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.

<table>
<thead>
<tr>
<th>Sponsor</th>
<th>Primary objective</th>
<th>Inclusion criteria</th>
<th>Treatment</th>
<th>Eltrombopag dosage</th>
<th>Design</th>
<th># Patients</th>
<th>Sponsor</th>
</tr>
</thead>
<tbody>
<tr>
<td>University Hospital Ulm</td>
<td>PR + CR at 6 months</td>
<td>age ≥ 18 years</td>
<td>CsA + Eltrombopag vs CsA + Placebo</td>
<td>150 mg (225 mg)</td>
<td>Placebo controlled</td>
<td>2 x 58</td>
<td>EMAA</td>
</tr>
<tr>
<td></td>
<td>CR at 3 months</td>
<td>age ≥ 15 years</td>
<td>MTG (ATGAM) + Eltrombopag vs hATG + CsA</td>
<td>150 mg</td>
<td>Open label</td>
<td>2 x 100</td>
<td>EBMT</td>
</tr>
</tbody>
</table>

RACE trial

Actual and Expected Accrual RACE trial

Data up to and Including February 2017

![Graph showing actual and expected accrual](image)

Figure: Actual and expected accrual RACE trial from July 2015 till July 2018 (last update: March 1st 2017). For more information see the RACE trial poster.

EMAA trial

![Diagram showing EMAA trial](image)

Figure: study schedule EMAA trial.

Severe Aplastic Anemia Working Party

Chair: Carlo Dufour (Genova)

Secretary: Régis Peffault de Latour (Paris)

Study coordination: Cora Knol, Paul Bosman and Nelleke van ‘t Veer (Leiden Data Office)

Numbers in registry

14,518 patients are registered with some type of Bone Marrow Failure in the EBMT registry. The tables below present some numbers per type of disease.

<table>
<thead>
<tr>
<th>Acquired BM failure syndrome</th>
<th>n</th>
</tr>
</thead>
<tbody>
<tr>
<td>Aplastic anaemia</td>
<td>10,749</td>
</tr>
<tr>
<td>Pure red cell aplasia (non congenital PRCA)</td>
<td>129</td>
</tr>
<tr>
<td>Paroxysmal nocturnal haemoglobinuria (PNH)</td>
<td>600</td>
</tr>
<tr>
<td>Pure white cell aplasia</td>
<td>11</td>
</tr>
<tr>
<td>Ameg. thrombocytopenia (non congenital)</td>
<td>48</td>
</tr>
<tr>
<td>Other</td>
<td>238</td>
</tr>
<tr>
<td>Unknown</td>
<td>122</td>
</tr>
<tr>
<td>TOTAL</td>
<td>11,897</td>
</tr>
</tbody>
</table>

Shwachman-Diamond and Eltrombopag

SDS : SAAWP-EBMT retrospective analysis of characteristics and outcome of patients affected by Shwachman-Diamond disease (SDS) who underwent HSCT

Pt: Simone Cesaro, Verona, Italy

DM: Paul Bosman, EBMT Data Office, Leiden

Actual accrual till March 2017: N= 28

Eltrombopag: to investigate the safety and efficacy of Eltrombopag in patients with SAA receiving the drug for initial immunosuppressive treatment or for incomplete response or to treat relapse.

Pt: Jakob Passweg, Basel, Switzerland

DM: Paul Bosman, EBMT Data Office, Leiden

Actual accrual till March 2017: N= 34

Publications 2016


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SAAWP Data Office

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