Response to immunosuppressive therapy and long-term outcomes for pediatric severe aplastic anemia

Retrospective study (data from 2002-2014)
25 institutions in the North American Pediatric Aplastic Anemia Consortium (NAPAAC)
314 pediatric patients (1-20 years) diagnosed with acquired severe aplastic anemia (SAA) and treated with immunosuppressive therapy (IST) with a minimum of two year-follow up

Treatments

- 264 Horse anti-thymocyte globulin + cyclosporine (hATG+CYA)
- 15 Horse anti-thymocyte globulin + Tacrolimus (hATG+Tacro)
- 15 Rabbit anti-thymocyte globulin (rATG) + Tacro or CYA
- 19 Cyclophosphamide (CTX)
- 1 Unspecified ATG+CYA

Median follow-up
62 months

Response to IST

- Complete response: 60.2 %, 54.5-65.7
- Deep response: 67.5 %, 62.0-72.7
- 5-year overall survival: 92 %, 88-95
- 5-year event free survival: 62 %, 56-68

All treatments

<table>
<thead>
<tr>
<th></th>
<th>95% CI</th>
<th>hATG+CYA</th>
<th>95% CI</th>
</tr>
</thead>
<tbody>
<tr>
<td>Complete response</td>
<td>54.5, 65.7</td>
<td>59.8 %</td>
<td>53.7, 65.8</td>
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<tr>
<td>Deep response</td>
<td>62.0, 72.7</td>
<td>68.2 %</td>
<td>62.2, 73.8</td>
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<td>5-year overall survival</td>
<td>88, 95</td>
<td>93 %</td>
<td>89, 96</td>
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<tr>
<td>5-year event free survival</td>
<td>56, 68</td>
<td>64 %</td>
<td>57, 69</td>
</tr>
</tbody>
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Rogers et al., Haematologica, 2019