Outline

• What is MDS?
  • When do we consider growth factors?

• G-CSF / GM-CSF

• Erythropoietin-stimulating agents (with or without G-CSF)

• Anabolic steroids / steroid derivatives

• Thrombopoietin receptor agonists
MDS Formation & Progression

WHO 2016 Classification

<table>
<thead>
<tr>
<th>Name</th>
<th>Dysplastic lineages</th>
<th>Cytopenias*</th>
<th>Ring sideroblasts as % of marrow erythroid elements</th>
<th>BM and PB blasts</th>
<th>Cytogenetics by conventional karyotype analysis</th>
</tr>
</thead>
<tbody>
<tr>
<td>MDS with single lineage dysplasia</td>
<td>1</td>
<td>1 or 2</td>
<td>&lt;15%/&lt;5%†</td>
<td>BM &lt;5%, PB &lt;1%, no Auer rods</td>
<td>Any, unless fulfills all criteria for MDS with isolated del(5q)</td>
</tr>
<tr>
<td>(MDS-SLD)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>MDS with multilineage dysplasia</td>
<td>2 or 3</td>
<td>1-3</td>
<td>&lt;15%/&lt;5%†</td>
<td>BM &lt;5%, PB &lt;1%, no Auer rods</td>
<td>Any, unless fulfills all criteria for MDS with isolated del(5q)</td>
</tr>
<tr>
<td>(MDS-MLD)</td>
<td></td>
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<td></td>
<td></td>
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<tr>
<td>MDS with ring sideroblasts</td>
<td></td>
<td></td>
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<td></td>
<td></td>
</tr>
<tr>
<td>(MDS-RS)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>MDS-RS with single lineage dysplasia</td>
<td>1</td>
<td>1 or 2</td>
<td>≥15%/≥5%†</td>
<td>BM &lt;5%, PB &lt;1%, no Auer rods</td>
<td>Any, unless fulfills all criteria for MDS with isolated del(5q)</td>
</tr>
<tr>
<td>(MDS-RS-SLD)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>MDS-RS with multilineage dysplasia</td>
<td>2 or 3</td>
<td>1-3</td>
<td>≥15%/≥5%†</td>
<td>BM &lt;5%, PB &lt;1%, no Auer rods</td>
<td>Any, unless fulfills all criteria for MDS with isolated del(5q)</td>
</tr>
<tr>
<td>(MDS-RS-MLD)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>MDS with isolated del(5q)</td>
<td>1-3</td>
<td>1-2</td>
<td>None or any</td>
<td>BM &lt;5%, PB &lt;1%, no Auer rods</td>
<td>del(5q) alone or with 1 additional abnormality except −7 or del (7q)</td>
</tr>
<tr>
<td>MDS with excess blasts</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>(MDS-EB)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>MDS-EB-1</td>
<td>0-3</td>
<td>1-3</td>
<td>None or any</td>
<td>BM 5%-9% or PB 2%-4%, no Auer rods</td>
<td>Any</td>
</tr>
<tr>
<td>MDS-EB-2</td>
<td>0-3</td>
<td>1-3</td>
<td>None or any</td>
<td>BM 10%-19% or PB 5%-19% or Auer rods</td>
<td>Any</td>
</tr>
<tr>
<td>MDS, unclassifiable (MDS-U)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>with 1% blood blasts</td>
<td>1-3</td>
<td>1-3</td>
<td>None or any</td>
<td>BM &lt;5%, PB = 1%, no Auer rods</td>
<td>Any</td>
</tr>
<tr>
<td>with single lineage dysplasia and pancytopenia</td>
<td>1</td>
<td>3</td>
<td>None or any</td>
<td>BM &lt;5%, PB &lt;1%, no Auer rods</td>
<td>Any</td>
</tr>
<tr>
<td>based on defining cytogenetic abnormality</td>
<td>0</td>
<td>1-3</td>
<td>&lt;15%§</td>
<td>BM &lt;5%, PB &lt;1%, no Auer rods</td>
<td>MDS-defining abnormality</td>
</tr>
<tr>
<td>Refractory cytopenia of childhood</td>
<td>1-3</td>
<td>1-3</td>
<td>None or any</td>
<td>BM &lt;5%, PB &lt;2%</td>
<td>Any</td>
</tr>
</tbody>
</table>

IPSS-R: risk stratification

Risk Factors

Cytogenetic risk

Marrow blasts

Hemoglobin

Platelets

Neutrophils

Treatment Patterns in MDS

- 5,162 MDS patients from EMR databases of community oncologists between 2006-2014

Scott BS, et al. Submission: ASH 2016 abstract
G-CSF / GM-CSF

What Lemmings Believe
102 patients with MDS-RAEB or RAEB-T and neutropenia

<table>
<thead>
<tr>
<th></th>
<th>G-CSF</th>
<th>Observation</th>
</tr>
</thead>
<tbody>
<tr>
<td>ANC at 3 months</td>
<td>&gt;4000</td>
<td>&lt;800</td>
</tr>
<tr>
<td>AML progression</td>
<td>14% (RAEB)</td>
<td>18% (RAEB)</td>
</tr>
<tr>
<td></td>
<td>60% (RAEB-T)</td>
<td>41% (RAEB-T)</td>
</tr>
<tr>
<td>Median OS (months)</td>
<td>10.4</td>
<td>21.4</td>
</tr>
<tr>
<td></td>
<td></td>
<td>P=0.02</td>
</tr>
</tbody>
</table>

BUT:  
1. Excess mortality in G-CSF arm was not due to AML transformation  
2. Patients in G-CSF arm had worse prognostic markers (age, % blasts)  
3. All deaths in G-CSF arm were among poor prognostic group  
4. 21 month OS in observation group was weirdly long (esp. in 1993)
Cochrane Review Data

• Searched 1950-2015, published papers and abstracts for RCTs of G-CSF / GM-CSF vs. standard care in new MDS

• 5 trials with G-CSF
  • No meta-analysis possible due to data not reported / not comparable between studies.
  • **Two studies showed improved neutropenia**

• 2 trials with GM-CSF
  • No meta-analysis possible due to data not reported / not comparable between studies.
G and Survival?

Overall Survival

1. High risk MDS randomized: 7+3 ± G-CSF \(^1\)
   \(\rightarrow\) HR 0.8 [0.44-1.47] favoring G-CSF

2. High risk MDS randomized: S-HAM ± GM-CSF\(^2\)
   \(\rightarrow\) HR 0.77 [0.32-1.86] favoring GM-CSF

3. “MDS with 10-30% blasts” randomized: LDAC + GM-CSF vs. LDAC + IL-3\(^3\)
   \(\rightarrow\) HR 0.90 [0.61-1.34] favoring GM-CSF

Cochrane did not combine studies using G-CSF and GM-CSF\(^4\), but even if they did...

2. Zwierzina et al. Leukemia 2005
Probably no effect or very small effect from G-CSF added to chemotherapy.

Buckley et al. Unpublished
Does G cause Progression?

Time to AML Progression

1. High risk MDS randomized: 7+3 ± G-CSF
   \[\rightarrow\] 1 of 33 in G-CSF arm and 2 of 31 in control arm (P=0.53)

2. MDS randomized: G-CSF vs. observation
   \[\rightarrow\] No difference in time to progression (P=0.7)

3. Intermediate risk MDS randomized: EPO ± G-CSF
   \[\rightarrow\] No difference in time to progression (P=ns)

4. High risk MDS randomized: S-HAM ± GM-CSF
   \[\rightarrow\] 7 of 59 in G-CSF arm and 8 of 59 in control arm (P=0.78)

Zwierzina et al. Leukemia 2005
Mantovani 1996 (abstract)
Does G Prevent Infection?

Infections

1. High risk MDS randomized: 7+3 ± G-CSF
   → RR 1.57 [0.65-3.80] favoring no G-CSF

2. High risk MDS randomized: S-HAM ± GM-CSF
   → RR 4.00 [0.89-18.05] favoring no GM-CSF

Cochrane did not combine studies using G-CSF and GM-CSF, but if they did...

[1] Zwierzina et al. Leukemia 2005
Growth Factors and Infection Risk

<table>
<thead>
<tr>
<th>Study</th>
<th>Factor</th>
<th>N</th>
<th>ES (95% CI)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Zwierzina</td>
<td>GM-CSF</td>
<td>118</td>
<td>4.00 (0.89, 18.05)</td>
</tr>
<tr>
<td>Ossenkoppele</td>
<td>G-CSF</td>
<td>64</td>
<td>1.57 (0.65, 3.80)</td>
</tr>
<tr>
<td>Overall</td>
<td></td>
<td></td>
<td>1.99 (0.93, 4.27)</td>
</tr>
</tbody>
</table>

(I-squared = 9.4%, p = 0.293)
Conclusion: G for MDS-related neutropenia

- **Treat underlying disease** (HCT, HMAs, etc.)
- G-CSF not routinely recommended for neutropenic prophylaxis.¹
  - May raise ANC vs. no treatment²
  - No clear impact on progression to AML²
  - No clear benefit on survival²
  - No clear benefit on infection prevention (when combined with chemo)²
  - Intermittent use in patients with severe infection and neutropenia

EPO-Stimulating Agents

“We're all out of the blood of your enemies. You'll have to settle for marmalade.”

New Yorker
Anemia? Why not transfuse?

Anemia? Why not transfuse?

Response to ESAs: Better Outcomes

Retrospective study of 543 patients with MDS getting ESA, stratified by response. Responders had:

- Decreased transfusions
- Decreased cardiac comorbidities
- Improved QOL
- Survival benefit

**ESA Response Rates**

<table>
<thead>
<tr>
<th>Study</th>
<th>Outcome</th>
<th>Responders/All patients</th>
<th>Proportion</th>
<th>95% CI</th>
</tr>
</thead>
<tbody>
<tr>
<td>Musto et al (2005)</td>
<td>IWG 2000 12 week</td>
<td>15/37</td>
<td>0.41</td>
<td>0.25 - 0.56</td>
</tr>
<tr>
<td>Stasi et al (2005)</td>
<td>IWG 2000 12 week</td>
<td>20/53</td>
<td>0.38</td>
<td>0.25 - 0.51</td>
</tr>
<tr>
<td>Mannone et al (2006)</td>
<td>IWG 2000 12 week</td>
<td>44/62</td>
<td>0.71</td>
<td>0.60 - 0.82</td>
</tr>
<tr>
<td>Gabriolove et al (2008)</td>
<td>IWG 2000 13 week</td>
<td>129/206</td>
<td>0.63</td>
<td>0.56 - 0.69</td>
</tr>
<tr>
<td>Gotlib et al (2009)</td>
<td>IWG 2000 18 week</td>
<td>16/24</td>
<td>0.67</td>
<td>0.48 - 0.86</td>
</tr>
<tr>
<td>Oliva et al (2010)</td>
<td>IWG 2000 24 week</td>
<td>29/40</td>
<td>0.72</td>
<td>0.59 - 0.86</td>
</tr>
<tr>
<td>Villegaset et al (2011)</td>
<td>IWG 2000 16 week</td>
<td>31/44</td>
<td>0.70</td>
<td>0.57 - 0.84</td>
</tr>
<tr>
<td>Kelaidi et al (2013a)</td>
<td>IWG 2000 12 week</td>
<td>57/95</td>
<td>0.60</td>
<td>0.50 - 0.70</td>
</tr>
<tr>
<td>Jang et al (2015)</td>
<td>IWG 2000 16 week</td>
<td>29/50</td>
<td>0.58</td>
<td>0.44 - 0.72</td>
</tr>
</tbody>
</table>

Test for residual heterogeneity: $P = 0.001$

Response rate (IWG 2000 criteria) for 9 studies – **lots of heterogeneity in response rate**
**Baseline EPO and Response Rates**

<table>
<thead>
<tr>
<th>Study</th>
<th>Outcome</th>
<th>EPO &lt;100 iu/l</th>
<th>Proportion</th>
</tr>
</thead>
<tbody>
<tr>
<td>Musto et al (2005)</td>
<td>12 week</td>
<td></td>
<td>0.65</td>
</tr>
<tr>
<td>Mannone et al (2006)</td>
<td>12 week</td>
<td></td>
<td>0.86</td>
</tr>
<tr>
<td>Gabrilove et al (2008)</td>
<td>13 week</td>
<td></td>
<td>0.76</td>
</tr>
<tr>
<td>Gotlib et al (2009)</td>
<td>18 week</td>
<td></td>
<td>0.78</td>
</tr>
<tr>
<td>Villegas et al (2011)</td>
<td>24 week</td>
<td></td>
<td>0.80</td>
</tr>
<tr>
<td>Jang et al (2015)</td>
<td>16 week</td>
<td></td>
<td>0.93</td>
</tr>
</tbody>
</table>

Test for residual heterogeneity: $P = 0.41$

Combined estimate for EPO <100 iu/l: 0.81

- **EPO <100 iu/l**: 81% response rate for EPO <100

<table>
<thead>
<tr>
<th>Study</th>
<th>Outcome</th>
<th>EPO &gt;200 iu/l</th>
<th>Proportion</th>
</tr>
</thead>
<tbody>
<tr>
<td>Musto et al (2005)</td>
<td>12 week</td>
<td></td>
<td>0.08</td>
</tr>
<tr>
<td>Stasi et al (2005)</td>
<td>24 week</td>
<td></td>
<td>0.12</td>
</tr>
<tr>
<td>Mannone et al (2006)</td>
<td>12 week</td>
<td></td>
<td>0.50</td>
</tr>
<tr>
<td>Gabrilove et al (2008)</td>
<td>13 week</td>
<td></td>
<td>0.31</td>
</tr>
<tr>
<td>Gotlib et al (2009)</td>
<td>18 week</td>
<td></td>
<td>0.50</td>
</tr>
<tr>
<td>Jang et al (2015)</td>
<td>16 week</td>
<td></td>
<td>0.39</td>
</tr>
</tbody>
</table>

Test for residual heterogeneity: $P = 0.01$

- **EPO >200 iu/l**: Too heterogeneous to combine... 8-50% response rate for EPO >200

Park S. BJH. 2016.
Baseline HGB and Response Rates

Response rates by baseline HGB – **higher is better**

Park S. BJH. 2016.
ESAs: Bigger Doses, More Responses

Meta-analysis of 30 studies (925 patients)\textsuperscript{1}

\begin{itemize}
  \item ESA formulation does not make a difference
  \item Note: longer duration can also yield more responses, as there is a significant difference in response rates at 12 vs. 26 weeks.\textsuperscript{2}
\end{itemize}

\textsuperscript{1} Moyo et al. Ann Hematol. 2008. 87(7):527-36
\textsuperscript{2} Terpos et al. BJH 2002. 118(1):174-80
## ESA Response Prediction*

<table>
<thead>
<tr>
<th>Patient Criteria</th>
<th>Probability of Response¹</th>
</tr>
</thead>
<tbody>
<tr>
<td>Transfusion need $&lt; 2$ U/month and serum EPO $&lt; 500$ U/L</td>
<td>74%</td>
</tr>
<tr>
<td>Only one of the above criteria</td>
<td>23%</td>
</tr>
<tr>
<td>Neither criteria</td>
<td>7%</td>
</tr>
</tbody>
</table>

*Caveats:
- Predictions based on EPO + G-CSF
- Dosing is a little impractical
  - EPO 10,000 U/day x5 days per week
  - G-CSF 75-300 mcg/day x3 days per week

---

ESAs + G-CSF: synergy?

- Theoretical basis: G-CSF may suppress apoptotic cell death of early hematopoietic progenitors

- Empirical basis: G-CSF efficacy in “EPO non-responders”
Maybe Synergy?

30 patients with MDS and anemia, randomized...

- EPO
  - 40% response
  - Cross-over for non-responders at 8 weeks

- EPO + G-CSF
  - 73% initial response
  - 44% cross-over response

** But are the 44% who crossed over responding to the addition of G-CSF, or are they LATE responders to EPO?**

Again, Maybe Synergy?

99 patients with MDS, anemia, EPO <500

Darbopoeitin 500 mcg q2wk

48% response rate at 12 weeks with DARBO

Filgrastim 300 mcg 2x/week added at 12 weeks in non-responders

Response rate (in whole population) up to 56% after G-CSF added

** Again, are we seeing an effect of G-CSF, or “late responders” to ESAs?

ESAs + G-CSF: synergy?

- **Meta-analysis**: studies of EPO monotherapy and EPO+G-/GM-CSF
- 15 studies identified, baseline characteristics similar between treatment groups

**ESAs + G-CSF: synergy?**

Overall response (white) and major response (grey)

<table>
<thead>
<tr>
<th>Treatment</th>
<th>Overall Response</th>
<th>Major Response</th>
</tr>
</thead>
<tbody>
<tr>
<td>Standard EPO</td>
<td>49.0%</td>
<td>27.2%</td>
</tr>
<tr>
<td>(N=5 studies)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Standard EPO + G</td>
<td>50.6%</td>
<td>30.5%*</td>
</tr>
<tr>
<td>(N=6 studies)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>High EPO</td>
<td>64.5%</td>
<td>44.9%</td>
</tr>
<tr>
<td>(N=4 studies)</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

IWG 2000 response criteria*

Summary on ESAs in MDS

- Response rate 40-50% in lower-risk MDS

- Better response with
  - Lower serum EPO (indicated for EPO <500 U/L)
  - Higher baseline HGB (indicated for HGB <10 g/dL)
  - <2 pRBC transfusions needed / month
  - Higher doses
  - ? Synergy with G-CSF

- Median duration of response ~2 years

- Based on clinical picture, also consider:
  - Iron deficiency: keep levels normal
  - Infection: suppression of erythropoiesis
  - PNH: especially in hypoplastic MDS
  - Progression of MDS: look at other counts, repeat marrow
Anabolic steroids / steroid derivatives

“Fill’er up with testosterone.”

New Yorker
Anabolic steroids / steroid derivatives

• Suggested to improve all blood cell lines
• Testosterone: largely negative results
• Danazol: mixed results
  – RCT 600 mg/day vs. placebo in 50 patients\(^1\)
    • Response defined as Hb >12, ANC >1500, PLT >150k
    • Response rate 26% vs. 0% regardless of MDS type
  – Many case series (n=11,202 evaluable patients)\(^2\)
    • Response rates 34% (wide range between studies)
    • May be higher in patients with PLT <50k

Danazol: Considered Use¹

- Not recommended for leukopenia / anemia
- Consider use with thrombocytopenia, particularly PLT <50k, if not a candidate for any other therapy
- Give 600 mg/day for at least 4 months
- Check LFTs

Thrombopoietin Receptor Agonists

“If you could take just one medication, which medication would it be?”

New Yorker
TPO Agonists in MDS

• 2014 meta-analysis found 5 RCTs
• 4 with romiplostim, 1 with eltrombopag
• Mostly small studies
• 3 involved another therapy in treatment and control groups (AZA, LEN, DEC)
Total bleeding events – romiplostim studies

<table>
<thead>
<tr>
<th>Study or subgroup</th>
<th>Weight</th>
<th>Risk ratio Random, 95% CI</th>
<th>Risk ratio Random, 95% CI</th>
</tr>
</thead>
<tbody>
<tr>
<td>Giagounidis et al (2014)</td>
<td>97.6%</td>
<td>0.92 [0.86, 0.99]</td>
<td></td>
</tr>
<tr>
<td>Greenberg et al (2013)</td>
<td>1.9%</td>
<td>0.88 [0.54, 1.44]</td>
<td></td>
</tr>
<tr>
<td>Wang et al (2012)</td>
<td>0.5%</td>
<td>1.32 [0.50, 3.52]</td>
<td></td>
</tr>
<tr>
<td>Total (95% CI)</td>
<td>100.0%</td>
<td>0.92 [0.86, 0.99]</td>
<td></td>
</tr>
</tbody>
</table>

Heterogeneity: Tau² = 0.00; Chi² = 0.55, df = 2 (P = 0.76); I² = 0%
Test for overall effect: Z = 2.29 (P = 0.02)

PLT transfusion rates – romiplostim studies

<table>
<thead>
<tr>
<th>Study or subgroup</th>
<th>Weight</th>
<th>Risk ratio Random, 95% CI</th>
<th>Risk ratio Random, 95% CI</th>
</tr>
</thead>
<tbody>
<tr>
<td>Giagounidis et al (2014)</td>
<td>66.3%</td>
<td>0.77 [0.67, 0.88]</td>
<td></td>
</tr>
<tr>
<td>Greenberg et al (2013)</td>
<td>27.6%</td>
<td>0.54 [0.36, 0.80]</td>
<td></td>
</tr>
<tr>
<td>Wang et al (2012)</td>
<td>6.1%</td>
<td>0.58 [0.22, 1.55]</td>
<td></td>
</tr>
<tr>
<td>Total (95% CI)</td>
<td>100.0%</td>
<td>0.69 [0.53, 0.88]</td>
<td></td>
</tr>
</tbody>
</table>

Heterogeneity: Tau² = 0.02; Chi² = 3.04, df = 2 (P = 0.22); I² = 34%
Test for overall effect: Z = 2.92 (P = 0.003)

### Progression of Disease

**Table:**

<table>
<thead>
<tr>
<th>Study or subgroup</th>
<th>Romiplostim</th>
<th>Control</th>
<th>Risk ratio</th>
<th>Risk ratio</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Events</td>
<td>Total</td>
<td>Events</td>
<td>Total</td>
</tr>
<tr>
<td>1.22.2 Romiplostim</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Giagounidis et al (2014)</td>
<td>10</td>
<td>167</td>
<td>4</td>
<td>83</td>
</tr>
<tr>
<td>Greenberg et al (2013)</td>
<td>1</td>
<td>15</td>
<td>0</td>
<td>14</td>
</tr>
<tr>
<td>Kantarjian et al (2010b)</td>
<td>2</td>
<td>27</td>
<td>1</td>
<td>13</td>
</tr>
<tr>
<td>Wang et al (2012)</td>
<td>2</td>
<td>27</td>
<td>0</td>
<td>12</td>
</tr>
<tr>
<td>Subtotal (95% CI)</td>
<td>236</td>
<td>122</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Total events</td>
<td>15</td>
<td></td>
<td>5</td>
<td></td>
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<tr>
<td>Heterogeneity: Tau² = 0.00, Chi² = 0.45, df = 3 (P = 0.93); I² = 0%</td>
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<tr>
<td>Test for overall effect: Z = 0.66 (P = 0.51)</td>
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</tbody>
</table>

**Figure:**

- **El trombopag**
  - Platzbecker et al (2013) | 5 | 9 | 3 | 49.6% | 0.93 [0.37, 2.33] |
  - Subtotal (95% CI) | 9 | 5 | 49.6% | 0.93 [0.37, 2.33] |
  - Total events | 5 | 3 | |
  - Heterogeneity: Not applicable |
  - Test for overall effect: Z = 0.16 (P = 0.87) |
  - Total (95% CI) | 245 | 127 | 100.0% | 1.12 [0.59, 2.15] |
  - Total events | 20 | 8 | |
  - Heterogeneity: Tau² = 0.00, Chi² = 0.86, df = 4 (P = 0.93); I² = 0% |
  - Test for overall effect: Z = 0.35 (P = 0.73) |
  - Test for subgroup differences: Chi² = 0.33, df = 1 (P = 0.56), I² = 0% |

Change in Marrow Blasts: eltrombopag vs. placebo

PFS: eltrombopag vs. placebo

HR = 0.66 [0.4-1.08], p=0.055

Eltrombopag group: a bit more heavily pre-treated

Control group: more poor-risk cytogenetics, more baseline transfusion dependence

Eltrombopag Efficacy

Percent of patients needing PLT transfusion each week

Take-Home Points

- **G-CSF** for neutropenia: scant evidence favoring any clinically meaningful outcome.
- **ESAs** for anemia: best evidence when baseline EPO <500 mU/mL and transfusion requirement low (<2 units / month)
- **ESA + G-CSF** for anemia: unclear evidence
- **Anabolic steroid derivatives**: can try danazol as a last resort for thrombocytopenia
- **TPO receptor agonists**: likely safe, more efficacy evidence for romiplostim
Thank you:

Bart Scott – for being the faculty mentor for this presentation

Pam Becker – for being the faculty discussant

Dan Martin & the MDS patients we’ve seen together