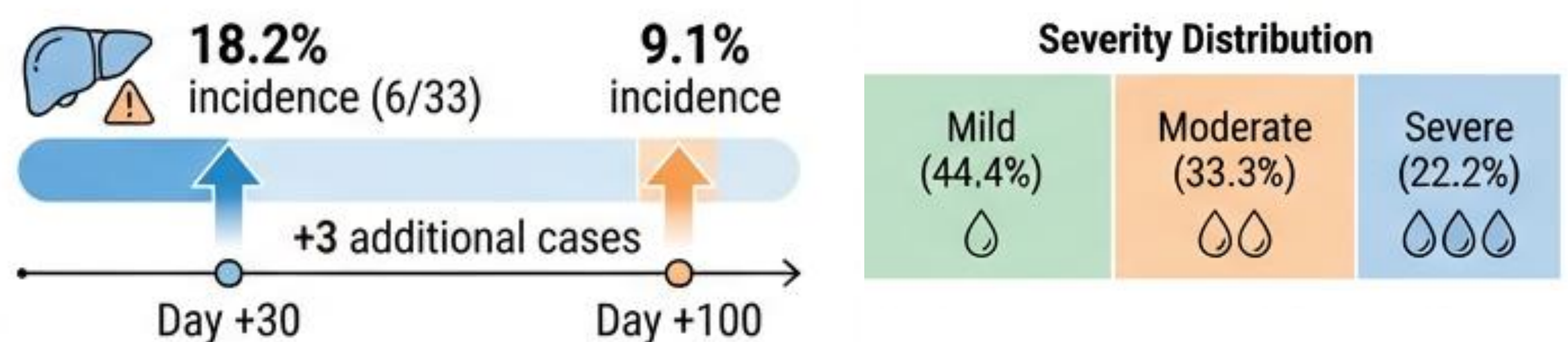
### Types of Transplants



### Prophylaxis Regimen



### SOS Incidence and Severity



### SOS by Specific Risk Factors

Risk Factor	Prevalence	SOS Cases Day +30	SOS Cases Day +100
High-Risk Neuroblastoma	30.0%	3/10	0/3
Adrenoleukodystrophy	27.3%	3/12	6/4
Prior HSCT	18.2%	3/1	0/3
Prior SOS	9.3%	1/1	1/10
Iron Overload	8.6%	1/1	1/10
Inotuzumab Exposure	7.5%	1/1	0/15
Gemtuzumab Exposure	6.9%	3/0	0/15

## CONCLUSION AND RELEVANCE

- ✓ In this real-world pediatric cohort at high risk for SOS and receiving defibrotide prophylaxis, almost a third ultimately developed SOS. While these descriptive findings cannot establish prophylactic efficacy, they help characterize outcomes across risk subgroups.
- ✓ Evidence from pediatric real-world settings is still limited and variable, underscoring the need for larger, controlled pediatric studies to define the role of defibrotide prophylaxis.

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