### Baseline Demographics and Disease Characteristics: Phase 2 and Phase 3 ENGAGE

<table>
<thead>
<tr>
<th>Parameter</th>
<th>Phase 2 (%)</th>
<th>Phase 3 (%)</th>
<th>ENGAGE (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Sex, n(%)</td>
<td>Male 9 (47%)</td>
<td>Female 10 (53%)</td>
<td>10 (48%)</td>
</tr>
<tr>
<td>Ethnicity, n(%)</td>
<td>Caucasian 2 (11%)</td>
<td>Asian 6 (30%)</td>
<td>7 (33%)</td>
</tr>
<tr>
<td>Age at treatment, Mean ± SD</td>
<td>33.6 ± 12.5</td>
<td>31.8 ± 11.5</td>
<td>31.8 ± 11.5</td>
</tr>
<tr>
<td>Plasma Lyso-GL-1, ng/mL</td>
<td>687 ± 104</td>
<td>687 ± 104</td>
<td>687 ± 104</td>
</tr>
</tbody>
</table>

Mean ± SD values indicate normal range.

#### Phase 2
- 98% of adverse events were mild or moderate
- 94% of adverse events were considered unrelated to eliglustat
- 7 patients withdrew from the trial:
  - Due to pregnancy
  - Due to asymptomatic nonsustained ventricular tachycardia detected during protocol-mandated cardiac telemetry (both after a single 50-mg dose on Day 1)
  - Due to a bone lesion that was retrospectively found to have been present at baseline
- 1 for administrative reasons (protocol at study site not approved beyond 2 years)

#### ENGAGE
- 99% of adverse events were mild or moderate
- 84% of adverse events were considered unrelated to eliglustat
- 12 patients withdrew from the trial:
  - 7 when eliglustat became commercially available in the United States
  - 2 due to pregnancy
  - 3 for reasons unrelated to an adverse event

### Results

#### Phase 2: Improvements in Clinical Outcomes at 8 Years

#### Phase 3 ENGAGE: Improvements in Clinical Outcomes at 4.5 Years

#### Correlations of Lyso-GL-1 and Clinical Parameters

#### Conclusion

These data show marked elevations in lyso-GL-1 in all patients before treatment with no overlap with normal values. Lyso-GL-1 levels correlated with disease parameters both at baseline and after treatment. These findings underscore the utility of lyso-GL-1 as a clinically useful biomarker for Gaucher disease type 1.

### References


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