The Dystroglycanopathies: 2012 Patient & Family Conference

Study Participant Data
Carrie Stephan
8/17/2001

A Very Special Day
The Early Days

A Clinical Evaluation of the FKRP Muscular Dystrophies

- describe signs & symptoms
- determine reasons for differences in disease severity
Clinical Trial Readiness for the Dystroglycanopathies

• ‘Wellstone Study’
• Longitudinal design
• Conducted in preparation for a clinical trial
  – Identify patients
  – Describe signs & symptoms
  – Define natural history and refine tools
Enrollment

Gender
- Female
- Male
Ages of Participants

<table>
<thead>
<tr>
<th>Age Range</th>
<th>Count</th>
</tr>
</thead>
<tbody>
<tr>
<td>0-9 years</td>
<td>7</td>
</tr>
<tr>
<td>10-19 yrs</td>
<td>16</td>
</tr>
<tr>
<td>20-29 yrs</td>
<td>5</td>
</tr>
<tr>
<td>30-39 yrs</td>
<td>5</td>
</tr>
<tr>
<td>40+</td>
<td>3</td>
</tr>
</tbody>
</table>
Current participants, genetics

- FKRP C826A, C826A: 13
- FKRP other: 1
- FKTN: 6
- POMT2: 14
- POMGnT1: 2
- Unknown: 1
Where in the world?

• Iowa
• California
• Illinois
• Wisconsin
• Canada, Indiana, Michigan, Virginia
• Alabama, Florida, Georgia, Kansas, Nebraska, Ohio, Tennessee, Washington
• North Carolina, Texas, Rhode Island
• (+1 CA, MI, KS)