

Insights into the Becker Muscular Dystrophy Experience from the Duchenne Registry, a Comprehensive Registry for Duchenne and Becker Muscular Dystrophy

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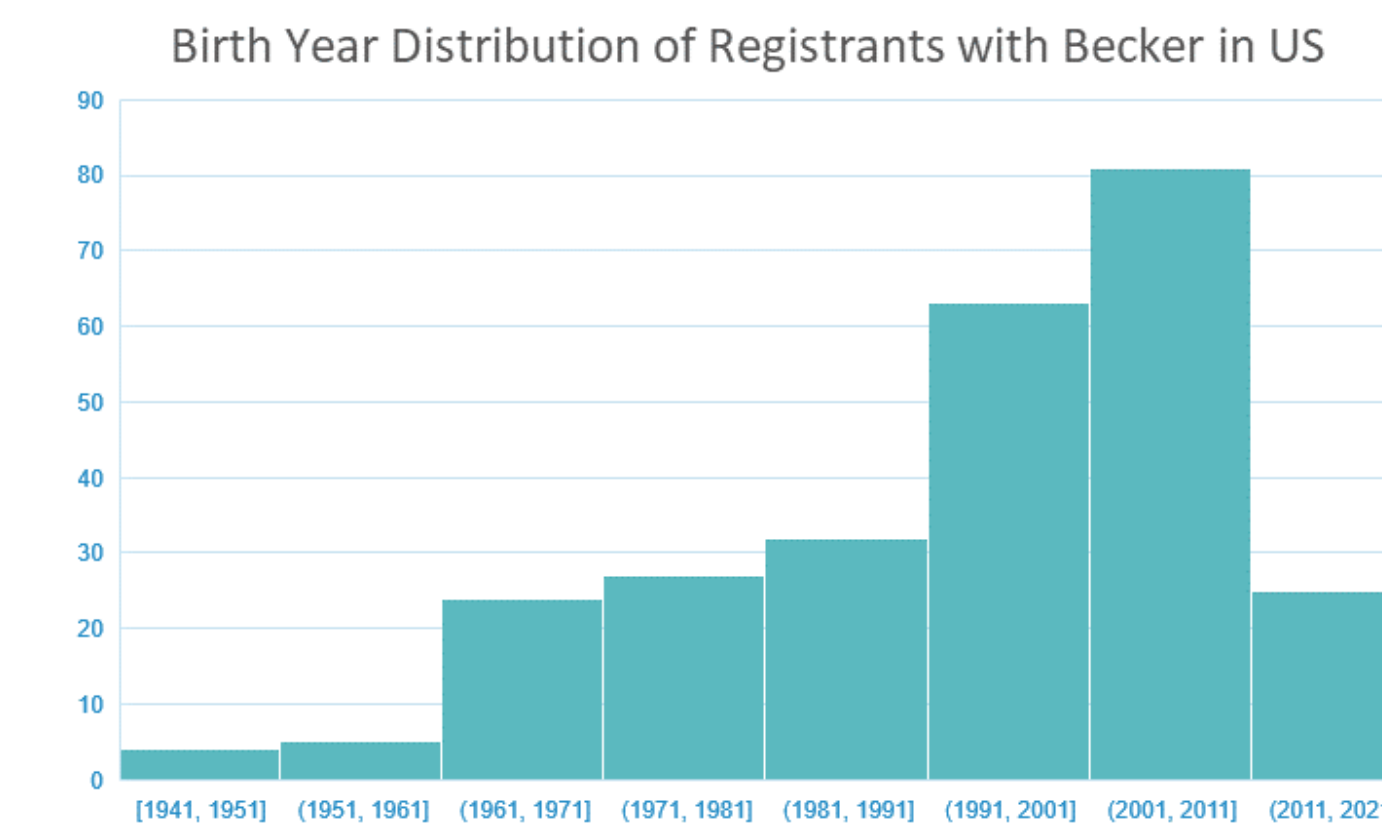
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Data from The Duchenne Registry from Registrants with Becker Muscular Dystrophy

- 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
 - Diagnosis: age, symptoms at onset
 - Muscle function: ambulation status, use of supportive devices
 - Genetic testing, family history
 - Corticosteroid use
 - Pulmonary, cardiac, bone and behavior

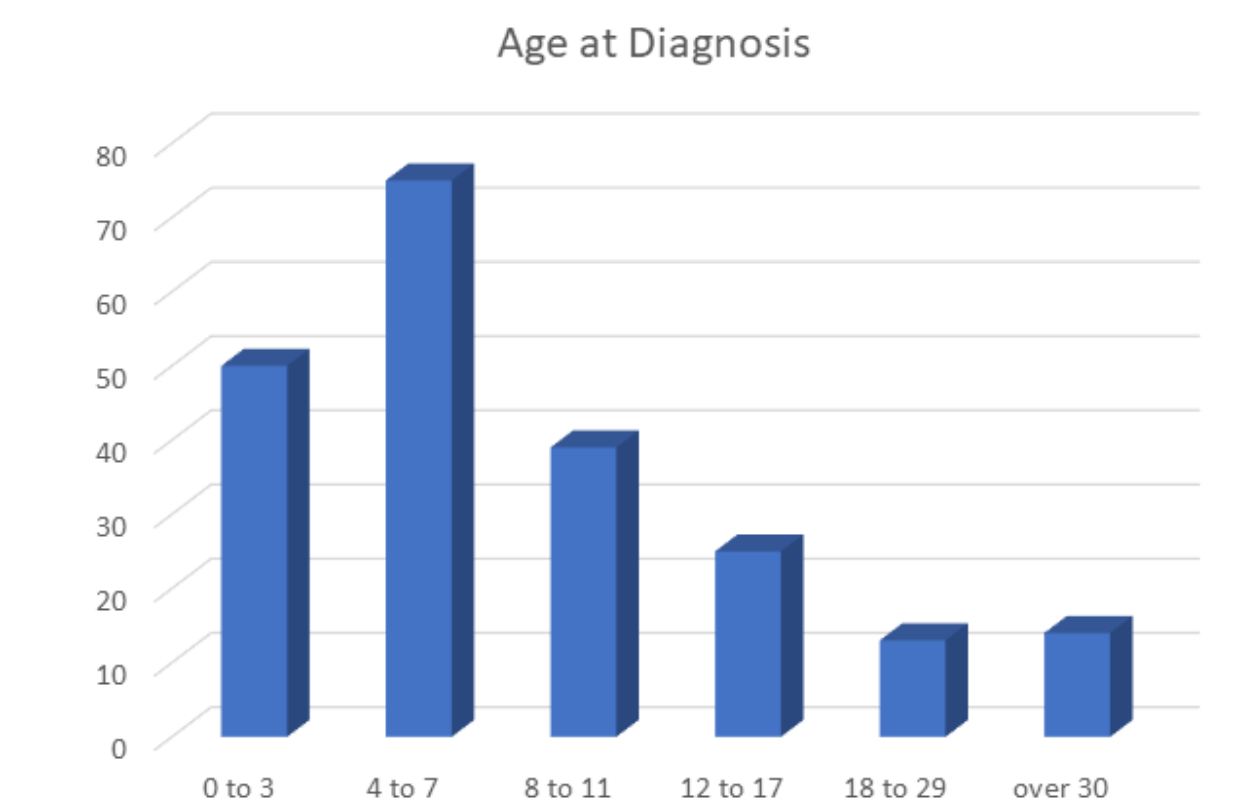
Data Available in the Duchenne Registry

- Data set through 2021
 - Unique registrants with Becker: 319 total, 201 from US
 - Records submitted: 657
- This analysis focuses on the US population



Average Age of Diagnosis of Becker is 10 Years Old

- Diagnosis occurs at a young age
- Average age 10 years
- Median age 7 years, several years older than for Duchenne

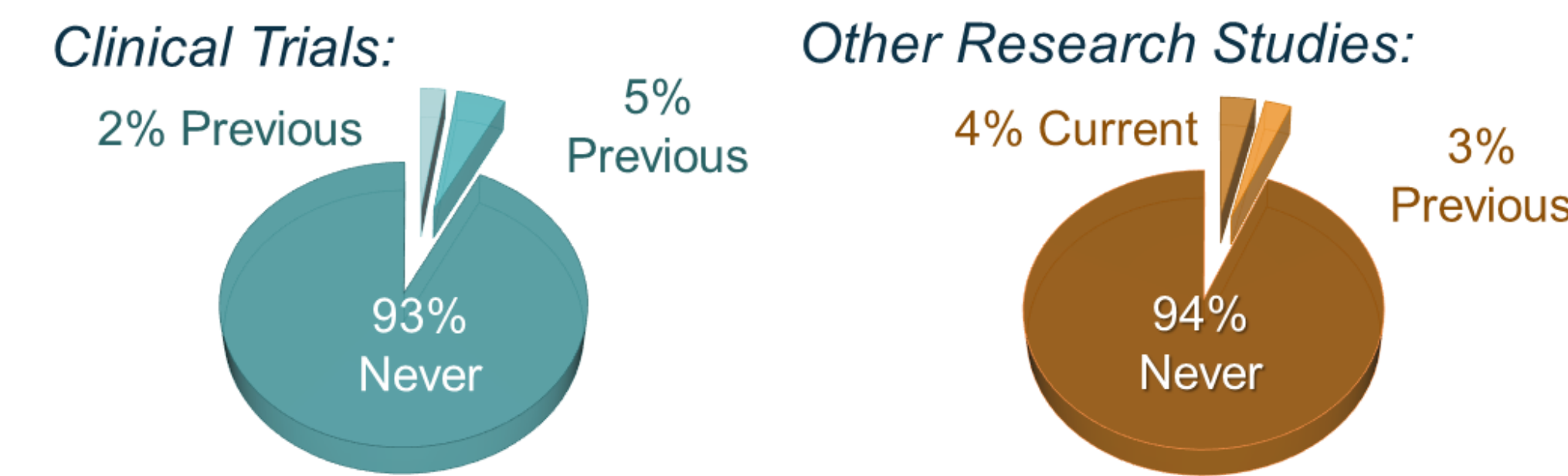


Symptoms at Diagnosis of Becker

Diagnosis occurs most often when symptoms are noticed by family members	enlarged calves	53%
	difficulty physical activities	51%
	muscle pain cramps	48%
	toe walking	42%
In comparison, ~15% of those with Duchenne had muscle pain/cramps as a presenting symptom	delays in motor development and learning	25%
	elevated liver enzymes	24%
	frequent falls	24%
	elevated CK level	15%
	delays developing language	11%
	newborn infant screen	1%
	heart problems cardiomyopathy	0%

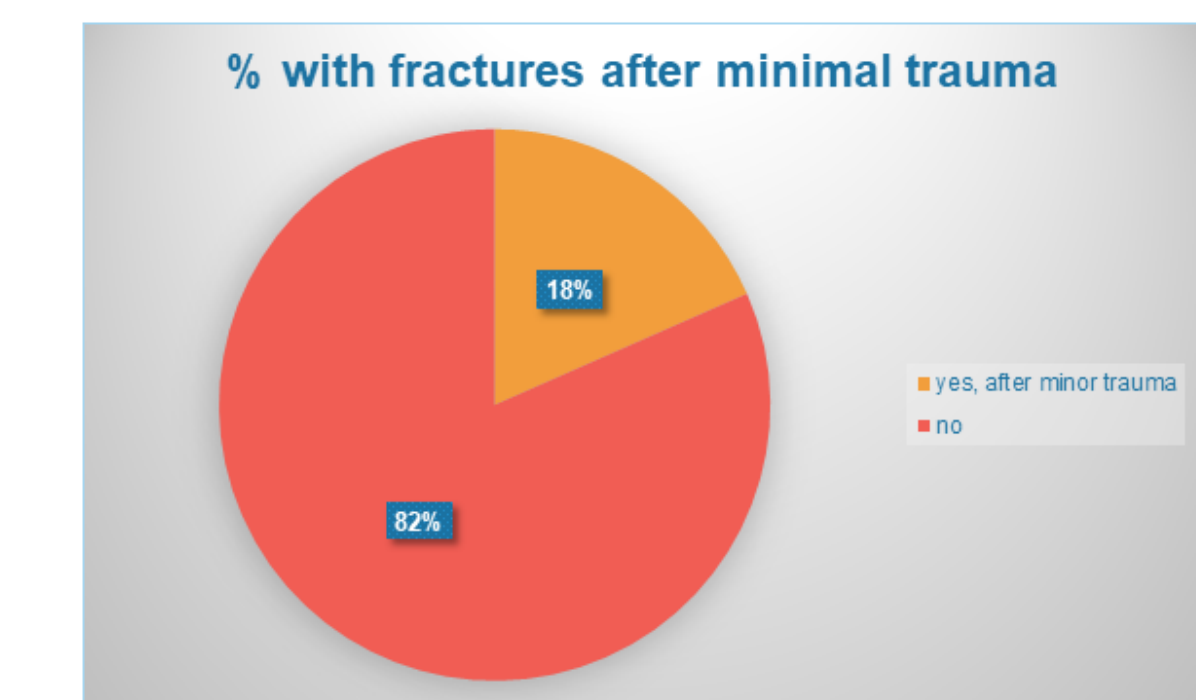
Current or Previous Clinical Trial Experience is ≤ 5% in Becker Registrants

- Only 2% of registrants were currently participating in clinical trials, with only 5% previously participating. Previous participation was similar in adults and children.
- A similar fraction reported participation in other research studies such as natural history studies

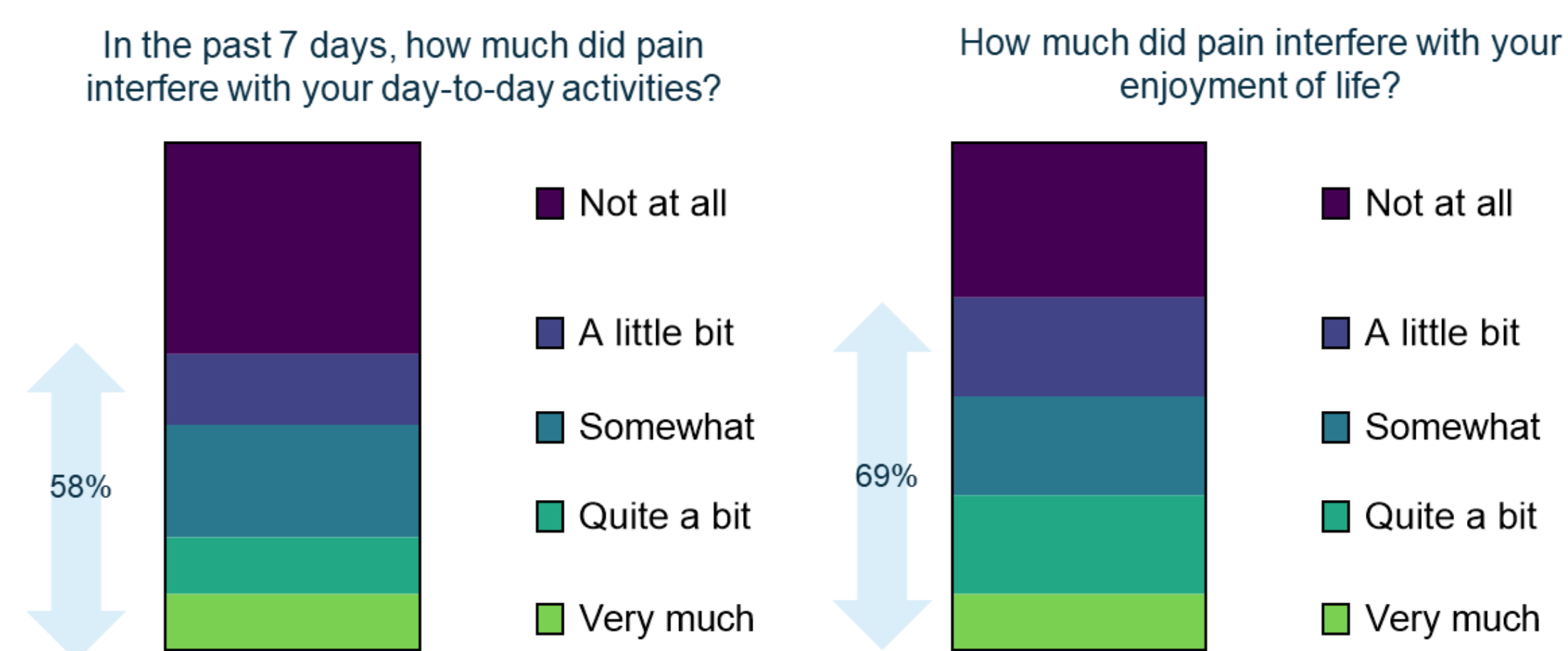


Bone Health in Becker

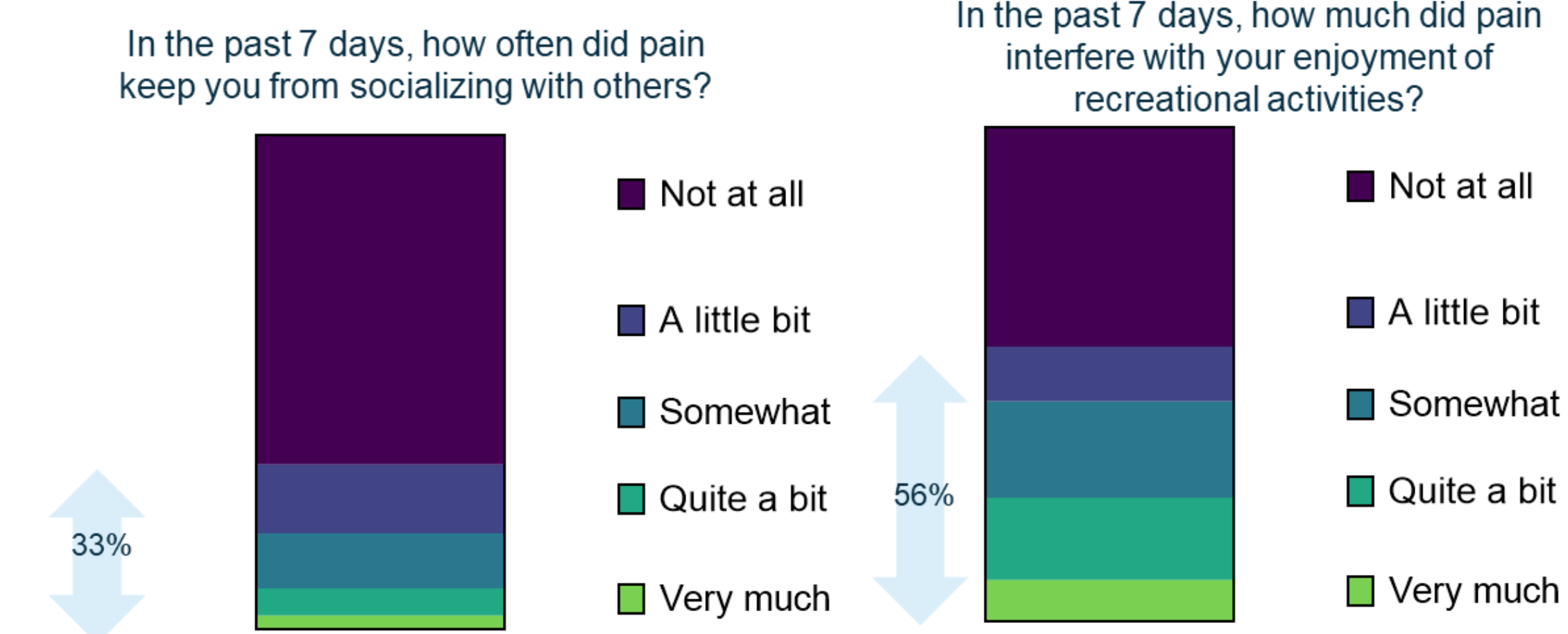
- Responses to "Have you broken a bone(s) following a minor trauma (like a simple fall) or no trauma?"
- Of the respondents who answered yes to having had a fracture, 77% had never been on corticosteroids



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

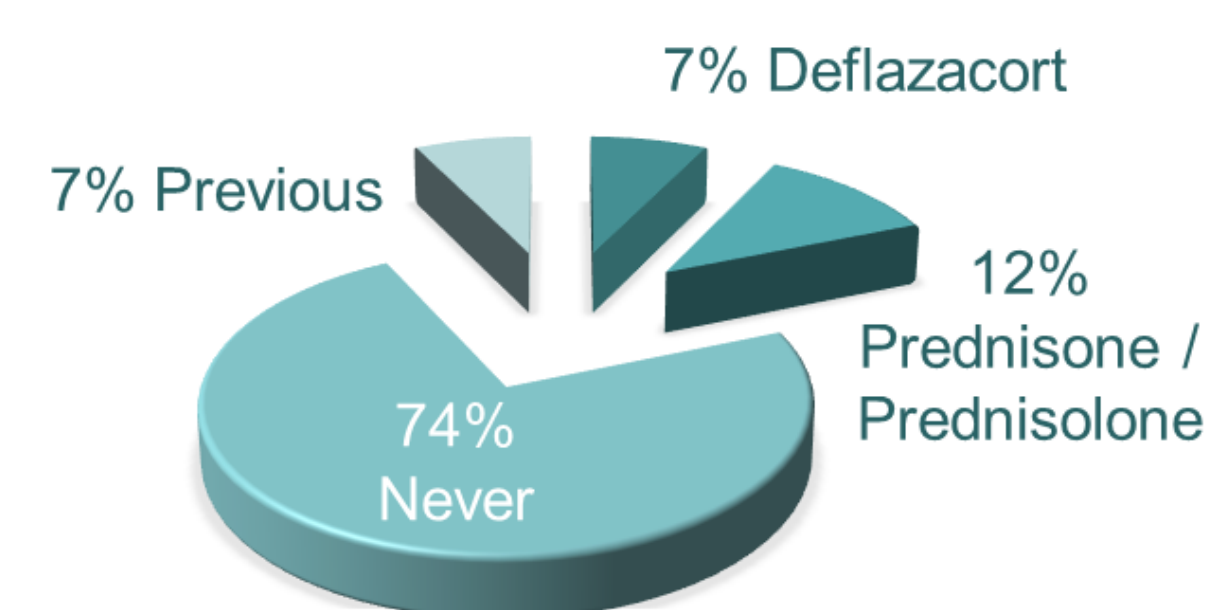


Pain Interferes with Social and Recreational Aspects of Life for Some Registrants with Becker



Thank you to the patients and their families!

Use of Corticosteroids in Becker



- 26% of respondents with Becker reported steroid use at some point.
- Median age at initiation: 6 years
- While steroid use was more common among children, 11% of adults reported current steroid use.

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 common, and not correlated with corticosteroid use
 - Despite the known side effect profile, corticosteroid use was not uncommon
 - There is an impact of pain on activities and quality of life and this deserves further investigation.
 - Survey results underscore the need for therapeutic approaches that impact both muscle function and quality of life.