Clinical trials with Eltrombopag as part of the initial treatment of AA in Europe

Table: Overview of the EMAA trial and RACE trial: study objective, inclusion criteria, treatment, eltrombopag dosage, design, number of patients and sponsor. For SAA Trial see separate poster

**Primary objective**
- PR + CR at 6 months
- CR at 3 months

**Inclusion criteria**
- age ≥ 18 years
  - Treatment requiring MAA (transfusion dependency or ANC < 1G/l or Thrombo < 30G/l or Hb < 8.5g/dl & Reti < 60G/l)
- No primary allo-SCT

**Treatment**
- CsA + Eltrombopag versus CsA + Placebo
- hATG (ATGAM) + CsA + Eltrombopag versus h ATG + CsA

**Eltrombopag dosage**
- 150 mg (225 mg) for moderate AA (EMAA)
- 150 mg for severe AA (EMAA)

**Design**
- Placebo controlled
- Open label

**Sponsor**
- University Hospital Ulm
- EBM

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**Numbers in Registry**

16216 patients are registered in the EBMT registry database with some type of Bone Marrow Failure. The tables below present the numbers per type of disease.

<table>
<thead>
<tr>
<th>Acquired BMF</th>
<th>n</th>
</tr>
</thead>
<tbody>
<tr>
<td>Aplastic anaemia</td>
<td>11985</td>
</tr>
<tr>
<td>Pure red cell aplasia (non congenital PRCA)</td>
<td>137</td>
</tr>
<tr>
<td>Paroxysmal nocturnal haemoglobinuria (PNH)</td>
<td>663</td>
</tr>
<tr>
<td>Pure white cell aplasia</td>
<td>12</td>
</tr>
<tr>
<td>Ameg. thrombocytopenia (non congenital)</td>
<td>54</td>
</tr>
<tr>
<td>Other</td>
<td>258</td>
</tr>
<tr>
<td>Unknown</td>
<td>115</td>
</tr>
<tr>
<td><strong>TOTAL</strong></td>
<td><strong>13224</strong></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Genetic BMF</th>
<th>n</th>
</tr>
</thead>
<tbody>
<tr>
<td>Fanconi</td>
<td>2066</td>
</tr>
<tr>
<td>Diamond-Blackfan (congenital PRCA)</td>
<td>338</td>
</tr>
<tr>
<td>Shwachman-Diamond</td>
<td>67</td>
</tr>
<tr>
<td>Dyserthropoietic anaemia</td>
<td>42</td>
</tr>
<tr>
<td>Dyserkosis congenita</td>
<td>133</td>
</tr>
<tr>
<td>Ameg. thrombocytopenia (congenital)</td>
<td>119</td>
</tr>
<tr>
<td>Congenital sideroblastic anaemia</td>
<td>20</td>
</tr>
<tr>
<td>Other</td>
<td>159</td>
</tr>
<tr>
<td>Unknown</td>
<td>43</td>
</tr>
<tr>
<td><strong>TOTAL</strong></td>
<td><strong>2992</strong></td>
</tr>
</tbody>
</table>

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**Call for Data!**

**ATG vs Campath in Fanconi – S. Samarasinghe (London, UK)**
- Data manager: Paul Bosman
- Accrual until February 2019: N=113
- Deadline: April 15th, 2019

**Partial Remission after IST – A. Rovo (Bern, Switzerland)**
- Data manager: Paul Bosman
- Accrual until February 2019: n=19
- Deadline: April 15th, 2019

**HAAA after liver TX – A. Mohseny (Leiden, The Netherlands)**
- Data manager: Paul Bosman
- Accrual until February 2019: N=7

**HSCT for Diamond Blackfan Anaemia – M. Miano (Genova, Italy)**
- Study preparation nearing completion

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**Publications 2018/2019**


Rice, C., HSCT in patients aged 50 years or older with severe Aplastic Anemia. BMT 2018 September


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**Save the date:**

Meeting SAAWP
November, 2019
Genova, Italy

For participation in, or information on SAAWP studies, please contact the EBMT Data Office in Leiden, The Netherlands:
SAAwpEBMT@lumc.nl