

Patient Reported Aspects of Becker Muscular Dystrophy from The Duchenne Registry a Comprehensive Registry for Duchenne and Becker Muscular Dystrophy (BMD)

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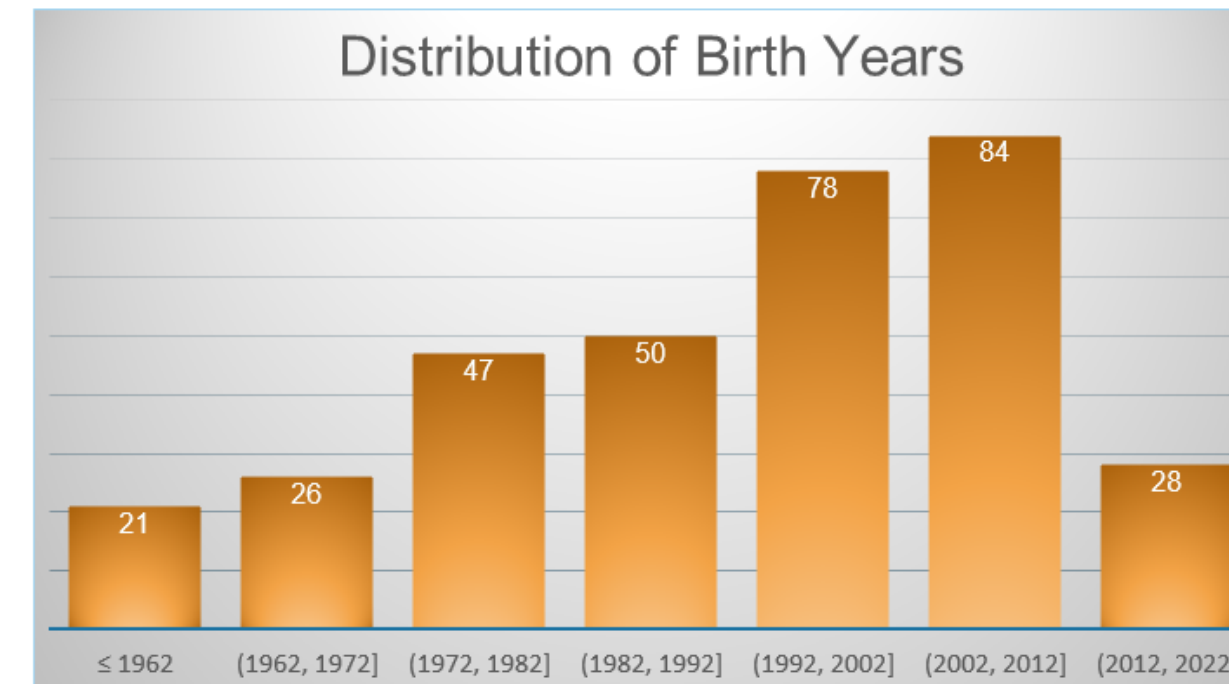
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Data from The Duchenne Registry from Registrants with Becker Muscular Dystrophy

- The Duchenne Registry has been collecting data since 2007 from those affected by Duchenne and Becker
 - Total records over 5000
 - Limited longitudinal data from follow-up entries
 - Patient, family entered data; limited data review possible, so limited curation
- Multiple surveys are collected that focus on different aspects of disease
 - Demographics
 - Diagnosis: age, symptoms at onset
 - Muscle function: ambulation status, use of supportive devices
 - Genetic testing, family history
 - Corticosteroid use
 - Pulmonary, cardiac, bone and behavior

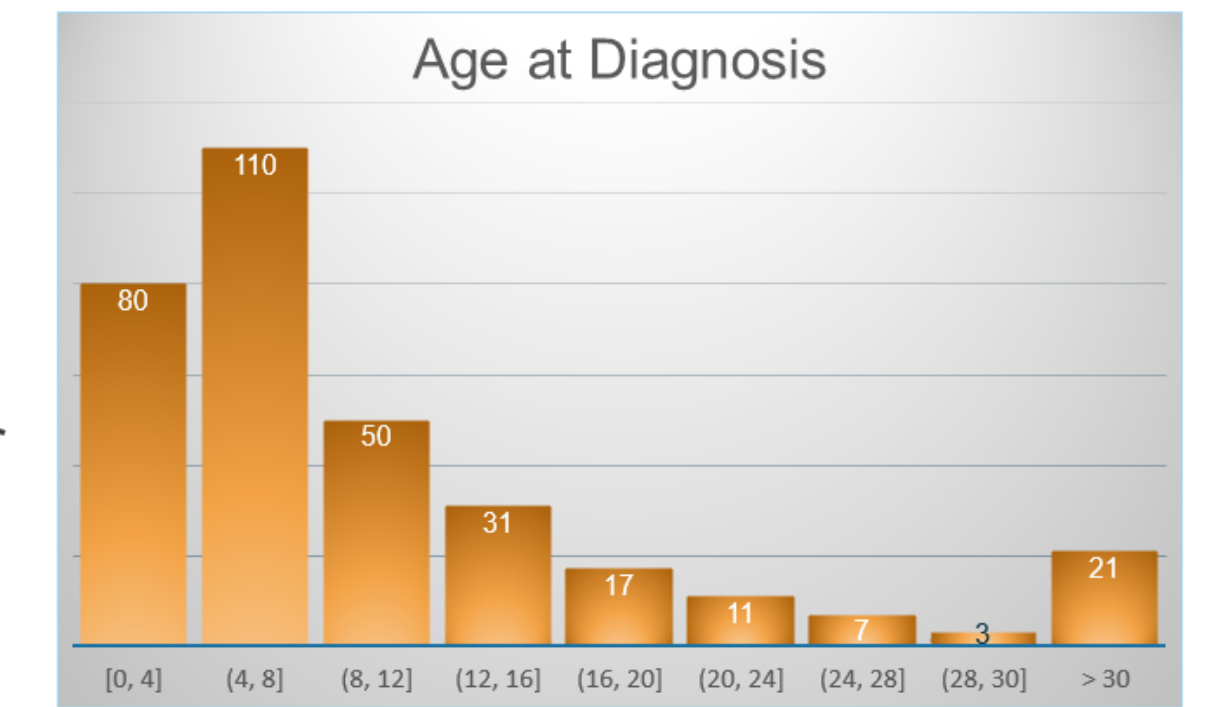
Data Available in the Duchenne Registry

- Data set through May 2022
 - Unique registrants with Becker: 334 total
 - Records submitted: 378
- This analysis includes all countries



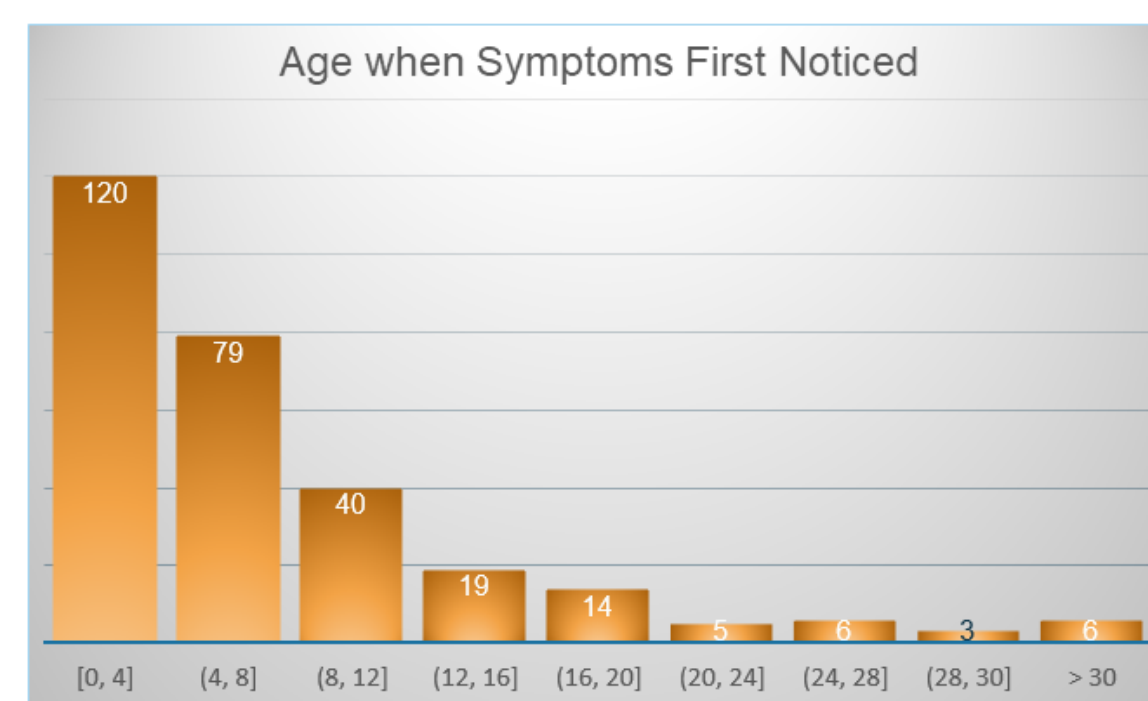
Average Age of Diagnosis of Becker is 10 Years Old

- Diagnosis occurs at a young age
- Average age of diagnosis 10 years
- Median age 7 years, several years older than for Duchenne
- Age of participants at time of survey 19.9 years



Symptoms First Noticed at an Average Age of 7 Years

- Median age 4 years, several years older than for Duchenne
- Symptom onset ranged from first year of life to age 58
- Median duration of symptoms prior to diagnosis was 2 years, with 25% ≥ 5 years and 12% ≥ 10 years



Symptoms at Diagnosis of Becker

- Respondents were asked about initial presentation, with more than one answer possible.
- Diagnosis occurred most often when symptoms are noticed by family members

Symptom	%
Difficulty with physical activities such as running or rising from the floor	65%
Enlarged calves	55%
Muscle pain/cramps	51%
Toe walking	43%
Frequent falls	33%
Delays in motor development (such as learning to walk)	27%
Elevated/high liver enzymes (ALT, AST, etc.)	25%
Family member with muscular dystrophy	22%
Elevated/high CK level	20%
Health care provider raised a concern	13%
Delays in developing language	12%
School/Preschool/Daycare raised a concern	4%
Newborn/infant screen	1%
Heart problems (such as cardiomyopathy)	1%

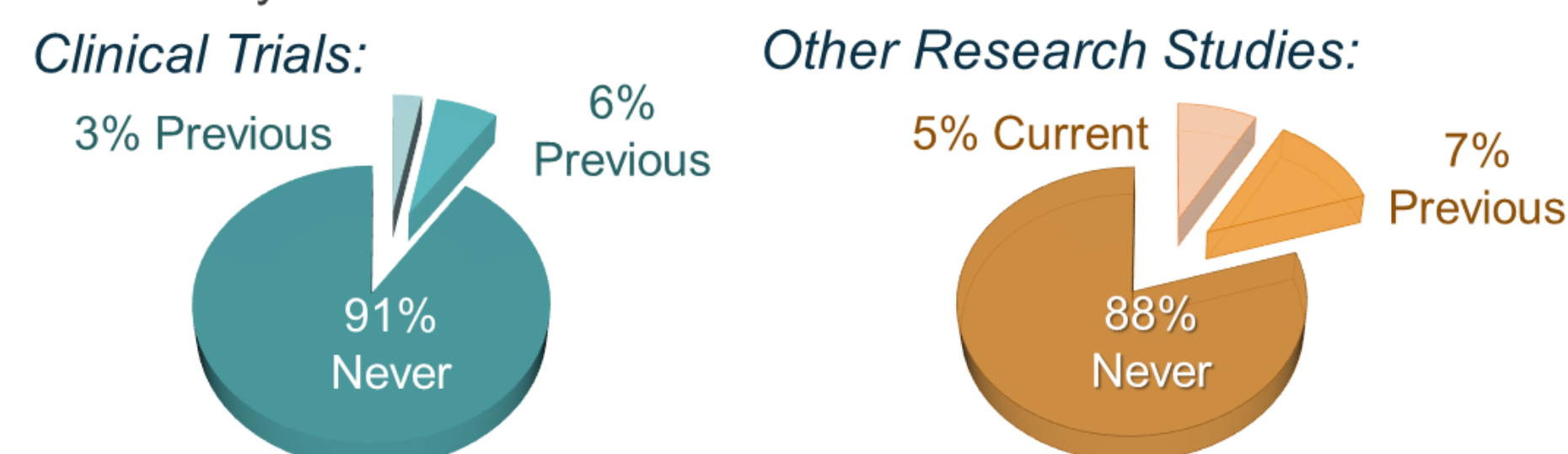
Use of Assistive Devices for Mobility is Common in Becker Registrants

	2 to 18 years	Adult
I can get around on my own but I sometimes need help from a mobility device.	14%	15%
I use a wheelchair or other mobility device and rarely or never walk.	8%	34%
I usually or always walk on my own without help or mobility devices.	74%	51%

- The average age of adult respondents was 35.
- Almost half of adults used a mobility device

Current or Previous Clinical Trial Experience is Rare in Becker Registrants

- Only 3% of registrants were currently participating in clinical trials, with only 6% previously participating. Previous participation was similar in adults and children.
- A similar fraction reported participation in other research studies such as natural history studies



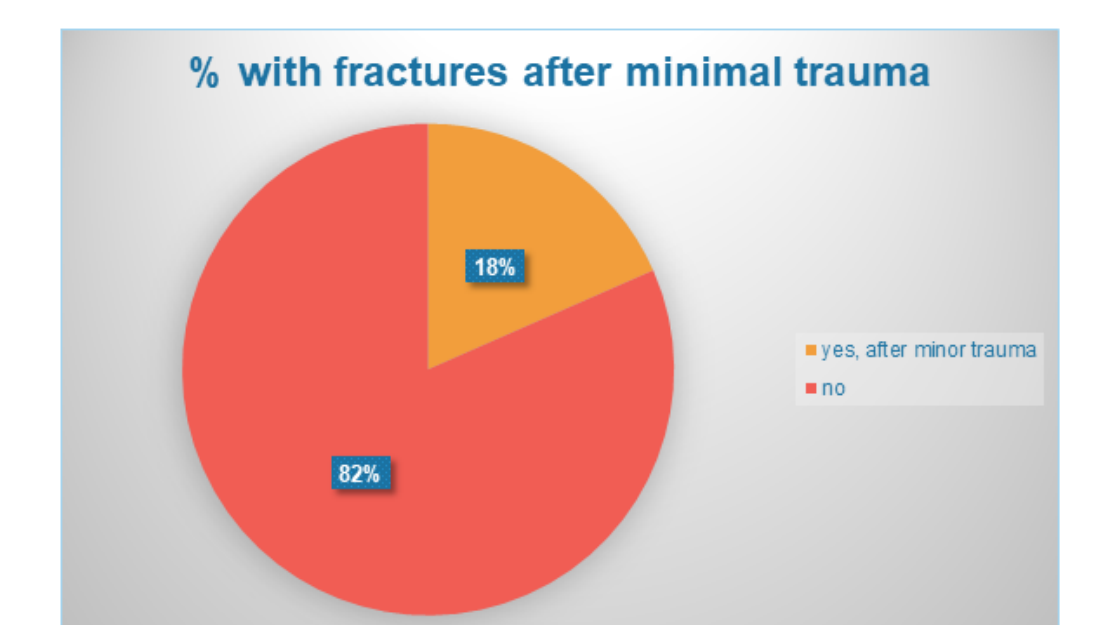
Current or Previous Corticosteroid Use is Relatively Uncommon

	%
Current deflazacort use	6%
Current prednisone/prednisolone use	9%
Previous steroid use	10%
Any previous steroid use	25%
No previous steroid use	75%

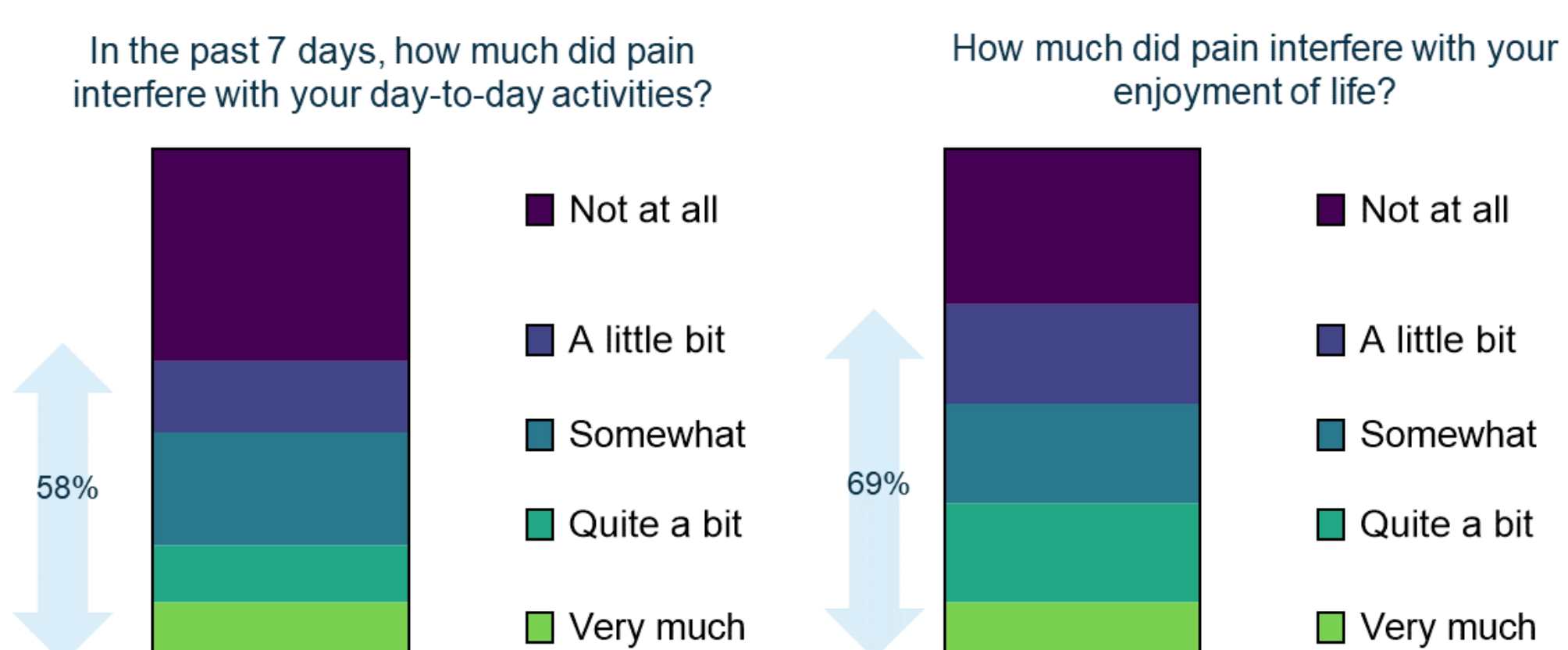
- Those currently on deflazacort were, on average, 17 years, while those on prednisone/prednisolone were 20 years, and those previously on steroids were 26 years.
- The average age for initiating steroids was 14 years
- Problems with side effects were the most common reason for stopping steroids, followed by not enough benefit

Bone Health in Becker

- Responses to "Have you broken a bone(s) following a minor trauma (like a simple fall) or no trauma?"
- Of the respondents who answered yes to having had a fracture, 76% had never been on corticosteroids

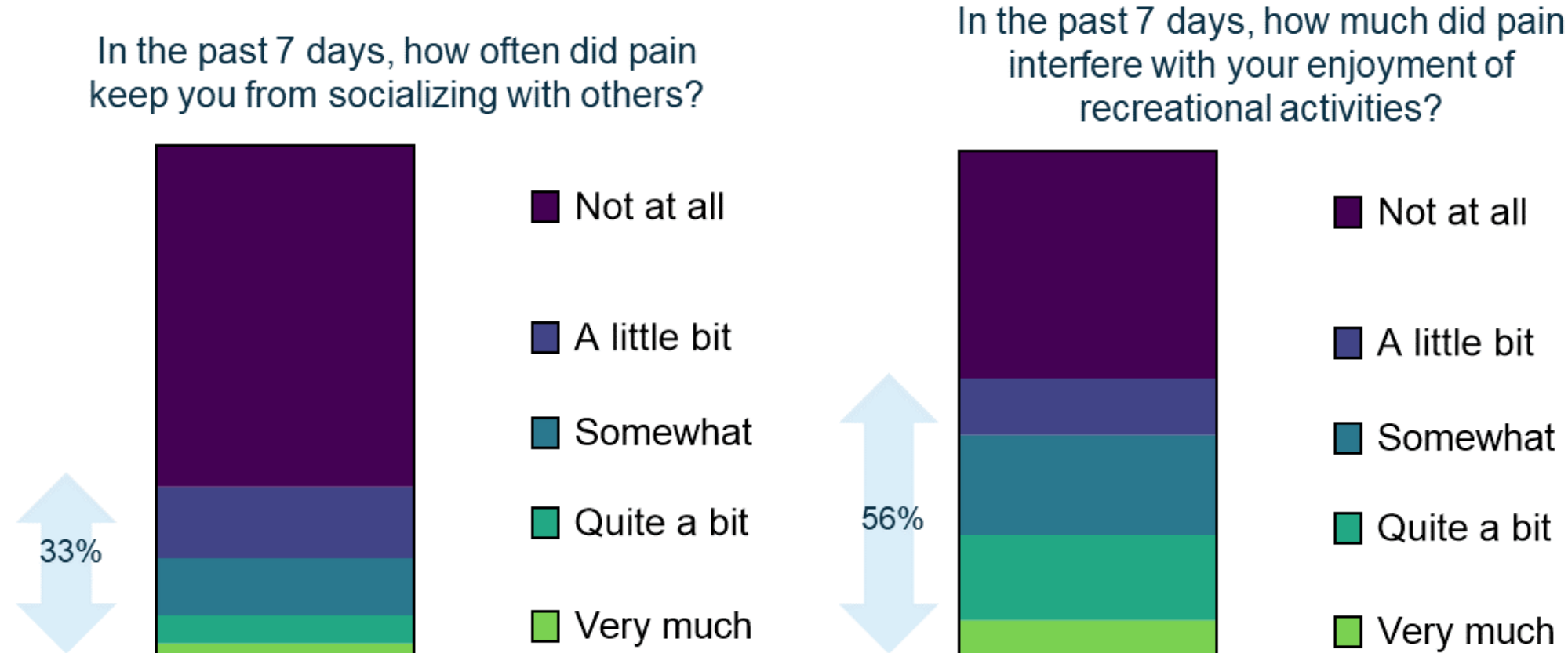


Pain Interferes with Daily Activities and Enjoyment of Life in Majority of Registrants with Becker



Data obtained using PROMIS validated questionnaire on pain

Pain Interferes with Social and Recreational Aspects of Life for Some Registrants with Becker



Data obtained using PROMIS validated questionnaire on pain

Conclusions

- While these data are from a self-selected group of patients who provided data to the Duchenne Registry, they provide a fuller picture of the disease course, and support the severity of the disease course of Becker muscular dystrophy
 - In these participants, diagnosis occurred early
 - Fractures after minimal trauma were not associated only with corticosteroid use
 - Despite the known side effect profile, corticosteroid use was currently or previously used in ~ one quarter of participants
 - There is an impact of pain on activities and quality of life, which deserves further investigation
 - Survey results underscore the need for therapeutic approaches that impact both muscle function and quality of life

Acknowledgments

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