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

- DM1 (n=457): 39%
- cDM1: 28%
- DM2: 17%
- Unknown/unsure: 14%
- Omitted: 2%

N=1,180 valid responses
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
Christopher Project represents a diverse gender, age and geographic sample of myotonic dystrophy patients.

<table>
<thead>
<tr>
<th>Characteristic</th>
<th>Value</th>
</tr>
</thead>
<tbody>
<tr>
<td># respondents</td>
<td>457</td>
</tr>
<tr>
<td>Male</td>
<td>39%</td>
</tr>
<tr>
<td>Female</td>
<td>61%</td>
</tr>
<tr>
<td>Mean age ± SD</td>
<td>45.0 ± 16.0</td>
</tr>
<tr>
<td>Mean age of onset ± SD</td>
<td>27.2 ± 14.9</td>
</tr>
</tbody>
</table>
## Symptom Prevalence

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

<table>
<thead>
<tr>
<th>Rank</th>
<th>Symptom</th>
<th>Christopher Project</th>
<th>PRISM Comparison*</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Muscle weakness</td>
<td>96%</td>
<td>94%(^a)</td>
</tr>
<tr>
<td>2</td>
<td>Daytime sleepiness</td>
<td>94%</td>
<td>88%(^b)</td>
</tr>
<tr>
<td>3</td>
<td>Fatigue</td>
<td>93%</td>
<td>91%(^c)</td>
</tr>
<tr>
<td>4</td>
<td>Myotonia</td>
<td>88%</td>
<td>90%(^d)</td>
</tr>
</tbody>
</table>

*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

<table>
<thead>
<tr>
<th>Rank</th>
<th>Symptom</th>
<th>Symptom Score (/3)</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Muscle weakness</td>
<td>2.16</td>
</tr>
<tr>
<td>2</td>
<td>Fatigue</td>
<td>2.14</td>
</tr>
<tr>
<td>3</td>
<td>Daytime sleepiness</td>
<td>2.08</td>
</tr>
<tr>
<td>4</td>
<td>Myotonia</td>
<td>1.68</td>
</tr>
</tbody>
</table>

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*

<table>
<thead>
<tr>
<th>Description</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>DM1 respondents 21 through 65 years</td>
<td>n=390</td>
</tr>
<tr>
<td>Report being employed full-time</td>
<td>17.4%</td>
</tr>
<tr>
<td>Report being employed part-time</td>
<td>13.1%</td>
</tr>
<tr>
<td>Report being “Unable to work due to DM”</td>
<td>46.2%</td>
</tr>
</tbody>
</table>

*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
Acknowledgements

Thank you to our 1,433 Survey Participants!

Christopher Project Team:
• Sarah Howe
• Don MacKenzie
• Diane Bade
• Cynthia Gagnon
• Chad Heatwole

Partners:
• MDF
• MDC
• MDA
• Stanford University
• University of Rochester
• GRIMN