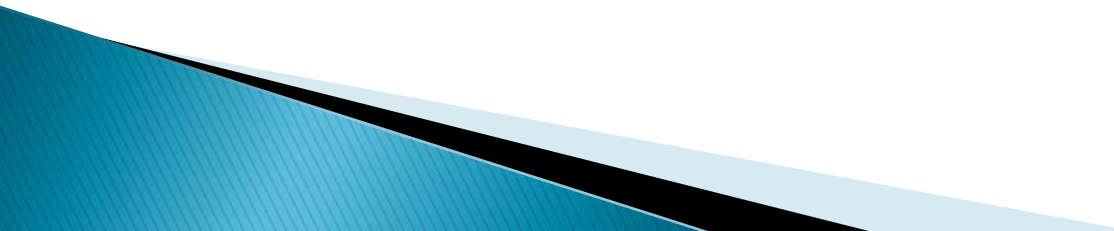


The Dystroglycanopathies: 2014 Patient and Family Conference

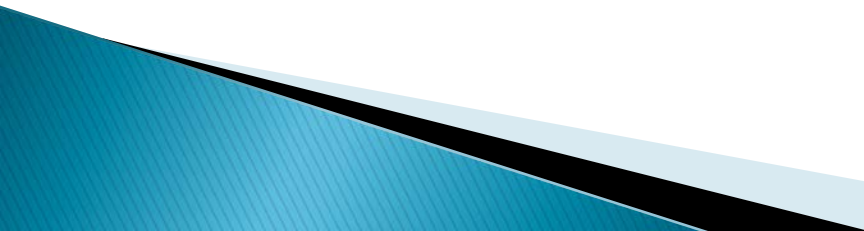
Carrie Stephan



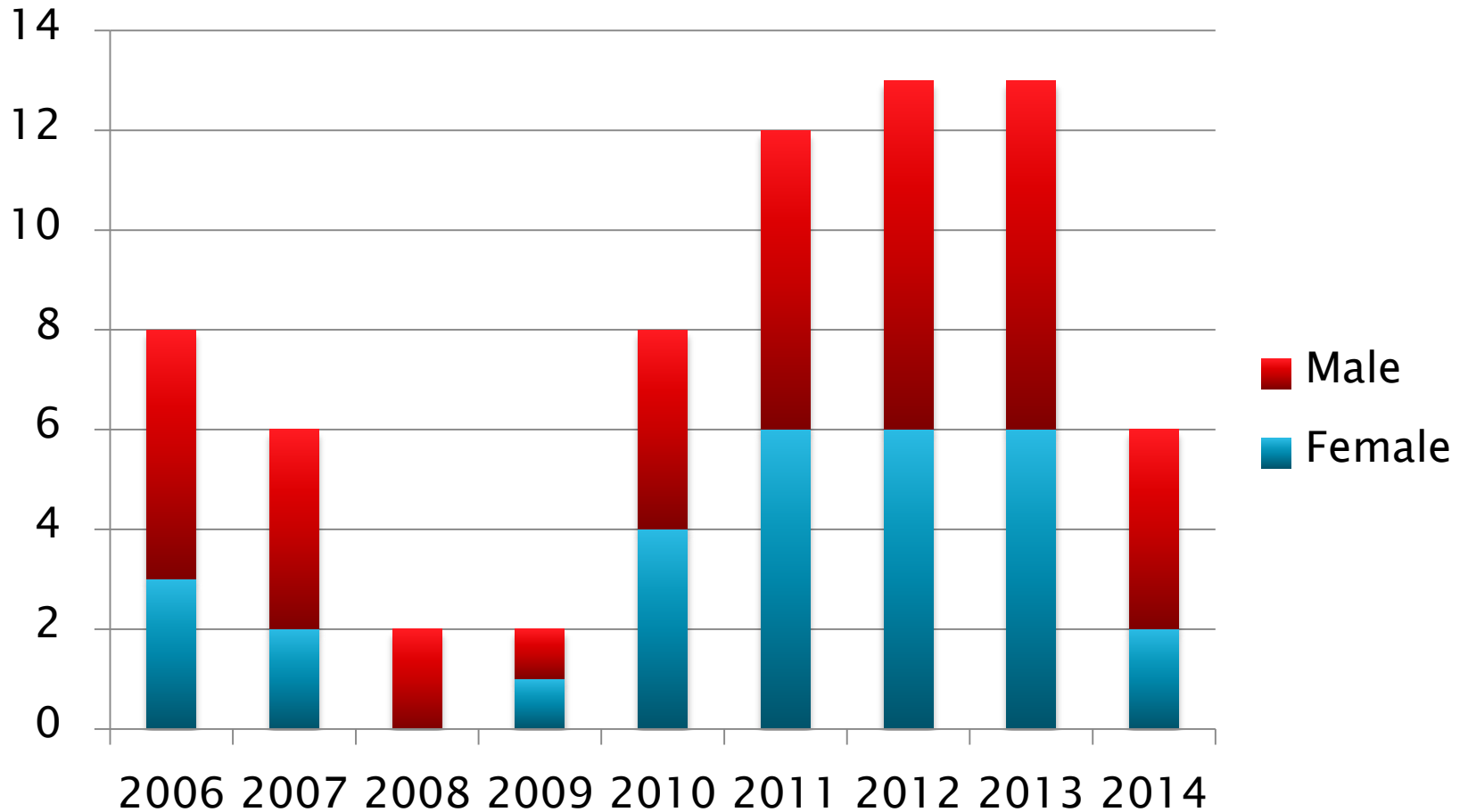
How it all started...

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

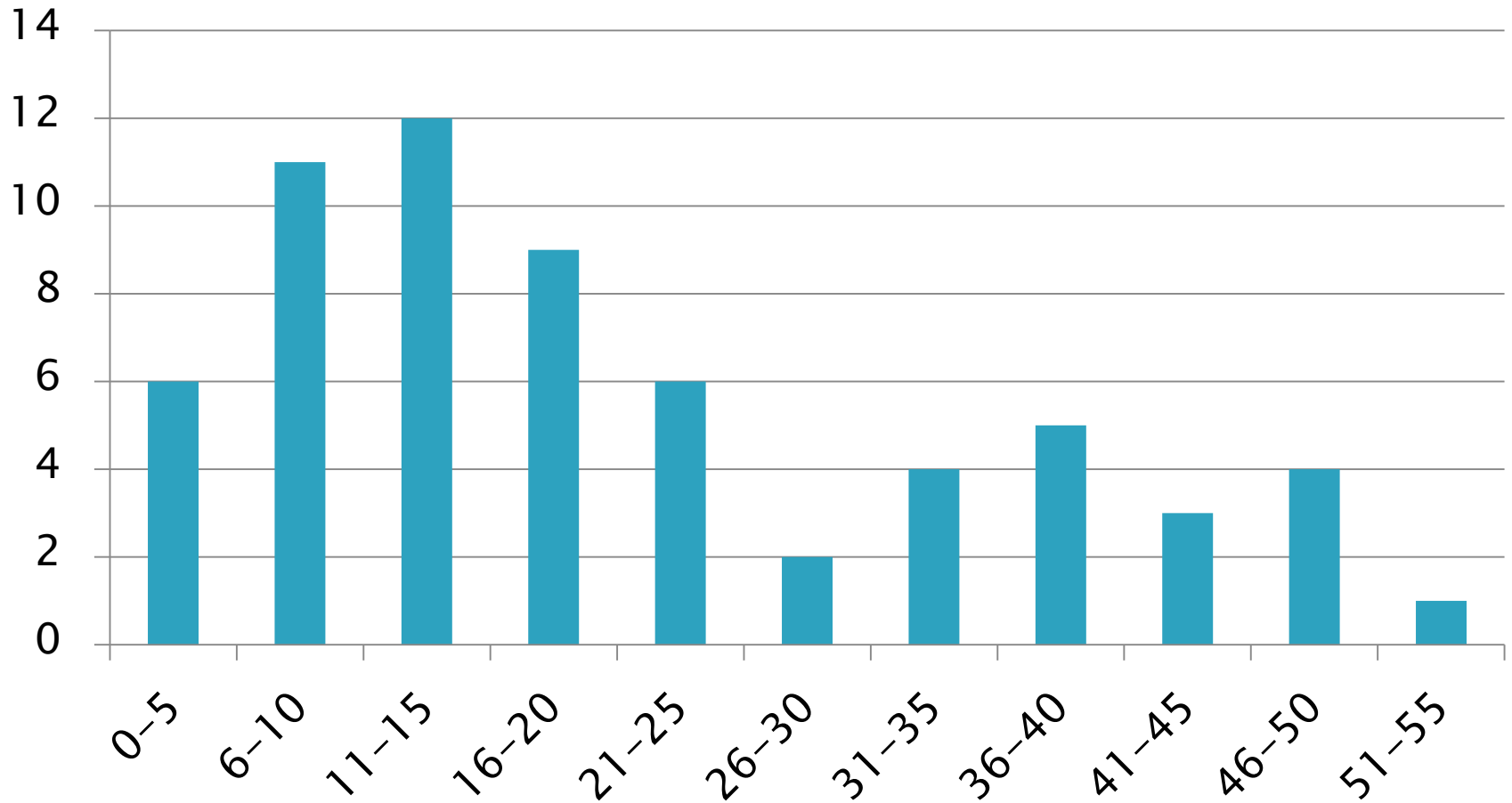
Clinical Trial Readiness for the Dystroglycanopathies

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

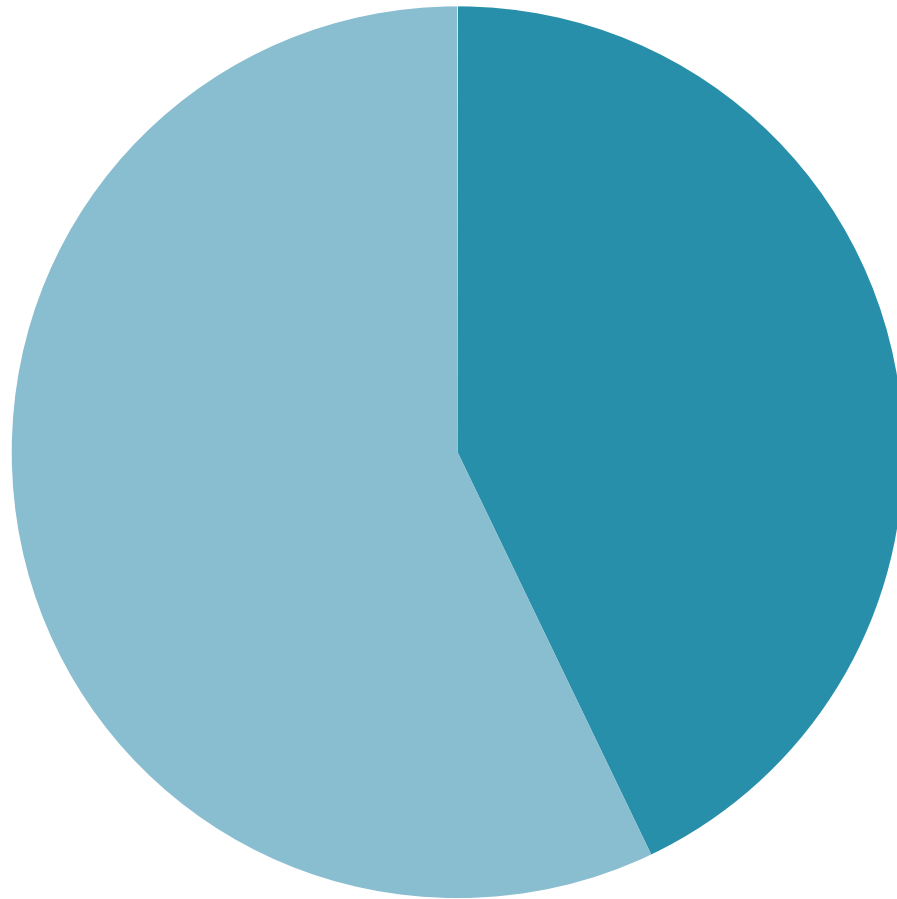
Total Enrollment per Year



Participant Age



Gender

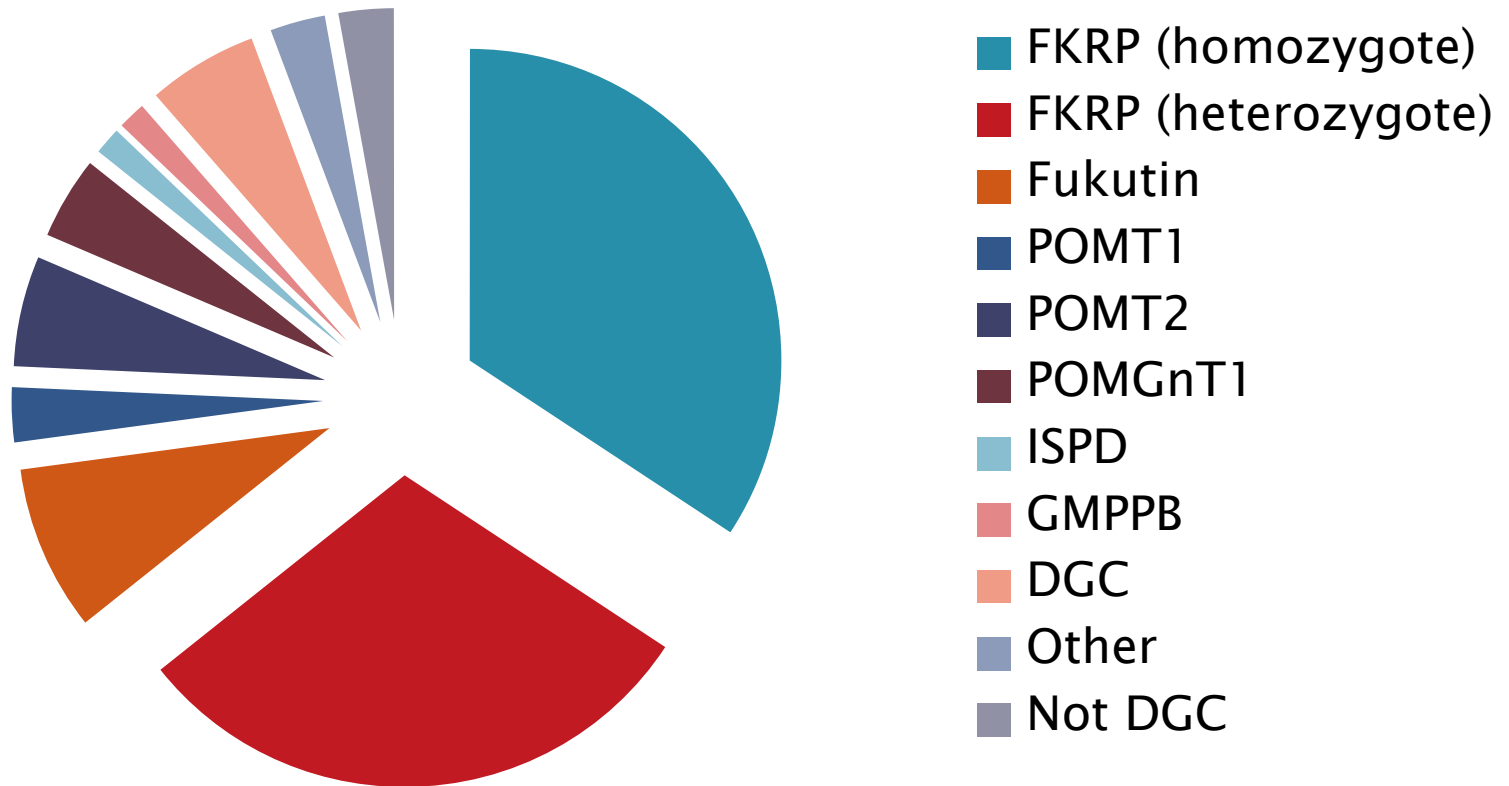


■ Female

■ Male

Participant Diagnosis

Column1



Conference Attendance

