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

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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 6000
- 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
  - Diagnostic: 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

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Thank you to the patients and their families!

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

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

Conclusion
- 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.