Evaluation of the long-term effect of G-CSF added to ATG and CSA in patients with newly diagnosed severe aplastic anemia

Open-label multicenter randomized study (NCT01163942)

192 patients with newly diagnosed severe aplastic anemia (SAA), median age: 46 years (2-81)

- 97 Anti-thymocyte globuline (ATG) + Cyclosporine (CSA) with G-CSF (150 ug/m²/d from day 8-240)
- 95 ATG + CSA without G-CSF

Median follow-up: 11.7 (10.9-12.5) years

- Overall survival (OS) at 15 years
  - ALL: 60±9%
  - G-CSF: 57±12%
  - non-G-CSF: 63±12%
  - P=0.92

- Event free survival (EFS) at 15 years
  - ALL: 24±7%
  - G-CSF: 24±10%
  - non-G-CSF: 23±10%
  - P=0.36

Development of:
- Myelodysplastic syndrome or acute myeloid leukemia(×): n=9
- Clonal cytogenetic abnormality(×): n=10
- Clinical paroxysmal nocturnal hemoglobinuria: n=18
- Solid tumor: n=10
- Osteonecrosis: n=8
- Chronic kidney disease: n=12

Cumulative incidence at 15 years(×):
- G-CSF: 8.5±3%
- non-G-CSF: 8.2±3%
- P=0.90

Cumulative incidence at any late event:
- G-CSF: 50±12%
- non-G-CSF: 49±12%
- P=0.65

G-CSF unlikely impacts on the outcome of severe aplastic anemia

Tichelli et al., Haematologica, 2020