Romiplostim for Thrombocytopenia Following Allogeneic Stem Cell Transplantation: A Case Series

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Introduction
• Thrombocytopenia is a relatively common complication following allogeneic hematopoietic stem cell transplantation
• It is associated with increased bleeding, transfusion requirements, chronic graft-versus-host disease and all-cause mortality
• Currently no approved treatments outside of supportive transfusions
• We report on the outcomes of 5 patients who received romiplostim for either primary engraftment failure or secondary failure of platelet recovery (SFPR)

Methods
• Retrospective case series of post-HSCT patients at our institution with either primary platelet engraftment failure or SFPR who were treated with romiplostim
  - 5 patients identified
  - Dose was 1mcg/kg, increased by 1mcg/kg per week at physician’s discretion
  - Response to romiplostim and platelet recovery were defined as 7 consecutive days of platelet count > 50 x 10^9/L with transfusion independence

Results
• 4 out of the 5 patients demonstrated a response to romiplostim
• Responses to romiplostim were sustained in the absence of significant bone marrow disease, which was found to contribute to recurrent thrombocytopenia
• Romiplostim was well-tolerated overall; one patient developed minimal fibrotic changes on bone marrow biopsy post-romiplostim

Table 1: Patient Characteristics and Response to Romiplostim

<table>
<thead>
<tr>
<th>Patient</th>
<th>Age/Gender</th>
<th>Indication for Transplant</th>
<th>Donor Source/Match</th>
<th>Primary Failure or SFPR</th>
<th># RPM doses</th>
<th>Time to Response (days)</th>
<th>Duration of Response (days)</th>
<th>Complications</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>41/M</td>
<td>Ph+ B-ALL</td>
<td>dUCB: 4/6, 4/6</td>
<td>SFPR</td>
<td>16</td>
<td>42</td>
<td>22</td>
<td>No</td>
</tr>
<tr>
<td>2</td>
<td>39/M</td>
<td>Aplastic anemia</td>
<td>MRD, 10/10</td>
<td>Primary</td>
<td>17</td>
<td>n/a</td>
<td>n/a</td>
<td>No</td>
</tr>
<tr>
<td>3</td>
<td>41/F</td>
<td>AML, MS</td>
<td>dUCB: 4/6, 4/6</td>
<td>Primary</td>
<td>8</td>
<td>27</td>
<td>407 (ongoing)</td>
<td>Focal 0-1+ fibrosis on BMAs</td>
</tr>
<tr>
<td>4</td>
<td>39/M</td>
<td>B-ALL</td>
<td>dUCB: 5/6, 6/6</td>
<td>SFPR</td>
<td>7</td>
<td>14</td>
<td>104</td>
<td>No</td>
</tr>
<tr>
<td>5</td>
<td>35/F</td>
<td>B-ALL</td>
<td>dUCB: 4/6, 4/6</td>
<td>Primary</td>
<td>10</td>
<td>58</td>
<td>404</td>
<td>No</td>
</tr>
</tbody>
</table>

Discussion/Conclusions
• Our series adds to the generally positive literature regarding romiplostim in this setting, as 4 out of the 5 patients demonstrated a platelet response
• Response was seen in both primary engraftment failure and SFPR; however romiplostim was not effective in the setting of significant bone marrow disease
• Although well-tolerated in this series, romiplostim needs to be evaluated in a randomized clinical trial in this setting to fully assess safety and efficacy