Specific Topics Panels: Panel 3 - Data Quality in Outcome Assessments

Mary Lin, CBER, FDA
Study flow: treatment phase

Apheresis product accepted (point of enrollment)

Apheresis

manufacture

Screening

bridging/
Lymphodepleting chemotherapy (optional)

infusion

Final product release
Subject disposition

<table>
<thead>
<tr>
<th>Screened</th>
<th>107</th>
</tr>
</thead>
<tbody>
<tr>
<td>Not meet eligibility criteria</td>
<td>12</td>
</tr>
<tr>
<td>Apheresis product not accepted at manufacturing facility</td>
<td>1</td>
</tr>
<tr>
<td>Death</td>
<td>2</td>
</tr>
<tr>
<td>Screening date after data cutoff</td>
<td>4</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Enrolled</th>
<th>88 (100%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Manufacturing failure</td>
<td>8* (9%)</td>
</tr>
<tr>
<td>Death</td>
<td>9* (10%)</td>
</tr>
<tr>
<td>Adverse event</td>
<td>2 (2%)</td>
</tr>
<tr>
<td>Infusion pending</td>
<td>4 (5%)</td>
</tr>
</tbody>
</table>

| Full analysis set                             | 68        |
| Subjects treated with products manufactured in Germany | 5         |
| Efficacy analysis set                         | 63        |

* 3 subjects counted twice, they died and their product did not successfully manufactured.
Efficacy results based on different analysis population

<table>
<thead>
<tr>
<th></th>
<th>Enrolled Set (n=88)</th>
<th>Modified Enrolled Set* (n=78)</th>
<th>Efficacy analysis Set (n=63)</th>
</tr>
</thead>
<tbody>
<tr>
<td>ORR (95% CI)</td>
<td>59.1% (48.1, 69.5)</td>
<td>66.7% (55.1, 76.9)</td>
<td>82.5% (70.9, 91.0)</td>
</tr>
<tr>
<td>CR</td>
<td>45.5%</td>
<td>54.8%</td>
<td>63%</td>
</tr>
<tr>
<td>CRi</td>
<td>13.6%</td>
<td>16.4%</td>
<td>19%</td>
</tr>
</tbody>
</table>

* subjects who were enrolled and their apheresis products were accepted at the U.S. facility.
Overall Treatment Experience for each subject

- Responder
- Non-responder/unknown
- SCT
- New cancer therapy other than SCT
- Relapsed disease
- Death

Month since Kymriah infusion
Ventilation Support Hours for each subject

Age (month)

- Cohort 1 Subject 1
- Cohort 1 Subject 2
- Cohort 1 Subject 3
- Cohort 2 Subject 1
- Cohort 2 Subject 2
- Cohort 2 Subject 3
- Cohort 2 Subject 4
- Cohort 2 Subject 5
- Cohort 2 Subject 6
- Cohort 2 Subject 7
- Cohort 2 Subject 8
- Cohort 2 Subject 9
- Cohort 2 Subject 10
- Cohort 2 Subject 11
- Cohort 2 Subject 12

Age at AVXS-101 infusion
Number of hours of ventilation support per day:
- 0
- 1-4
- 5-8
- 9-12
- 13-15
- 16-20
- > 20
Developmental Milestones Achieved for each subject

Developmental Milestones achieved:
- ✷ Supine rolls over back to front
- ▲ Sits alone greater than/equal to 30 secs
- □ Walks independently
- ▲ Sits alone greater than/equal to 15 secs
- ❄ Prone: Hold head to vert axis, legs ext
- ✯ Cruises holding on may stand w/out help
- ● Runs, walks down stairs one hand held