

The Dystroglycanopathies: 2012 Patient & Family Conference

Study Participant Data
Carrie Stephan

8/17/2001

A Very Special Day

The Early Days

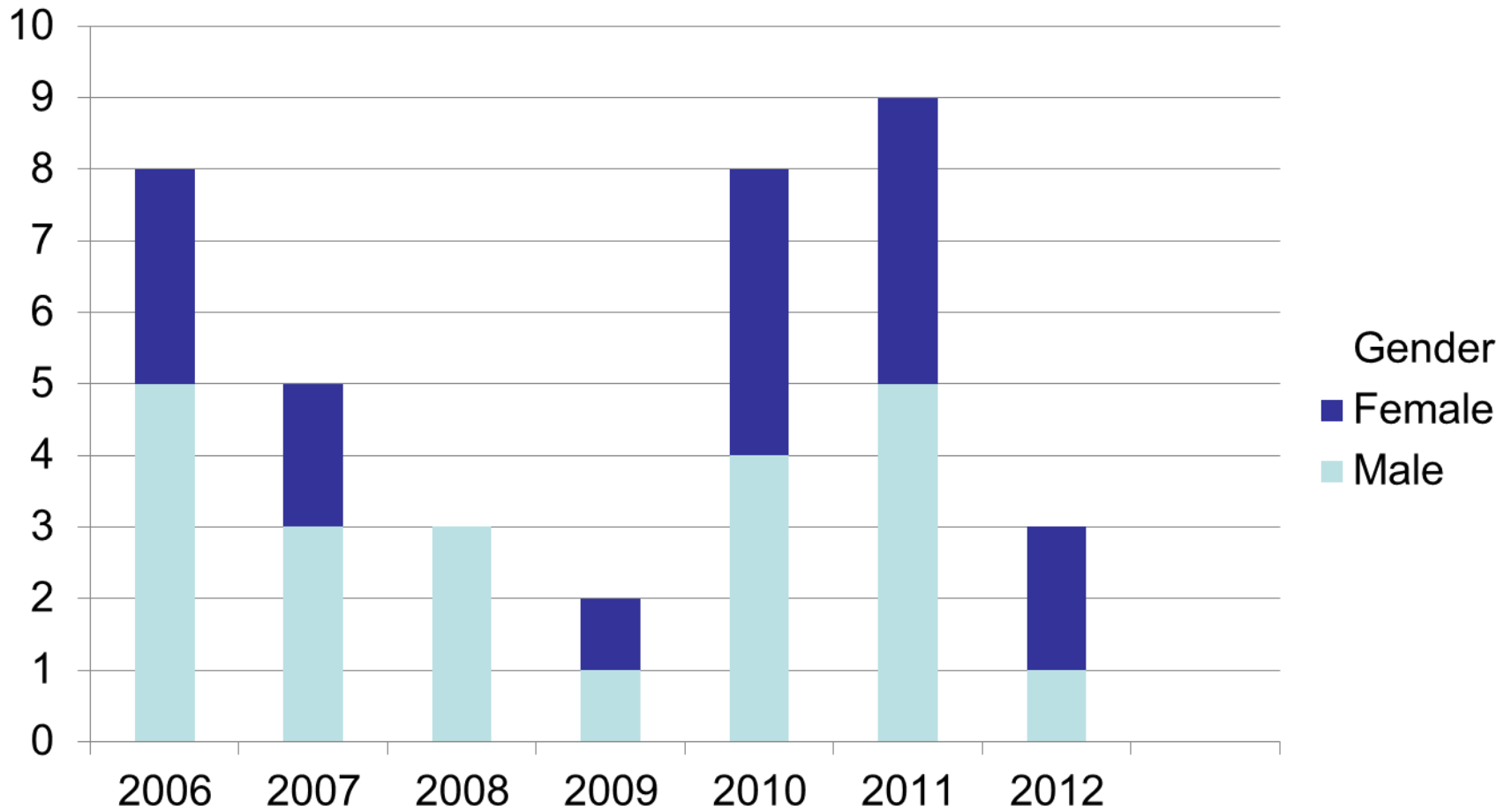
A Clinical Evaluation of the FKRP Muscular Dystrophies

- 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



Ages of Participants

Age Range

0-9 years = 7

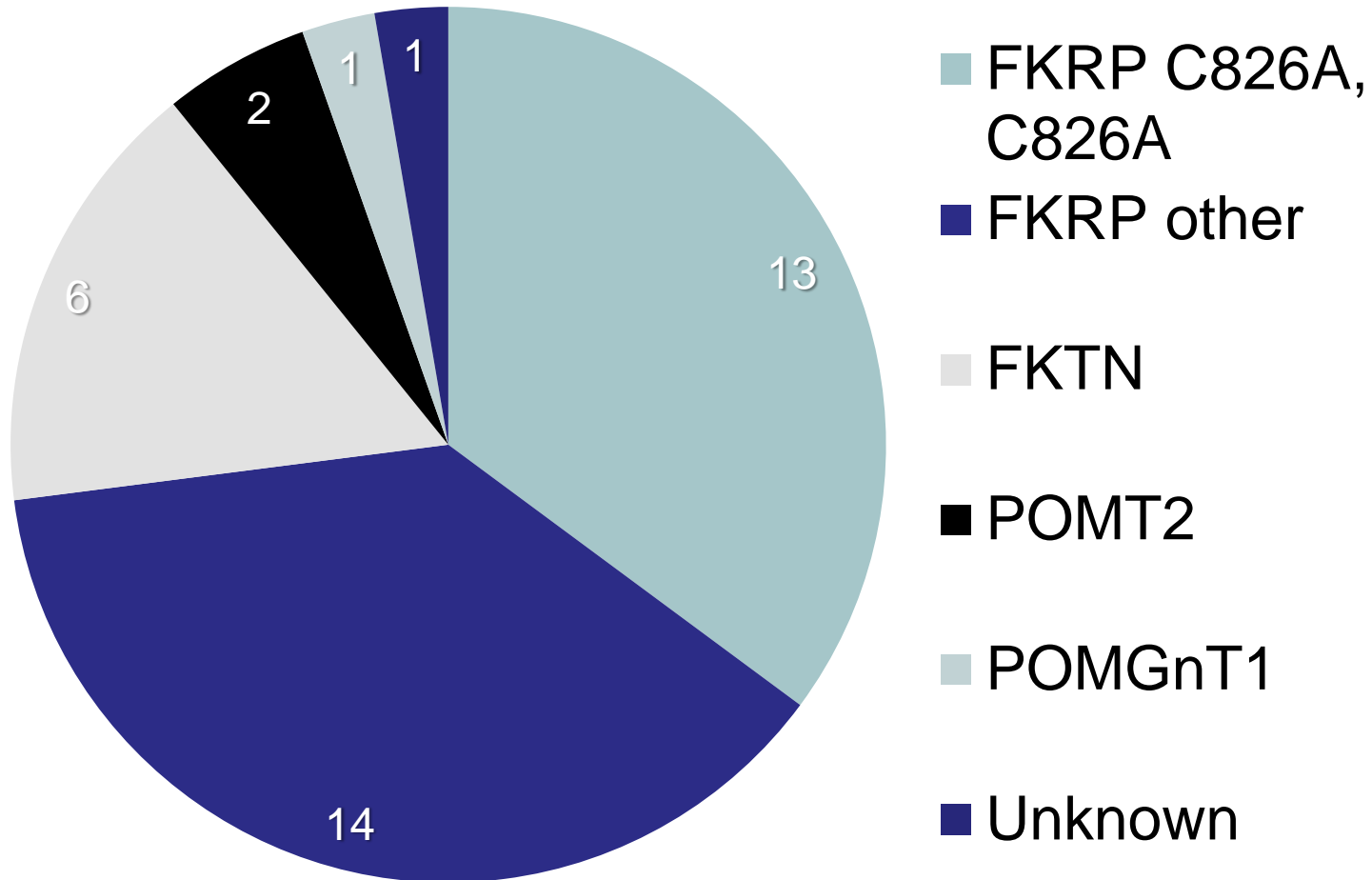
10-19 yrs = 16

20-29 yrs = 5

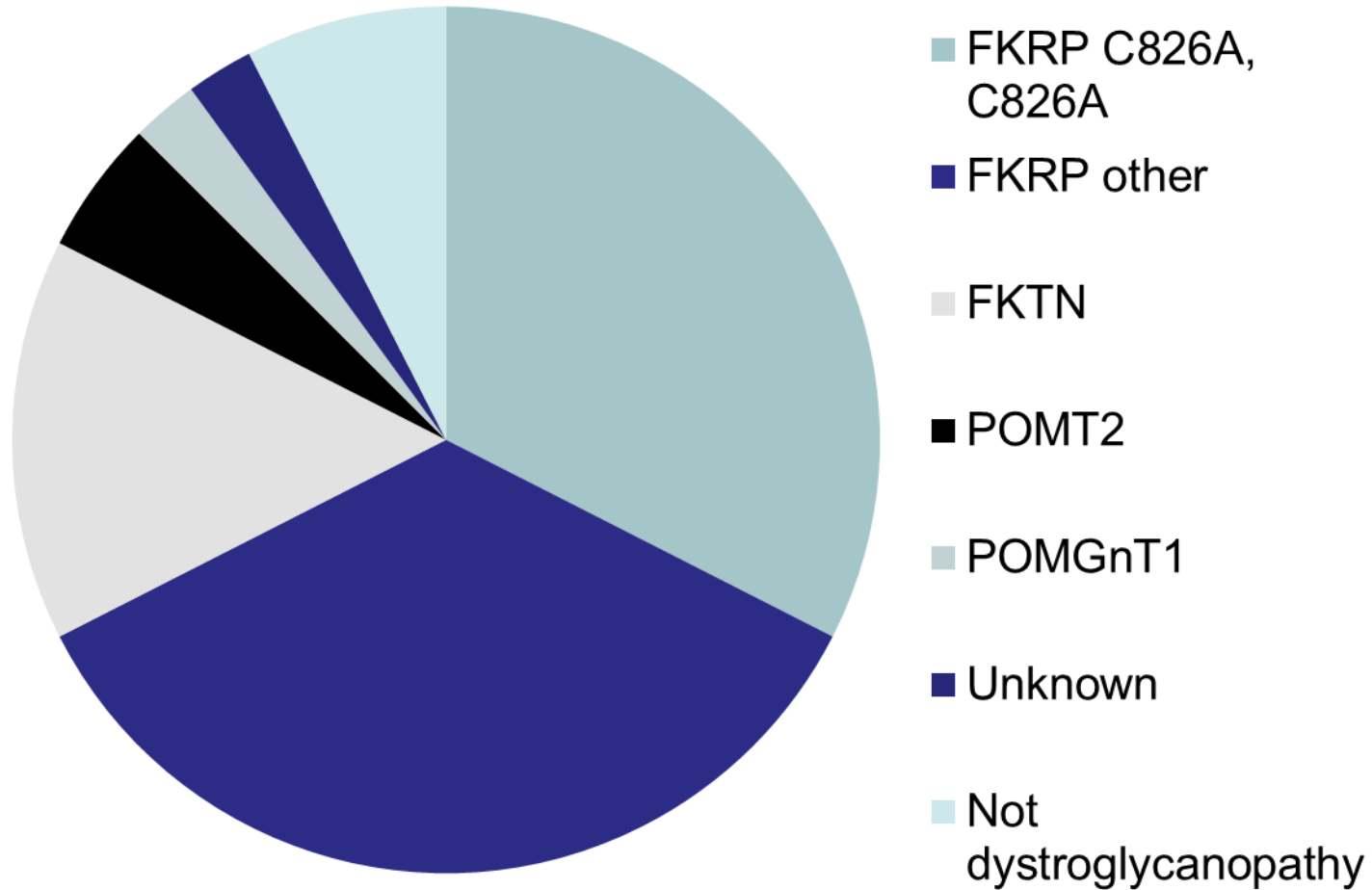
30-39 yrs = 5

40+ = 3

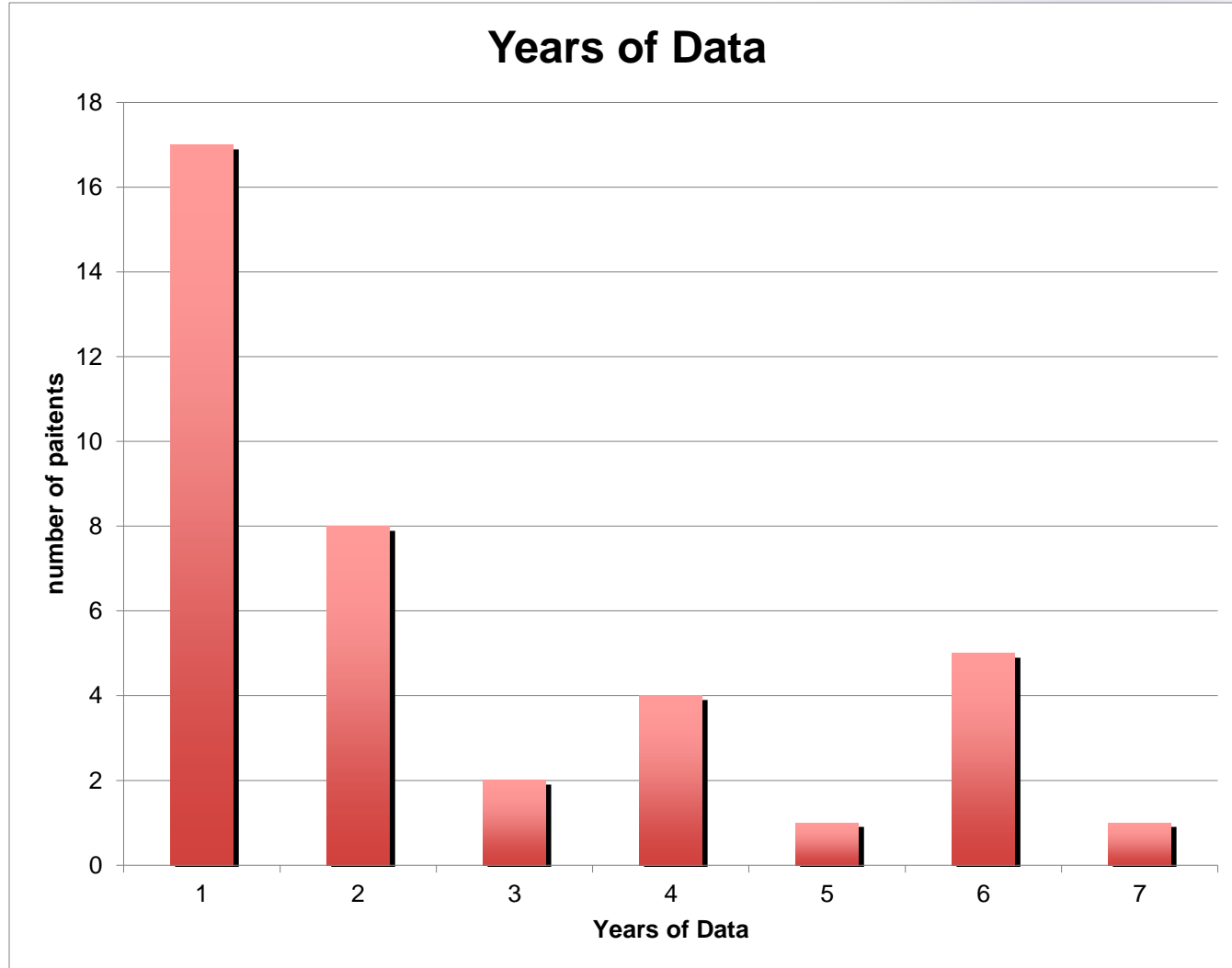
Current participants, genetics



Dystroglycanopathy Genetics + Other



Years of Data



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)