The Dystroglycanopathies: 2014 Patient and Family Conference

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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
Participant Age

- 0-5: 6
- 6-10: 10
- 11-15: 12
- 16-20: 9
- 21-25: 8
- 26-30: 2
- 31-35: 4
- 36-40: 5
- 41-45: 3
- 46-50: 3
- 51-55: 1
Gender

- Female
- Male
Participant Diagnosis

Column 1

- FKRP (homozygote)
- FKRP (heterozygote)
- Fukutin
- POMT1
- POMT2
- POMGnT1
- ISPD
- GMPPB
- DGC
- Other
- Not DGC
Conference Attendance

2011 2012 2013 2014

Patients
Family
Total