

The Dystroglycanopathies: 2013 Patient and Family Conference

Carrie Stephan

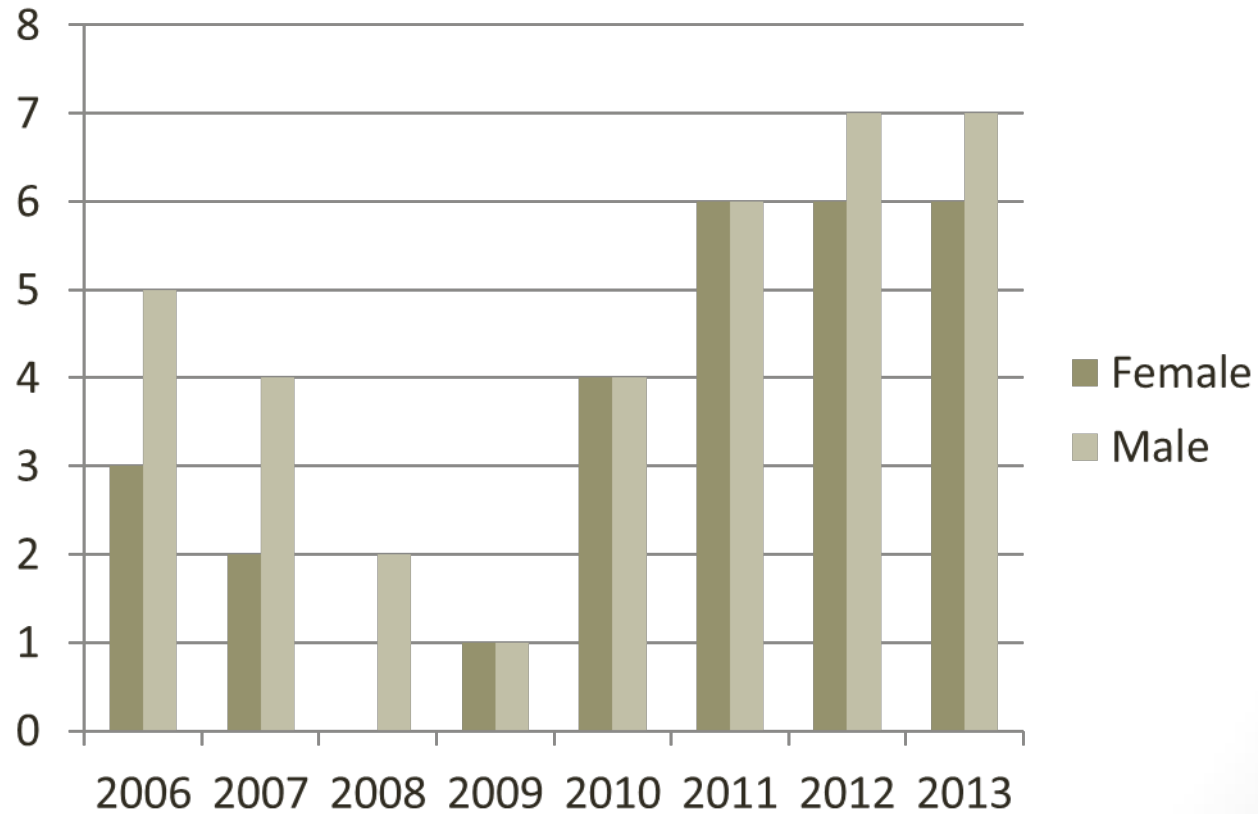
The Early Days

- A Clinical Evaluation of the FKRP Muscular Dystrophies
 - 'FKRP Study'
 - describe signs & symptoms
 - determine reasons for differences in disease severity

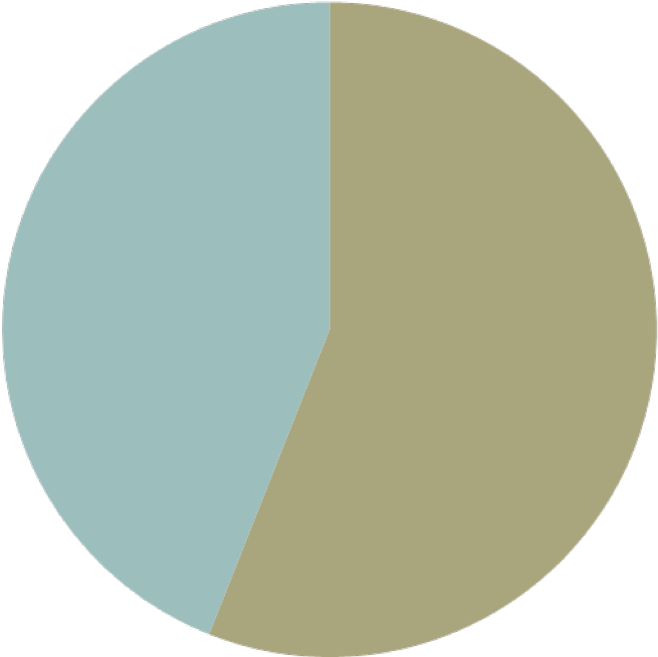
Clinical Trial Readiness for the Dystroglycanopathies

- 'Wellstone Study'
- Longitudinal design
- Conducted in preparation for a clinical trial
 - Identify patients
 - Describe signs & symptoms
 - Define natural history and refine tools

Total enrollment per year

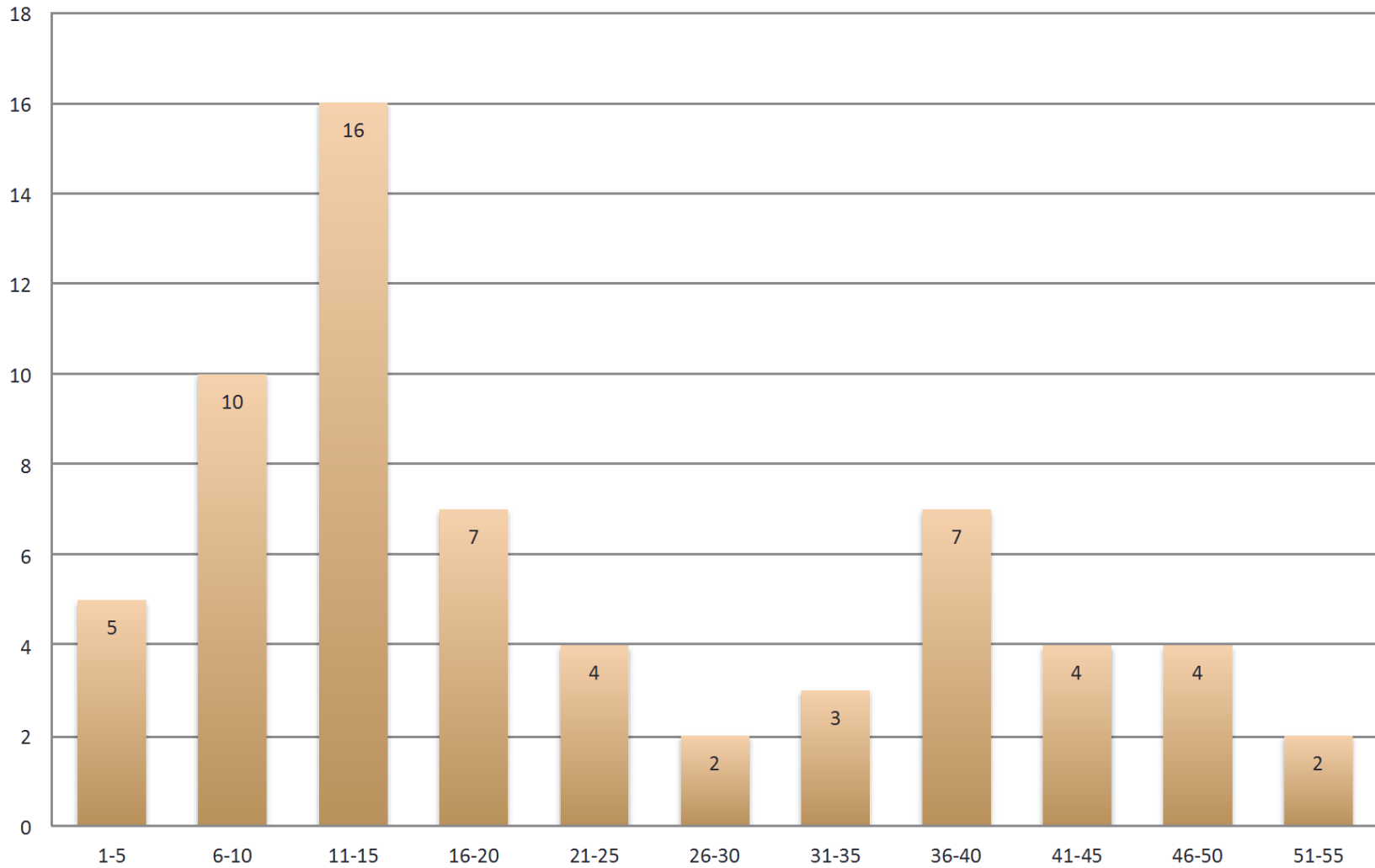


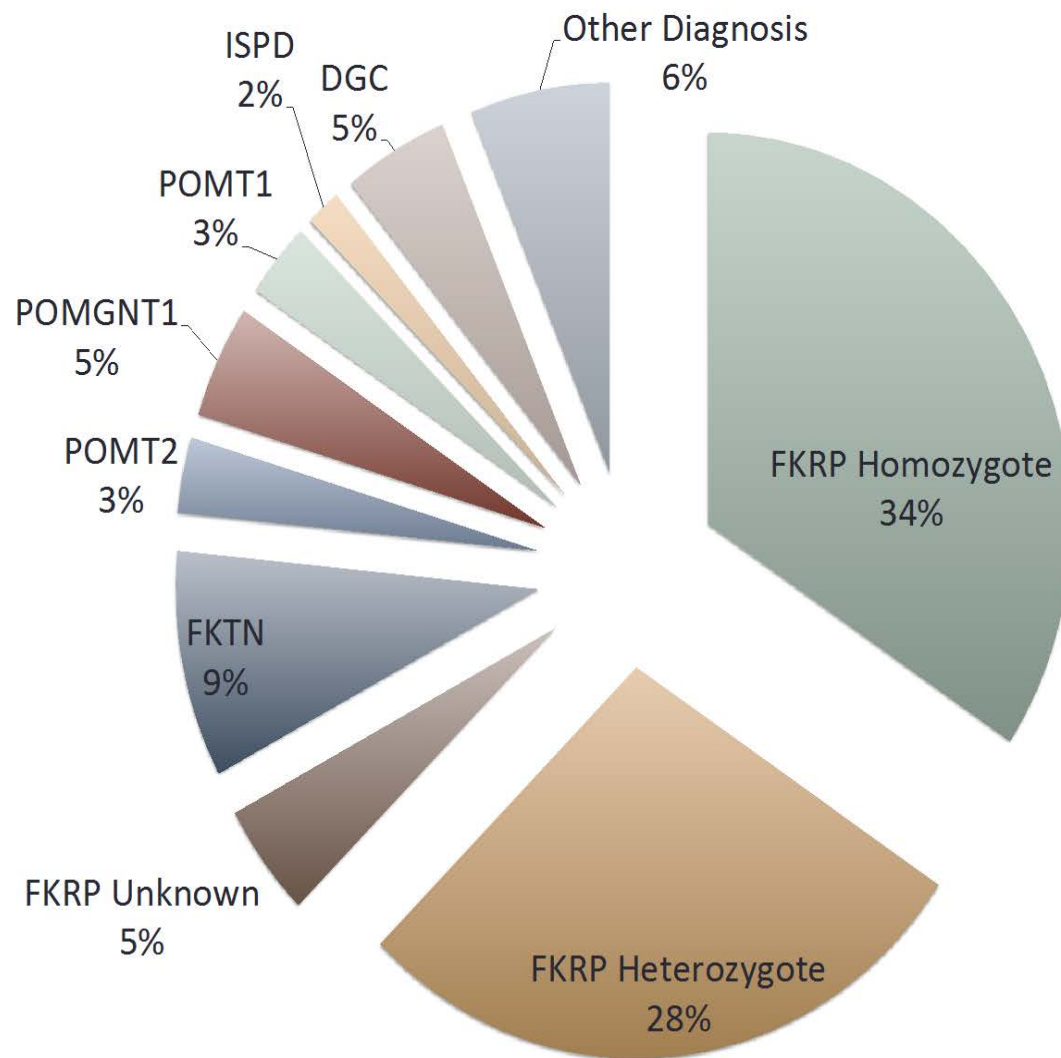
Gender



- Male
- Female

Participant Age







Conference Attendance

