the
christopher
project

MDF FDA Workshop

Thursday, September 17, 2015

Presented by Katharine A. Hagerman, PhD

The Christopher Project

A comprehensive survey of patients and families about their experience in living with myotonic dystrophy.

Aims:

- Examine disease complexity

- Understand unmet needs

- Identify what matters most

- Improve health outcomes

The Christopher Project

Collaboration across North America:

- Patients and families

- National advocacy organizations

- Health care providers and disease experts

The Christopher Project

Survey Distribution
- 3,967 surveys mailed out
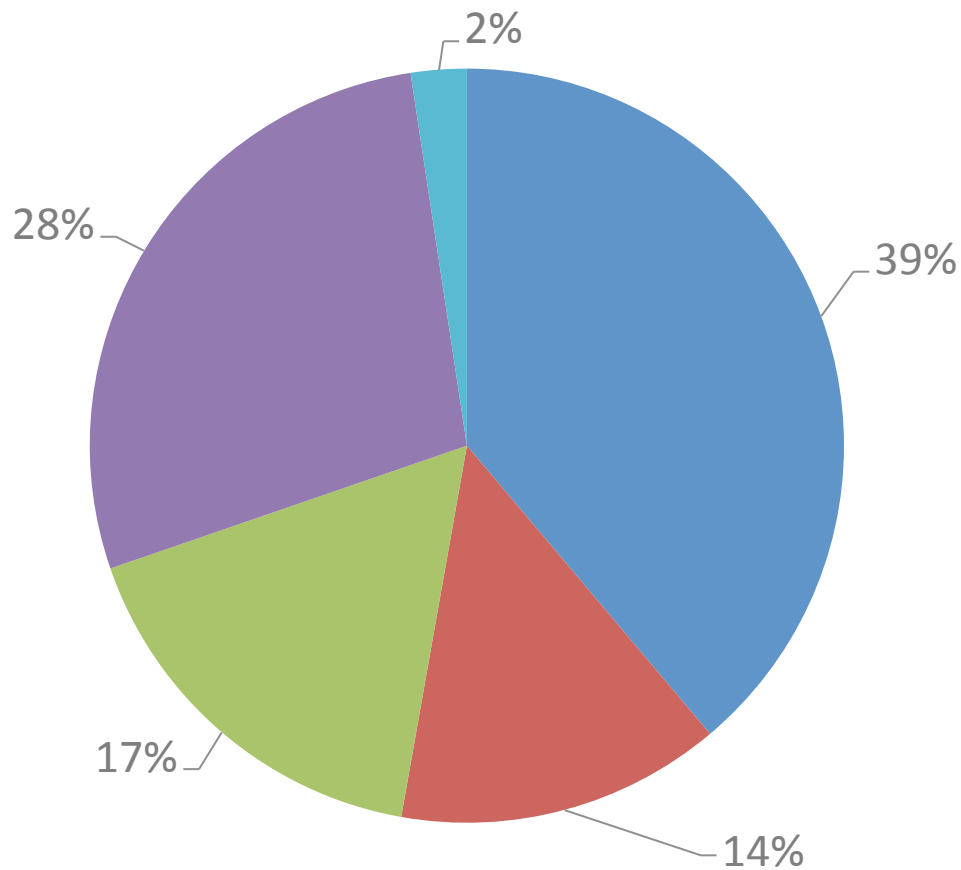- 1,433 received = 36% response rate

Over 100 questions on:
- Demographics
- Current Health
- Diagnostic Experience
- Information and Resources
- Physical Symptoms
- Health Care Experience
- Managing Daily Life
- Insurance

Administered under human subjects ethics
approval in U.S.A. and Canada

Christopher Project Respondents

Survey Question: What type of myotonic dystrophy do you have?

2%

28%

39%

17%

14%

N=1,180 valid responses

- DM1 (n=457)
- cDM1
- DM2
- Unknown/unsure
- Omitted

Data Presented Today

457 respondents living with DM1:
- 78% report confirmed genetic diagnosis
- does not include congenital DM1

Topics:
- Demographic profile
- Symptom prevalence and impact
- Employment picture
- Understanding complexity
- Comparison with PRISM study

Demographic Profile

Christopher Project represents a diverse gender, age and geographic sample of myotonic dystrophy patients.

Characteristic	Value
# respondents	457
Male	39%
Female	61%
Mean age ± SD	45.0 ± 16.0
Mean age of onset ± SD	27.2 ± 14.9

Symptom Prevalence

Percentage of DM1 respondents who report that they experience the specific symptom:

Rank	Symptom	Christopher Project	PRISM Comparison*
1	Muscle weakness	96%	94%[a]
2	Daytime sleepiness	94%	88%[b]
3	Fatigue	93%	91%[c]
4	Myotonia	88%	90%[d]

*Corresponding PRISM Item:
a: Problems with hands or arms
b: Impaired sleep or daytime sleepiness
c: Fatigue
d: Myotonia

Top 4 Symptoms by Relative Impact

Rank	Symptom	Symptom Score (/3)
1	Muscle weakness	2.16
2	Fatigue	2.14
3	Daytime sleepiness	2.08
4	Myotonia	1.68

Christopher Project Symptom Rating Scale:

0: Have Symptom with NO Impact

1: Have Symptom with MINOR Impact

2: Have Symptom with MODERATE Impact

3: Have Symptom with MAJOR Impact

Employment Picture

Major challenges in obtaining and retaining employment*

DM1 respondents 21 through 65 years	n=390
Report being employed full-time	17.4%
Report being employed part-time	13.1%

Report being "*Unable to work due to DM*"	46.2%

*U.S. Department of Labor; Bureau of Labor Statistics
http://data.bls.gov/timeseries/LNS14000000

Understanding Complexity

- Christopher Project data captures the variability, heterogeneity and severity of this complex disease

- Enables examination of differences, similarities and relationships among subgroups; examples:
  - Prevalence of muscle weakness, fatigue and myotonia vary significantly based on *current age*
  - Prevalence of daytime sleepiness is significantly higher for *age of onset* between 21-30 years of age
  - Impact of muscle weakness increases with *age* until 61+, when impact lessens

- Provides basis and insight for further research and therapeutic development

Christopher Project & PRISM

- Both studies capture and illuminate the experience of myotonic dystrophy patients and families

- Results between studies are highly comparable

- Christopher Project confirms and reinforces PRISM findings regarding symptom prevalence and relative impact

Summary

- Christopher Project highlights the value of patient reported outcomes while capturing extensive data on the patient and family experience
    - Heavy symptom burden: muscle weakness and sleep/fatigue issues are critical
    - Sufficiently powered to yield critical insight into complex disease presentation (heterogeneity, variability and progression)
    - Insight into numerous aspects of daily life
    - Confirms and reinforces other patient-reported findings

> Overall study findings will be submitted for peer-reviewed publication in Q4 2015
>
> Christopher Project data will be available to qualified parties upon request

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