

SAMENVATTING RACE

Study Title

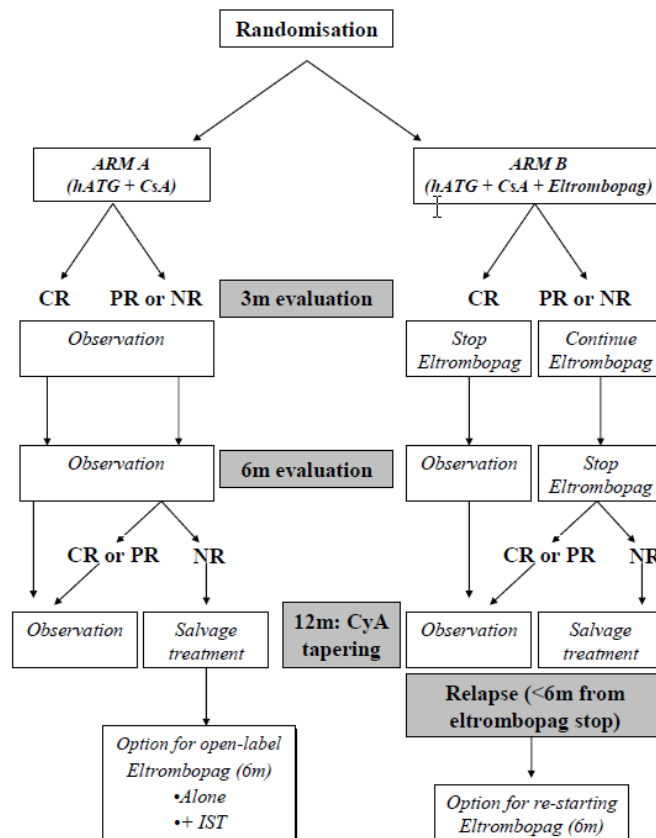
A prospective Randomized multicenter study comparing horse Antithymocyte globuline (hATG) + Cyclosporine A (CsA) with or without Eltrombopag as front-line therapy for severe aplastic anemia patients

Indication

Aplastic Anemia

Study Objectives

To increase the rate of complete response (defined as Hb >10 g/dL, ANC > 1,00/ μ L and Plt > 100,000 μ L) at 3 months in untreated severe AA patient



INCLUSIECRITERIA

1. Diagnosis of severe or very severe aplastic anemia, defined by

- At least two of the following:
 - Absolute neutrophil counts $<0.5 \times 10^9/L$ or $<0.2 \times 10^9/L$
 - Platelet counts $<20 \times 10^9/L$
 - Reticulocytin counts $60 \times 10^9/L$

- Hypocellular bone marrow (<30% cellularity), without evidence of fibrosis or malignant cells

2. Male or female ≥ 15 years

MW/KT

Samenvatting versie 2, base on protocol vs 3.1

RACE trial

06-04-2016 / 20-08-2018

EXCLUSIECRITERIA

1. prior immunosuppressive therapy with ATG, or any other lymphocyte depleting agent
2. Eligibility to a sibling allogeneic stem cell transplantation
3. Evidence of a myelodysplastic syndrome, defined by the presence of myelodysplastic features, excess of blasts or karyotype abnormalities typical of MDS (according to revised WHO 2008 criteria) should be included in this category, and are not eligible for the study; *patients with del(20q), +8 and -Y are not included in this category, and are eligible for this study.*
4. History or clinical suspicion of constitutional aplastic anemia
5. History of malignant tumors with active disease within 5 years from enrollment and/or chemo-therapy
6. Previous history of stem cell transplantation
7. Treatment with cyclosporine A < 2 weeks before enrollment
8. CMV viremia, as defined by positive PCR or pp96 test