

# Is upfront unrelated stem cell transplantation in severe aplastic anemia a new standard?

No

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# Disclosures

- **Alexion, Novartis, Pfizer**
  - Consultancy
  - Honoraria
  - Research funding
- **MSD, Jazz pharmaceuticals**
  - Consultancy
  - Research funding
- **Amgen**
  - Research funding

# Why a No position while being a transplanter?

- Drastic improvement of Immunosuppressive treatment in AA in the recent period (2014 and after)
- Better long-term follow-up for responders to IST
- Excellent results of MUD in refractory patients
- Level of evidence of the data yet available
- “Technical” problems

# MUD upfront: the No position

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# Drastic improvement of IST in AA

The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

## Horse versus Rabbit Antithymocyte Globulin in Acquired Aplastic Anemia

Phillip Scheinberg, M.D., Olga Nunez, R.N., B.S.N., Barbara Weinstein, R.N.,  
Priscila Scheinberg, M.S., Angélique Biancotto, Ph.D., Colin O. Wu, Ph.D.,  
and Neal S. Young, M.D.

- Standard = horse ATG plus cyclosporine  
> 60-70% response & 30% refractory at 6 months

# Drastic improvement of IST in AA

- Horse ATG + Cyclosporine + Eltrombopag
  - Cohort 1: day 15 - 6months
  - Cohort 2: day 15 - 3 months
  - Cohort 3: day 0 – 6 months

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	Cohort 1 N=30	Cohort 2 N=31	Cohort 3 N=31
	N (%)	N (%)	N (%)
3 months			
OR	23 (77)	24 (77)	<b>23/25 (92)</b>
CR	5 (17)	8 (26)	<b>11/25 (44)</b>
6 months			
OR	24 (80)	27 (87)	<b>19/20 (95)</b>
CR	10 (33)	8 (26)	<b>12/20 (60)</b>

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# Drastic improvement of IST in AA

## RACE study



A prospective **R**andomized multicenter study comparing horse **A**ntithymocyte globuline (hATG) + **C**yclosporine A (CsA) ± **E**ltrombopag as front-line therapy for severe aplastic anemia patients.

### PRINCIPAL INVESTIGATORS

*Regis Peffault de Latour (Paris)*

*Antonio M Risitano (Naples)*

145/200 patients included; complete accrual January 2019

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# Better long-term follow-up for responders to IST

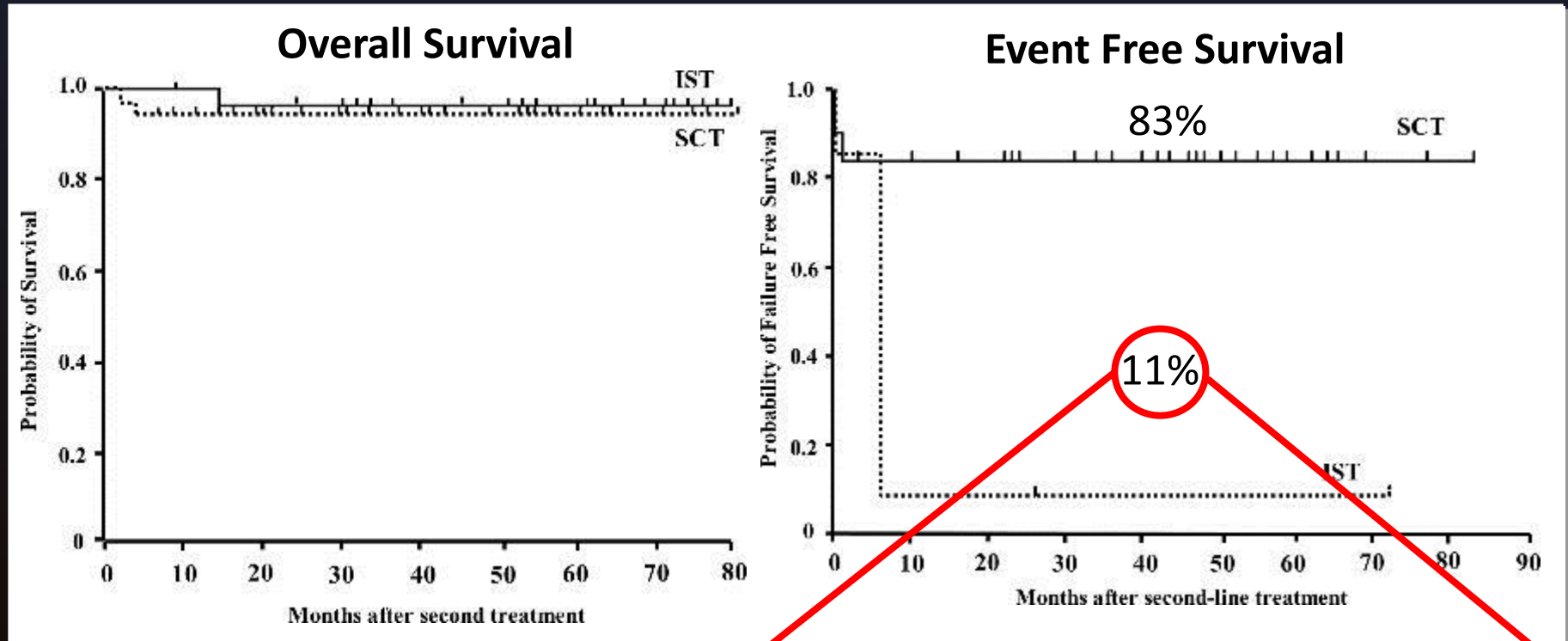
- **Responders to IST in AA**
  - >90% of patients in the Eltrombopag era (...)
  - Children are better responders
  - Better long-term overall survival
  - Less (if not) clonal evolution
    - › Paroxysmal Nocturnal hemoglobinuria
    - › Myelodysplastic syndrome
    - › Acute myeloid leukemia

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# MUD BMT is superior to IST after IST failure

Prospective multicenter trial / Refractory pediatric patients only



**Second IST and EFS**  
No response / CSA dependance / Relapse  
Clonal evolution

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# Quality of the data yet available

- **Upfront unrelated BMT (retrospective)**
  - 24 MUD, 5 MMUD; alemtuzumab (Campath)
  - Median age 8.4 years
  - 2-year OS of 96% and EFS of 92%
    - › 1 graft failure
    - › 1-year cGvHD of 19%
    - › 1 death (idiopathic pneumonia syndrome)
  - Quality of life (?)

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“technical problem” (but real life)

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  - (.../...)

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«The term “standard treatment” is generally used to describe the most commonly recommended treatment option»

*National Comprehensive Cancer Network guidelines*

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*National Cancer Institute*

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My answer is **No**

Safer to initiate MUD search at diagnosis and pursue MUD BMT at 3-6 months after IST



# Thank you!

## The French Reference Center for aplastic anemia in Paris



Saint-Louis Hospital



Robert Debré Hospital



Institute of Hematology, IUH St-Louis

F Sicre, T Leblanc, A Baruchel, G Socié  
N Vasquez, W. Cuccuini, J Soulier (Fanconi Team),  
C Kannengiesser, E Lainey, L Da Costa (Telomeres team)

