Access to Horse Antithymocyte Globulin Remains a Major Barrier in the Management of Severe Aplastic Anemia: A Moroccan Single-Center Experience

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INTRODUCTION

Horse antithymocyte globulin (hATG) combined with cyclosporine is the recommended first-line immunosuppressive therapy for severe acquired aplastic anemia (SAA) in patients who are not candidates for allogeneic hematopoietic stem cell transplantation. However, access to hATG remains limited in many low- and middle-income countries. We aimed to describe the real-world impact of restricted hATG availability on therapeutic practice in Morocco.

METHODS

- **Retrospective, descriptive, single-center study**
- **Study period:** January 2017 – December 2024
- **Population:** Patients with acquired aplastic anemia treated with antithymocyte globulin (ATG)
- **Center:** Department of Hematology , Cheikh Khalifa International University Hospital, Casablanca
- **Data analyzed:** Clinical characteristics, treatment modalities, adverse events, and outcomes

RESULTS

Our study included **24 patients.** The mean age was **34 years**. Female predominance (**54%**), with a M/F sex ratio of **0.85**.
All patients received **ATG, glucocorticoids, and cyclosporine A,** while **eltrombopag** was administered to **21/24 patients (87.5%).**
At diagnosis, the most common clinical presentation was a combined anemic and hemorrhagic syndrome (13/24, 54.2%), followed by isolated anemia (5/24, 20.8%)

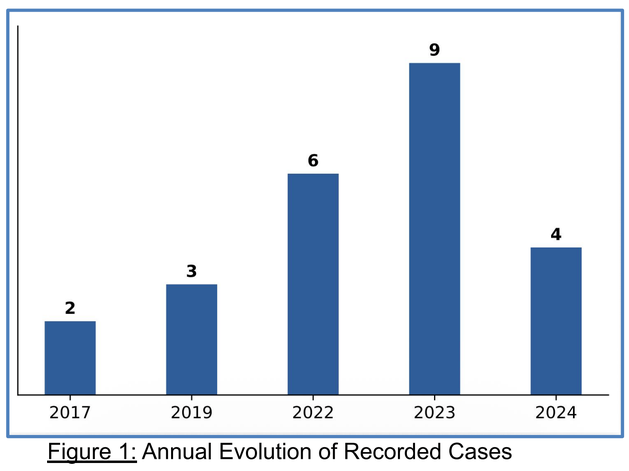

Figure 1: Annual Evolution of Recorded Cases

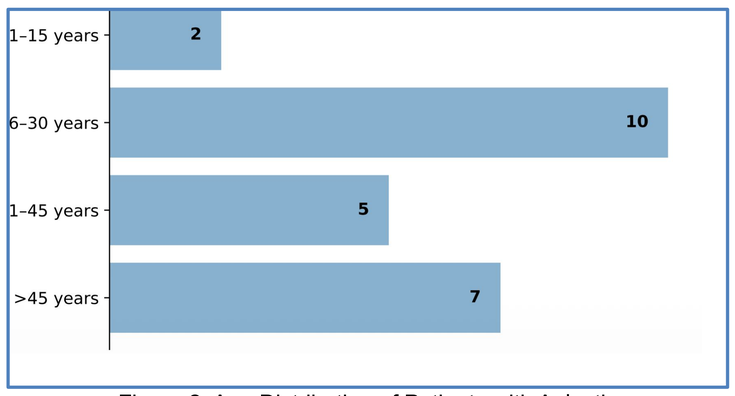

Figure 2: Age Distribution of Patients with Aplastic Anemia

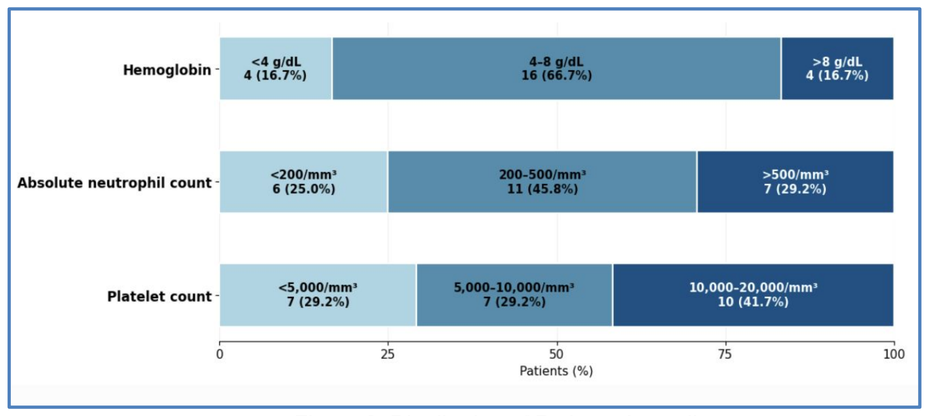

Figure 3: Distribution of Patients According to Hematological Parameters at Diagnosis

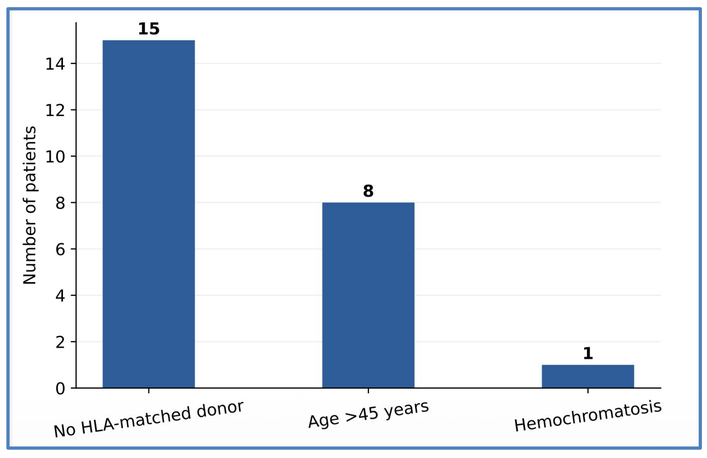

Figure 4: Indications for ATG-Based Immunosuppressive Therapy

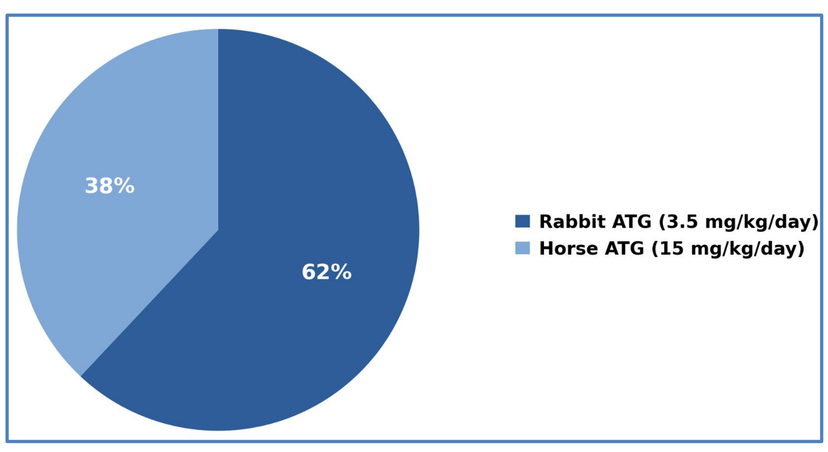

Figure 5: Type of Antithymocyte Globulin Administered

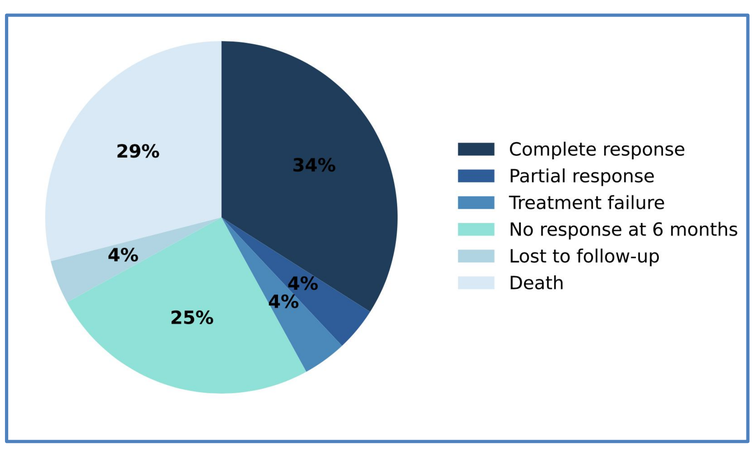

Figure 6: Long-Term Outcomes

DISCUSSION

- **Horse ATG (hATG) + cyclosporine remains the reference immunosuppressive backbone** for patients with severe aplastic anemia (SAA) who are **not** candidates for upfront matched-sibling transplantation.
- In the randomized NIH trial by **Scheinberg et al.**, hATG resulted in a significantly higher 6-month hematologic response than rabbit ATG (**68% vs 37%**) and improved overall survival (1).
- A meta-analysis including **1,636 patients** confirmed superior 6-month response rates with hATG compared with rabbit ATG, with comparable early mortality and clonal evolution (3).
- The addition of **eltrombopag** to standard immunosuppressive therapy has further improved hematologic responses, reinforcing hATG-based therapy as the preferred platform when available.
- **In Morocco, hATG is not routinely available and can only be accessed through a Temporary Authorization for Use (ATU)**, representing a **major barrier** to timely standard-of-care therapy.
- Restricted access may result in:
  - → **Treatment delays**
  - → **Use of rabbit ATG as an alternative**
  - → **Potentially compromised hematologic response and outcomes**
- Experience from other restricted-access settings suggests that **named-patient access programs can enable hATG-based therapy**, although with additional logistical constraints.
- **Our experience highlights that hATG availability is not merely a logistical issue but a determinant of treatment quality and equity in SAA. Improving access is essential to align local practice with international standards.**

CONCLUSION

Limited access to hATG substantially influences therapeutic decision-making for severe aplastic anemia in Morocco, resulting in frequent use of **rabbit ATG** despite international guideline recommendations favoring hATG. Improving **access** to guideline-recommended immunosuppressive therapy should be considered a **priority** to reduce **disparities** in care and optimize outcomes in resource-limited settings.

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