Elevating Becker Outcomes: Unlocking New Insights, Mobilizing Communities, and Redefining Care

Industry forum at the 2026 MDA Clinical & Scientific Conference

Monday March 9, 2026

Hilton Orlando, Orlando, Florida, USA

Edgewise® THERAPEUTICS

Disclosures

Roxana Donisa Dreghici is an employee of Edgewise Therapeutics and is a shareholder.

Program Overview

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VP Clinical Development
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Introduction
Roxana Donisa Dreghici, MD

Matthew Wicklund, MD, FAAN
Professor of Neurology
Vice Chair for Research
Department of Neurology
UT Health San Antonio

Becker Muscular Dystrophy Natural History
Matthew Wicklund, MD, FAAN

Abby Bronson, MBA
VP Patient Advocacy and External Innovation
Edgewise Therapeutics

Activating the Becker community: The Progress Made to Date
Abby Bronson, MBA

Amit Sachdev, MD, MS
Associate Chief Medical Officer, MSU Healthcare
Medical Director, Department of Neurology
Assistant Professor, Neuromuscular Medicine
Michigan State University

Multi-Disciplinary Care and Management of Becker
Amit Sachdev, MD, MS

This presentation is intended for HCP audiences. Sevasemten is an investigational agent that is not currently approved in any territory.

Program Objectives

- Highlight that Becker muscular dystrophy (Becker) is a serious neuromuscular disease with a large unmet need.
- Increase awareness of the published data from recent Becker natural history studies.
- Elevate clinician understanding of what the findings mean for individuals living with Becker and how it impacts care and management strategies.
- Acknowledge the progress made to date in the Becker community while also discussing the current challenges with care heard directly from the patient community.
- Increase clinician awareness of multi-disciplinary and proactive management strategies to improve care for individuals living with Becker.
- Explore additional options to continue to improve care for the Becker community.

Becker Natural History: The Latest Insights

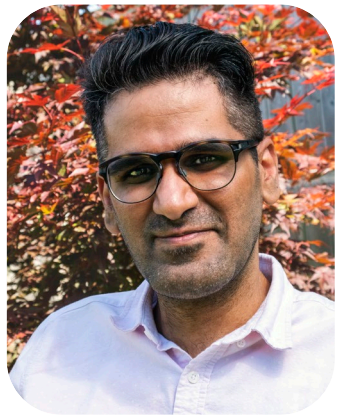
Matthew Wicklund, MD, FAAN
Professor of Neurology
Vice Chair for Research
Department of Neurology
UT Health San Antonio

Edgewise THERAPEUTICS

Disclosures

Matthew Wicklund has received research funding from Avidity Biosciences, Catalyst Pharmaceuticals, and Fulcrum Therapeutics; served on a DSMB for Applied Therapeutics and PepGen; and consulted for Amicus Therapeutics, Edgewise Therapeutics, Jazz Pharmaceuticals, Juvena Therapeutics, ML Bio Solutions, Sarepta Therapeutics, and UCB.

Becker Muscular Dystrophy Is A Serious, Progressive, and Debilitating Neuromuscular Disease



Becker is an X-linked recessive disease caused by mutations in the *DMD* gene, affecting approximately 1:18,000 male births.

Becker is a multi-systemic disease that can lead to irreversible loss of muscle function and premature death.

There are currently no approved treatments for Becker.

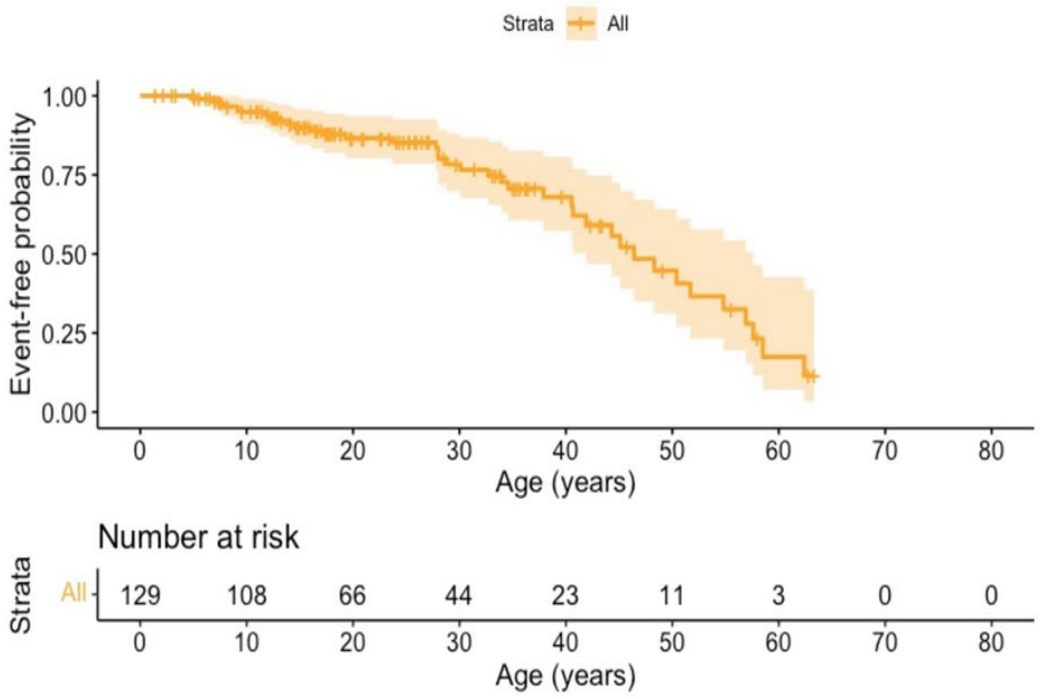
Disease management consists of symptom management and monitoring.

Becker, Becker Muscular Dystrophy
Magot A, et al. J Neurol. 2023;270(10):4763-4781.

Becker Muscular Dystrophy is a Severe Disease Characterized by Irreversible Decline in Motor Function

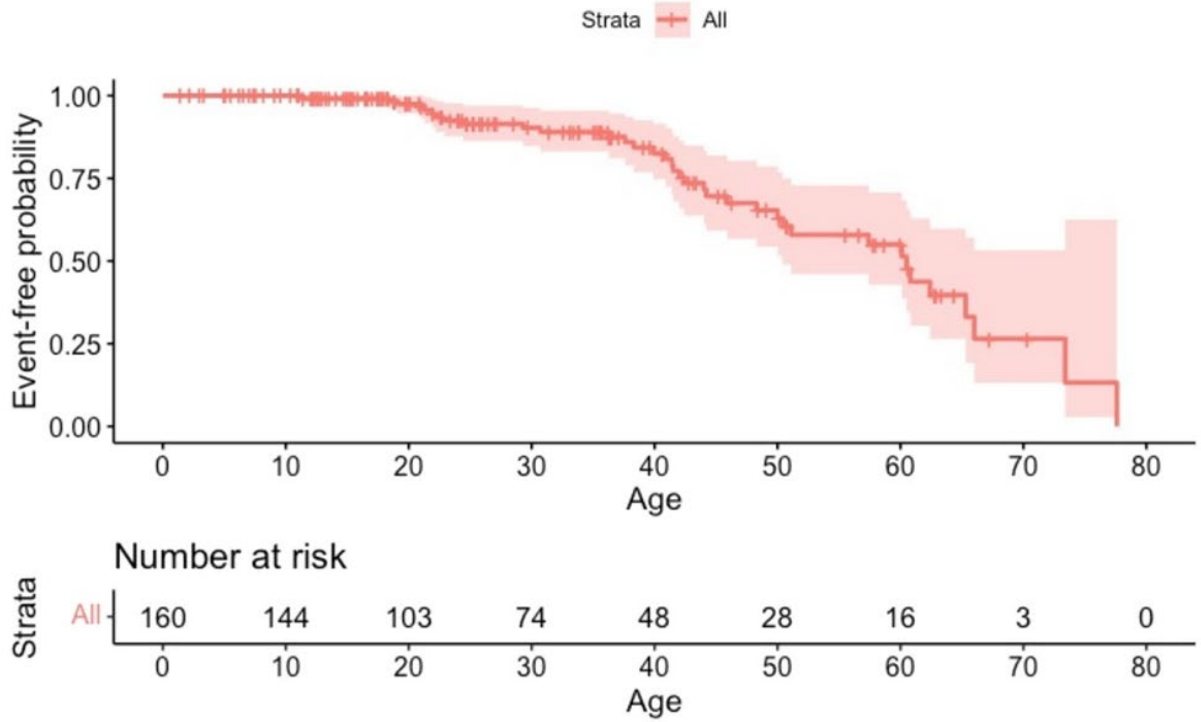
Time-To-Event Analysis of Ambulant with Aids Milestone

N = 129, events = 38, median “survival” age 46.4 years (95% CI 41.9, 57.6)
event-free probability: probability of retaining ambulation either with no limitations or with limitations but without aids



Time-to-Event Analysis of Non-Ambulant Milestone (LoA)

N = 160, events 36, median “survival” age 60.5 years (95% CI 50.4, NA)
event-free probability: probability of retaining ambulation



CI, confidence interval; LoA, loss of ambulation
Riguzzi P, et al. J Neurol. 2025;272(7):448.

Natural History Plays an Important Role in Disease Education, Patient Care, and Drug Development

A natural history study is an observational study that is designed to track the natural course of a disease.

Use of Natural History Research Examples

Disease Education

- Increase the community's understanding of disease progression

Patient Care

- Evaluate signs and symptoms of a disease to improve diagnosis
- Identify ways to improve patient care

Drug Development

- Identify appropriate populations and outcome measures
- Aid in the design of clinical trials
- Comparator to determine effectiveness of study treatment

Liu J, et al. J Clin Pharmacol. 2022;62(Suppl 2):S38-S55.

NSAA: A Well-Established And Validated Measure of Global Function

The North Star Ambulatory Assessment (NSAA) is a clinically meaningful assessment to longitudinally assess function.[1,2,3]

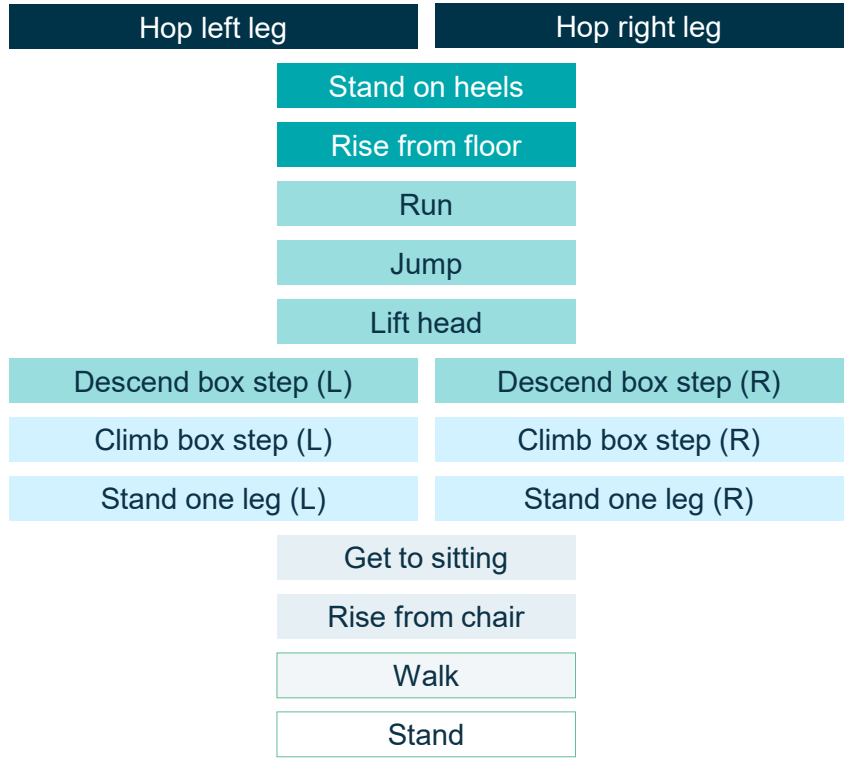
Consists of 17 items that have real world implications for patients living with muscular dystrophy.[1,3]

Each activity scores from 0 to 2 based on ability to be completed[2]:

- 0 – cannot perform
- 1 – able to perform independently with compensation
- 2 – can perform normally

Individuals can receive a total of 0-34 possible points, with 34 indicating that the individual is able to perform all 17 items normally.[2]

The North Star Ambulatory Assessment[2]



NSAA, North Star Ambulatory Assessment

1. Scott E, et al. Physiother Res Int. 2012;17(2):101-9. Epub 2011Sep3. 2. Mercuri E, et al. PLoS One. 2016;11(8):e0160195. 3. Lowes, L., et al. Poster presented at the 29th Annual Congress of the World Muscle Society; October 8-12, 2024; Prague, Czechia. #322P.

Multiple Natural History Studies in Becker Muscular Dystrophy Longitudinally Assess Function Through Exploring NSAA and Other Functional Measures

RESEARCH ARTICLE OPEN ACCESS

Longitudinal Changes of Motor Function in Becker Muscular Dystrophy

Luca Bello,[1] Pietro Riguzzi,[1] Giuliana Capece,[1] Martina Penzo,[1] Angela Petrosino,[1] Elena Sogus,[1] Sara Mastellaro,[1] Michela Caroli,[1] Matteo Villa,[1] Daniele Sabbatini,[1,2] Domenico Gorgoglione,[1] Sara Vianello,[1] Gianni Sorarù,[1] and Elena Pegoraro[1]

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Neurol Genet 2025;11:e200285. doi:10.1212/NXG.0000000000200285

RESEARCH ARTICLE OPEN ACCESS

Sensitivity of Different Clinical Outcome Measures in Assessing Adults With Becker Muscular Dystrophy

A 3-Year Natural History Study

Esther J. Schrama,[1] Zaïda Koeks,[1] Nienke M. Van De Velde,[1] Iris Alleman,[2] Jules J. van Benthem,[3] Pieteke W. van Weperen,[4] Melissa T. Hooijmans,[5,6] Hermien E. Kan,[7,8] Pietro Spitali,[9] Nina Ajmone Marsan,[10] Douwe E. Atsma,[10] Hermine A. van Duyvenvoorde,[8,11] Jan J.G.M. Verschuuren,[1] and Erik H. Niks[1,8]

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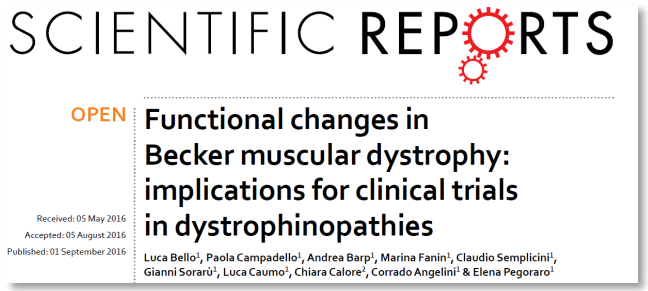
Neurology® 2025;105:e214071. doi:10.1212/WNL.0000000000214071



SCIENTIFIC REPORTS

OPEN

Functional changes in Becker muscular dystrophy: implications for clinical trials in dystrophinopathies

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Published: 01 September 2016

Luca Bello[1], Paola Campadello[1], Andrea Barp[1], Marina Fanin[1], Claudio Semplicini[1], Gianni Sorarù[1], Luca Caumo[1], Chiara Calore[2], Corrado Angelini[1] & Elena Pegoraro[1]

ORIGINAL ARTICLE

european journal of neurology

Lessons for future clinical trials in adults with Becker muscular dystrophy: Disease progression detected by muscle magnetic resonance imaging, clinical and patient-reported outcome measures

Bram De Wel[1,2] | Louise Iterbeke[2] | Lotte Huysmans[3,4] | Ronald Peeters[5] | Veerle Goosens[5] | Nicolas Dubuisson[6] | Peter van den Bergh[6] | Vinciane Van Parijs[6] | Gauthier Remiche[7] | Liesbeth De Waele[8,9] | Frederik Maes[3,4] | Patrick Dupont[10] | Kristl G. Claeys[1,2]

Findings from the Longitudinal CINRG Becker Natural History Study

Paula R. Clemens[a,*], Heather Gordish-Dressman[b], Gabriela Niizawa[a], Ksenija Gorni[c], Michela Guglieri[d], Anne M. Connolly[e], Matthew Wicklund[f], Tulio Bertorini[g], Jean Mah[h], Mathula Thangarajh[i], Edward C. Smith[j], Nancy L. Kuntz[k], Craig M. McDonald[l], Erik Henricson[l], S Upadhyayula[m], Barry Byrne[n], Georgios Manousakis[o], Amy Harper[l], Susan Iannaccone[p] and Utkarsh J. Dang[q]

Characterisation of a large, single-centre cohort of patients with Becker muscular dystrophy to inform standardised care guidelines

Pietro Riguzzi[1,2] · Holly Borland[1] · Meredith K. James[1] · John Bourke[1] · Chiara Marini Bettolo[1] · Robert Muni Lofra[1] · Jordi Diaz-Manera[1] · Giorgio Tasca[1] · Marianela Schiava[1] · Maha ElSeed[1] · Elizabeth Harris[1] · Emma Grover[1] · Chloe Geagan[1] · Carla Bolano Diaz[1] · Ariele Barreto Haagsma[1] · Doaa Salman[1] · Tara Reeves[1] · Goknur S. Kocak[1] · Emma Robinson[1] · Peter Waldock[1] · Michelle McCallum[1] · Jassi Michell-Sodhi[1] · Dionne Moat[1] · Karen Wong[1] · Ana Topf[1] · Elena Pegoraro[2] · Luca Bello[2] · Volker Straub[1] · Michela Guglieri[1]

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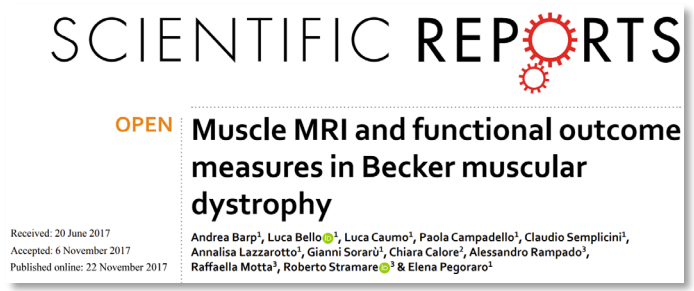

SCIENTIFIC REPORTS

OPEN

Muscle MRI and functional outcome measures in Becker muscular dystrophy

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Published online: 22 November 2017

Andrea Barp[1], Luca Bello[1], Luca Caumo[1], Paola Campadello[1], Claudio Semplicini[1], Annalisa Lazzarotto[1], Gianni Sorarù[1], Chiara Calore[2], Alessandro Rampado[3], Raffaella Motta[3], Roberto Stramare[3] & Elena Pegoraro[1]

NSAA, The North Star Ambulatory Assessment

1. Bello L, et al. Sci Rep. 2016;6:32439. 2. De Wel B, et al. Eur J Neurol. 2024;31(7):e16282. 3. Bello L, et al. Neurol Genet. 2025;11(4):e202285. 4. Schrama EJ, et al. Neurology. 2025;10597):e214071. 5. Riguzzi P, et al. J Neurol. 2025;272(7):448. 6. Barp A, et al. Sci Rep. 2017;7(1):16060. 7. Clemens PR, et al. J Neuromuscul Dis. 2024;11(1):201-212.

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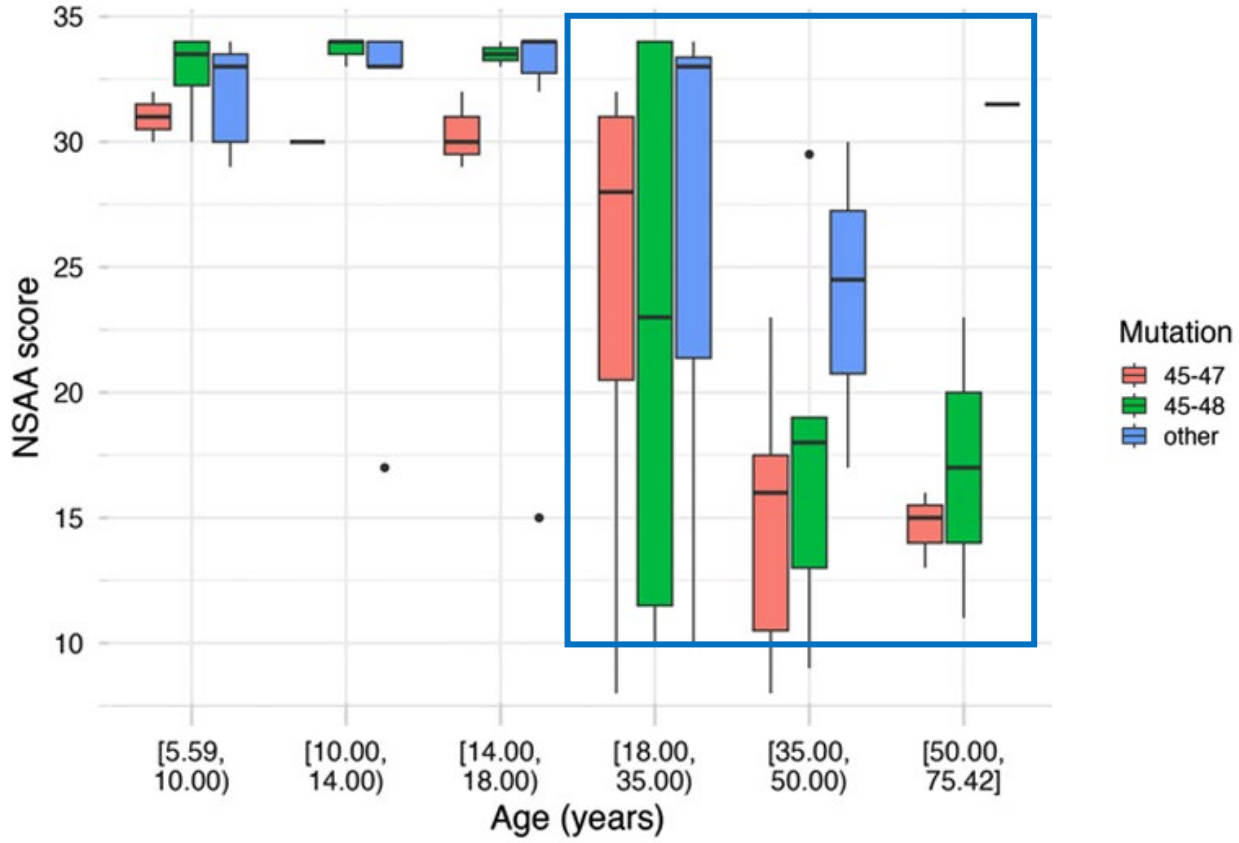
NSAA Score Decreases with Increasing Age After Age 18

- One median observation per participant per age interval used to analyze NSAA
- For most participants less than 18 year, NSAA scores were near top of the scale.
- The NSAA score decreased with increasing age after age 18 years.

Boxplot of Median NSAA Per Participants Stratified by Age Group & Mutation Status



NSAA, North Star Ambulatory Assessment
Clemens PR, et al. J Neuromuscul Dis. 2024;11(1):201-212.

Trajectory in NSAA Shows Progressive Decline


RESEARCH ARTICLE OPEN ACCESS

Longitudinal Changes of Motor Function in Becker Muscular Dystrophy

Luca Bello,[1] Pietro Riguzzi,[1] Giuliana Capece,[1] Martina Penzo,[1] Angela Petrosino,[1] Elena Sogus,[1] Sara Mastellaro,[1] Michela Caroli,[1] Matteo Villa,[1] Daniele Sabbatini,[1,2] Domenico Gorgoglione,[1] Sara Vianello,[1] Gianni Sorarù,[1] and Elena Pegoraro[1]

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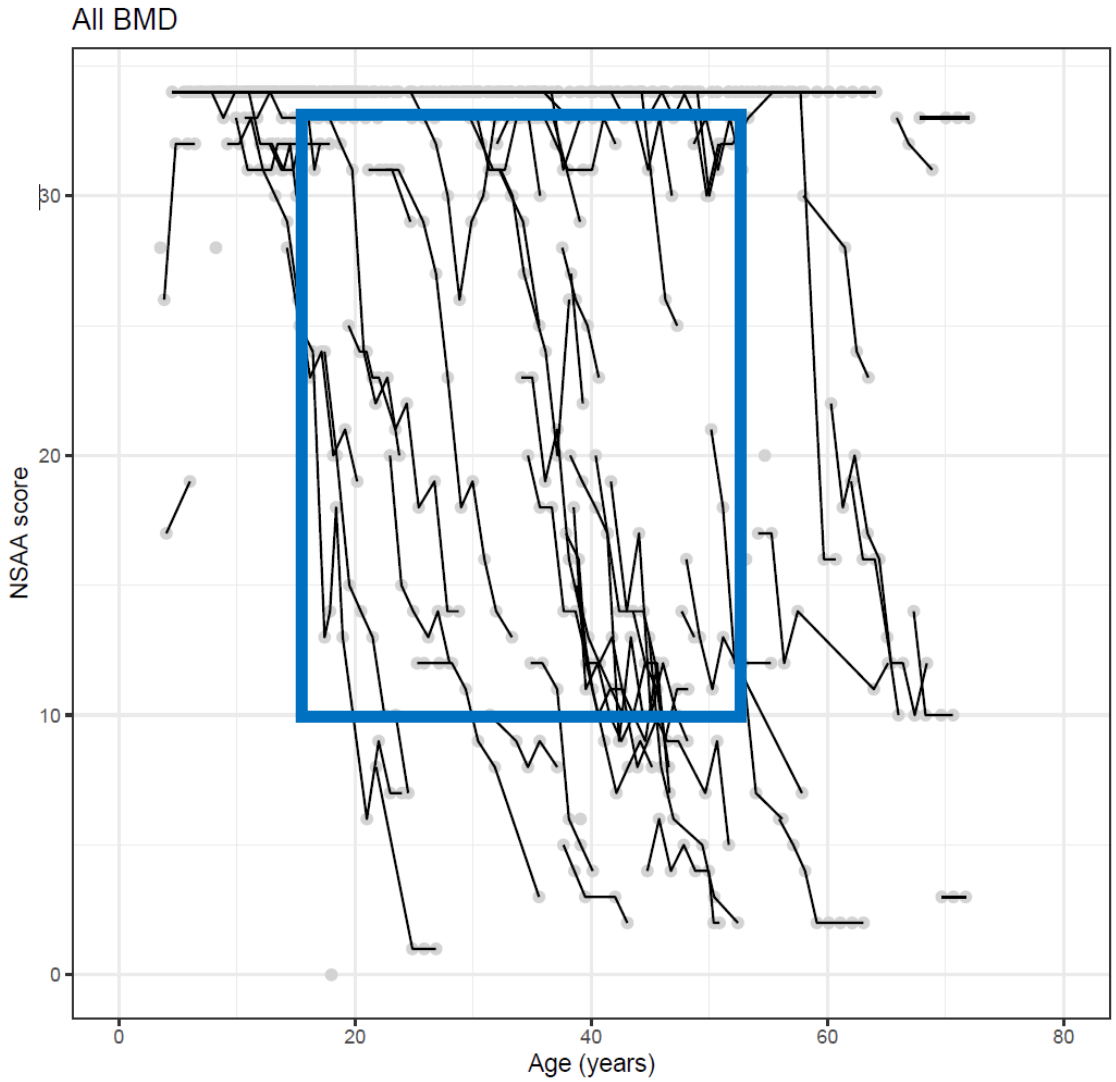
Neurol Genet 2025;11:e200285. doi:10.1212/NXG.0000000000200285



- 100 ambulatory participants were followed annually for 6.1 ± 3.1 years
- Those with NSAA between 10 and 32 (n=36, 233 observations) had an annual decrease of 1.0 points
- A clear ceiling effect evident with the annual decrease for those with NSAA 33-34 of -0.05 points
- In this Italian study, 49% of adults had NSAA of 33-34, reflecting the disproportional number of individuals with minimally progressive mutations due to routine CK testing practices

CK, creatine kinase; NSAA, The North Star Ambulatory Assessment
Bello L, et al. Neurol Genet. 2025;11(4):e200285.

Fastest, Most Significant Decline in Patients with Baseline NSAA Scores 10-32


RESEARCH ARTICLE OPEN ACCESS

Longitudinal Changes of Motor Function in Becker Muscular Dystrophy

Luca Bello, Pietro Riguzzi, Giuliana Capece, Martina Penzo, Angela Petrosino, Elena Sogus, Sara Mastellaro, Michela Caroli, Matteo Villa, Daniele Sabbatini, Domenico Gorgoglione, Sara Vianello, Gianni Sorarù, and Elena Pegoraro

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Neurol Genet 2025;11:e200285. doi:10.1212/NXG.0000000000200285

Table 4 Linear Mixed Model Parameters for Yearly NSAA Change in Participants Grouped by Baseline NSAA Score

Baseline NSAA score	n >18 y with longitudinal evaluations	n (evaluations)	Estimate of yearly change	SE	*p* Value
All	89	504	−0.64	0.04	<0.0001
34	36	193	−0.04	0.01	0.0043
33	8	44	−0.01	0.04	n.s
30–32	8	52	−0.95	0.15	<0.0001
25–29	4	26	−1.40	0.18	<0.0001
20–24	9	72	−1.28	0.08	<0.0001
15–19	8	49	−0.68	0.10	<0.0001
10–14	7	34	−0.96	0.12	<0.0001
5–9	4	17	−0.01	0.04	n.s
0–4	5	16	−0.27	0.12	0.048

Abbreviations: NSAA = North Star Ambulatory Assessment; SE = standard error.
p Values pertain to longitudinal evaluations performed in adults (older than 18 years) and were estimated with the Satterthwaite method from linear mixed models, including all evaluations.

Becker, Becker muscular dystrophy; NSAA, North Star Ambulatory Assessment; n.s, not significant; SE, standard error
Bello L, et al. Neurol Genet. 2025;11(4):e200285.

NSAA Decline Highly Variable Across Genotypes


RESEARCH ARTICLE OPEN ACCESS

Longitudinal Changes of Motor Function in Becker Muscular Dystrophy

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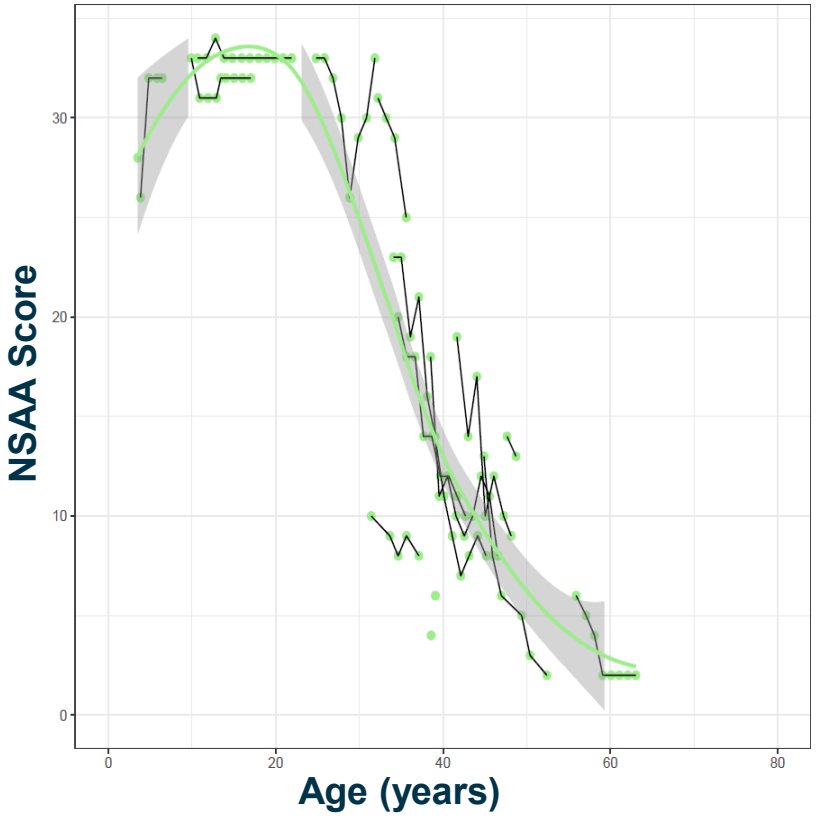
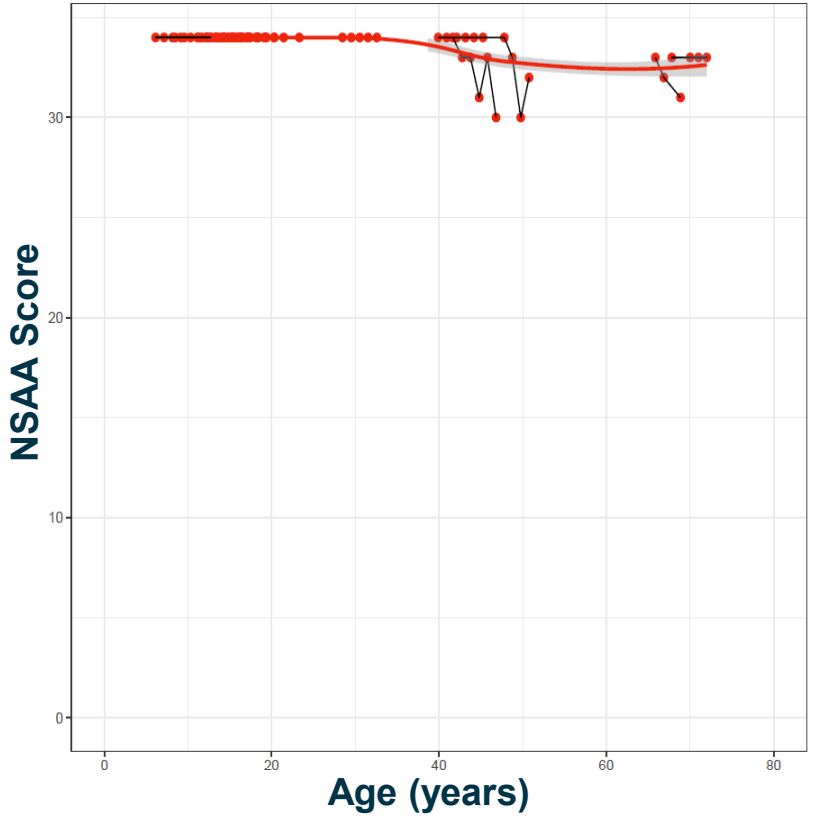
Neurol Genet 2025;11:e200285. doi:10.1212/NXG.0000000000200285

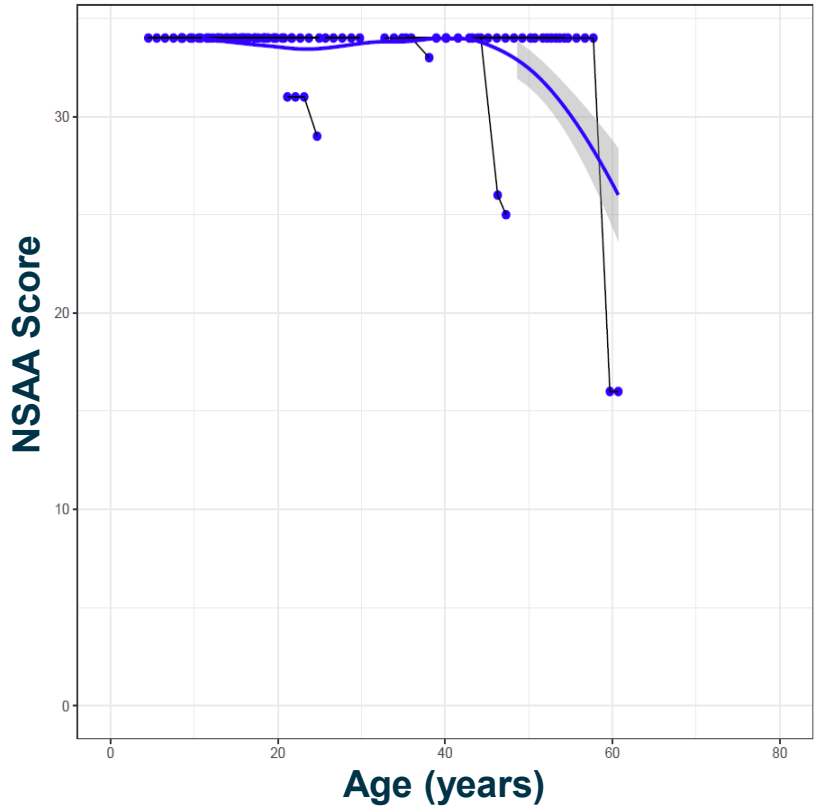
Deletions 45-47
(n=14)



Deletions 48
(n=12)



Deletions x-51
(n=15)



NSAA, North Star Ambulatory Assessment
Bello L, et al. Neurol Genet. 2025;11(4):e200285.

Select Genotypes have Minimal Disease Progression and can be Largely Identified by Exclusion of NSAA 33-34

- In Italy, CK is routinely measured, resulting in a disproportionate number of patients identified with very mild disease courses; two of these genotypes, deletions ending in exon 51 and deletions of exon 48 have minimal annual NSAA decline (-0.05 points/year)

- These genotypes are uncommon elsewhere

Genotype	Italy (Bello, 2025)		Netherlands (Schrama, 2025)	US / Canada / UK (Clemens, 2024)	UK (Riguzzi, 2025)
	Annual NSAA change	N=89	N=36	N=83	N=155
Very slowly progressing					
X-51	-0.08	12		3	5
Del 48	-0.03	15	--	0	2
Overall		32 (36%)	0 (0%)	3 (4%)	7 (5%)
Typically progressing		57 (64%)	36 (100%)	80 (97%)	155 (95%)

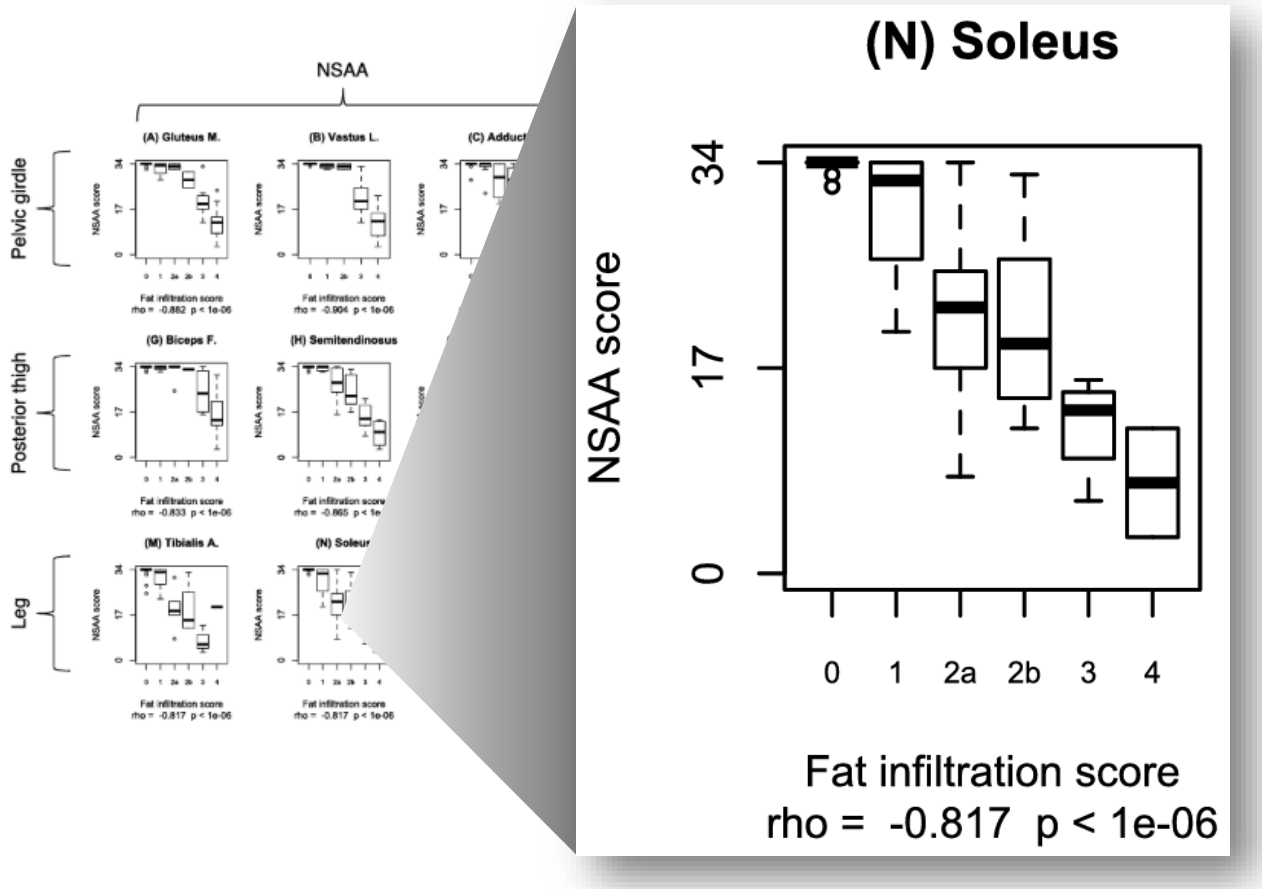
NSAA, North Star Ambulatory Assessment

1. Bello L, et al. Neurol Genet. 2025;11(4):e200285. 2. Riguzzi P, et al. J Neurol. 2025;272(7):448. 3.Schrama, EJ, et al. Neurology. 2025;105(7): e214071. 4. Clemens PR, et al. J Neuromuscul Dis. 2024;11(1):201-2012.

MRI Data Supports that Fat Infiltration Increases in the Lower Limbs as NSAA Scores Decrease

- 51 Becker patients underwent muscle MRI and were evaluated with functional measures at time of MRI
- Various lower limb muscles were assessed
- There was an increase in fat infiltration as NSAA score decreased, as shown in the Soleus boxplot for example
- **Fat infiltration scores correlated with baseline NSAA**

Boxplots of NSAA scores by T1w Mercuri Scores in 9 Lower Limb Muscles

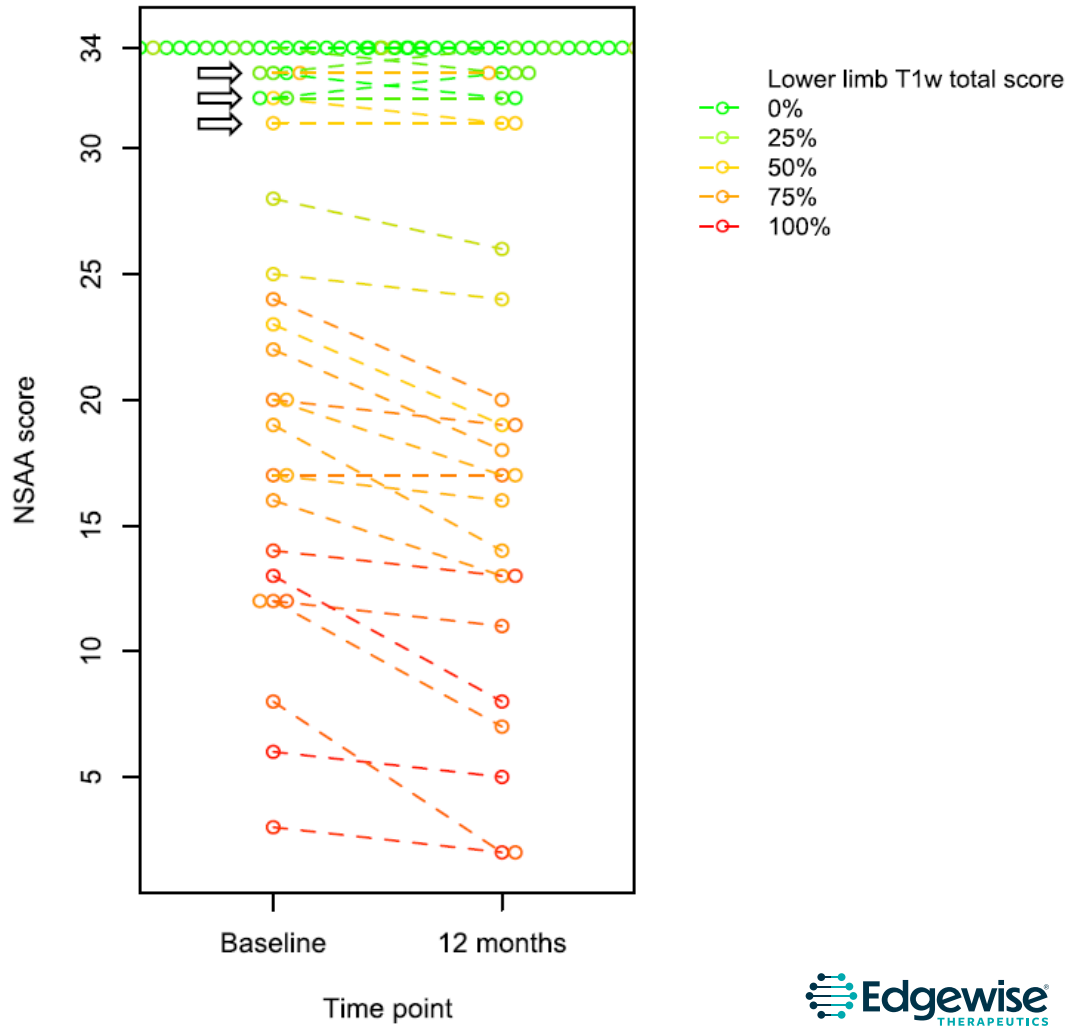


Becker, Becker muscular dystrophy; NSAA, North Star Ambulatory Assessment
Barp A, et al. Sci Rep. 2017;7(1):16060.

Individual MRI Data of Lower Limb Scores Supports that Baseline NSAA can Predict Changes after 1 Year, with Increased Decline at Lower NSAA Scores

- **Baseline NSAA ≤30:** 50% – 100% lower limb T1w total score at baseline and after 1 year
- **Baseline NSAA above 30:** 0-50% lower limb T1w total score at baseline and after 1 year
- Those who started at a lower baseline NSAA score experienced increased fat infiltration after 1 year
- **Baseline NSAA can predict changes after 1 year, with increased decline in those with lower baseline NSAA scores**

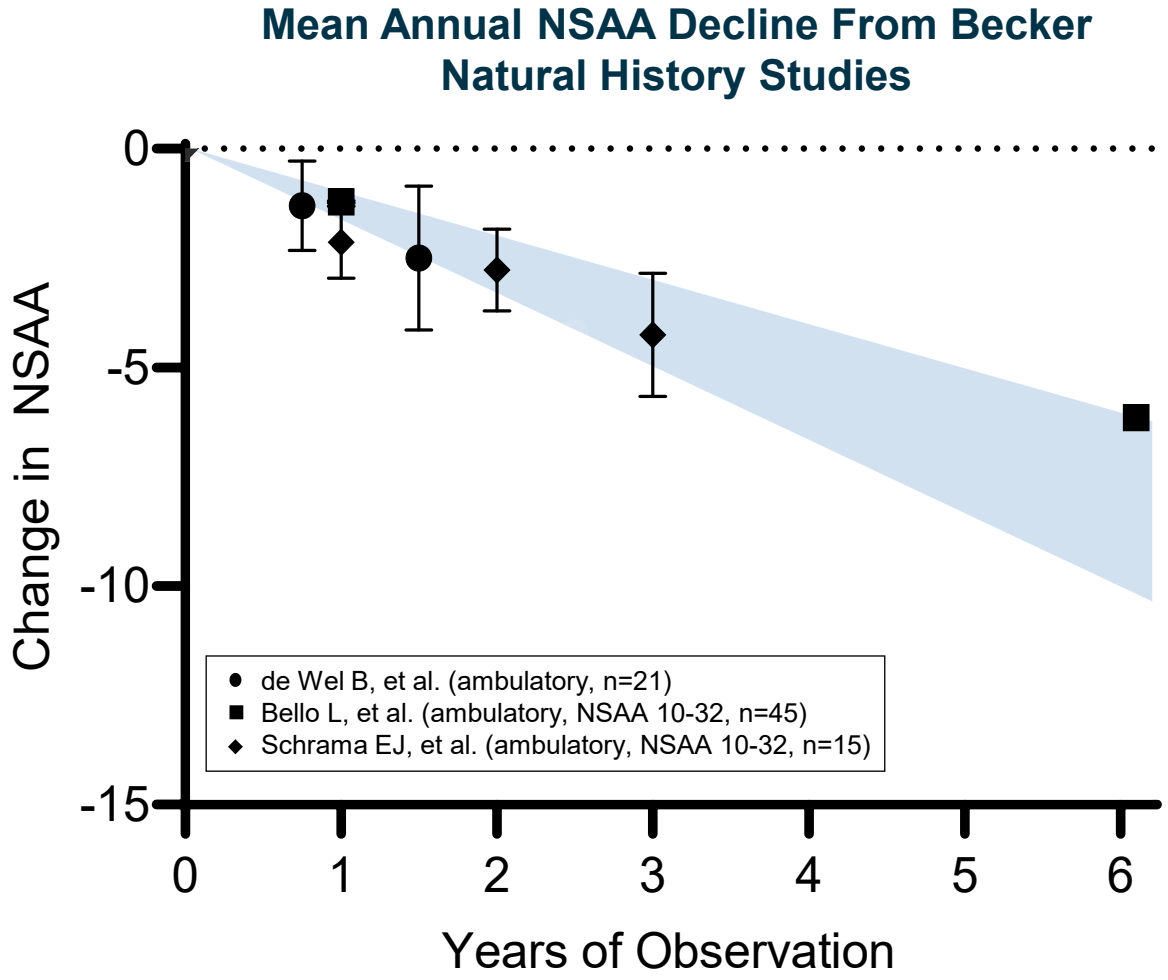
NSAA scores at baseline and after 1 year



Lower limb muscles analyzed were from the pelvic girdle, posterior thigh, and leg
NSAA, North Star Ambulatory Assessment
Barp A, et al. Sci Rep. 2017;7(1):16060

Combined Natural History Studies Show Mean Annual Rates of Decline in NSAA between 1.0 and 1.7 Points

- Multiple natural history studies in individuals with Becker demonstrate a **NSAA average score decline of 1.0 to 1.7 points annually**[1,2,3,4]
- Becker natural history studies support that NSAA decline is consistent in Becker patients who are already progressing
- Slowing or stabilizing disease progression is an important and urgent goal for Becker patients



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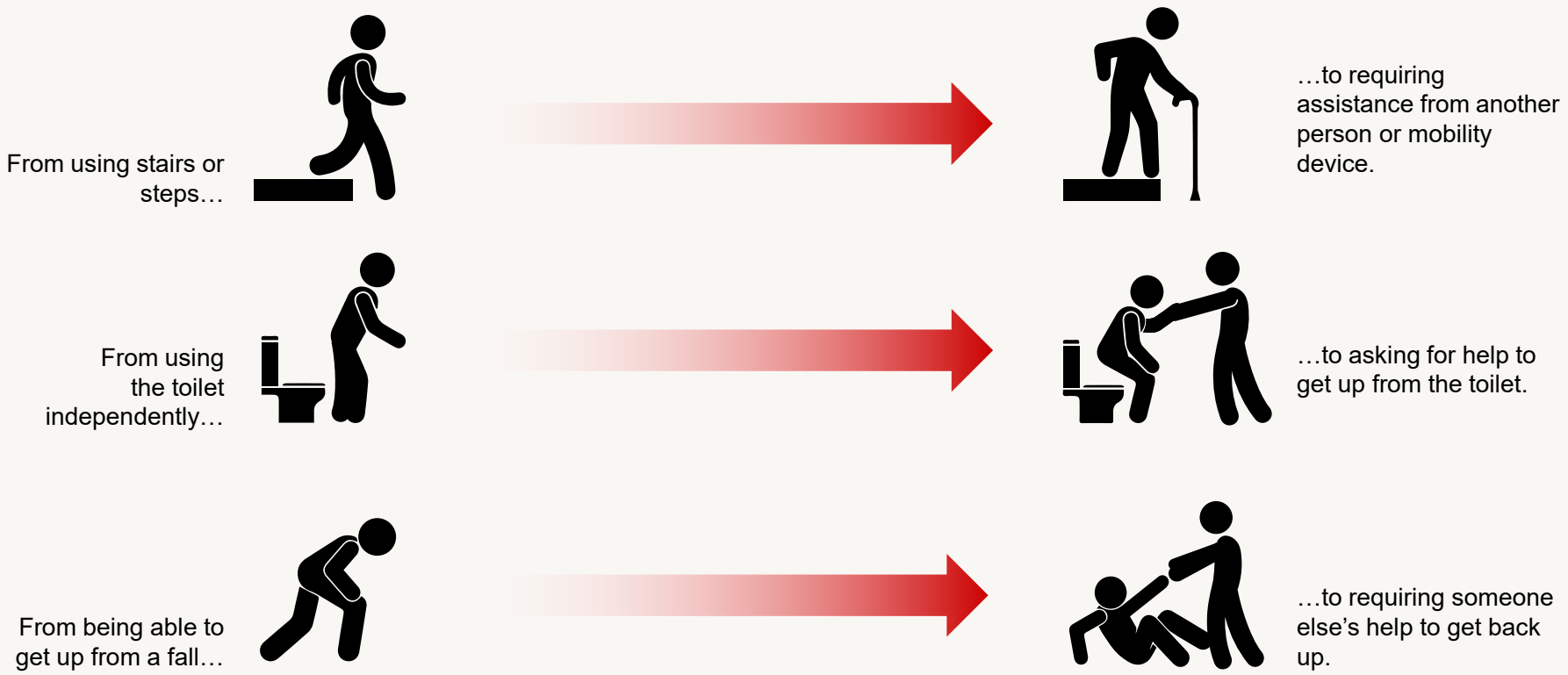
How a 1-Point NSAA Change in Becker Could Be Interpreted

NSAA decreases can relate directly to loss of a motor ability or need for compensation to perform activities independently.[1] For example, **this decline could look like:**



NSAA, North Star Ambulatory Assessment

1. Bello L, et al. Sci Rep. 2016;6:32439.

Key Takeaways

Becker muscular dystrophy is a **serious, progressive, and debilitating** muscular dystrophy.

The NSAA is a **clinically meaningful functional measure** utilized in Becker natural history studies to longitudinally assess function.

Natural history studies show that Becker patients experience an average decline in NSAA between **1.0 and 1.7 points annually,** with those who have a baseline NSAA score of 10-32 demonstrating the greatest range of decline.

Slowing or stabilizing disease progression is important and urgent goal in Becker muscular dystrophy.

Becker, Becker muscular dystrophy; NSAA, North Star Ambulatory Assessment

Activating the Becker Community: The Progress Made to Date

Abby Bronson, MBA
VP, Patient Advocacy and External Innovation
Edgewise Therapeutics

Edgewise THERAPEUTICS

Overview of the Becker Landscape ~10 Years Ago

Clinical Research

- Standards of care and guidelines solely dedicated to Becker did not exist
- Minimal published natural history study data available
- Very few clinical trials inclusive of only Becker patients

Healthcare Providers

- Low levels of awareness and understanding of Becker
- Minimal tools solely dedicated to Becker that clinicians could use to support patient care (i.e. rating scales, etc.)
- Minimal distinguishment of Becker muscular dystrophy from Duchenne muscular dystrophy and perception of “mild”

Patient Community

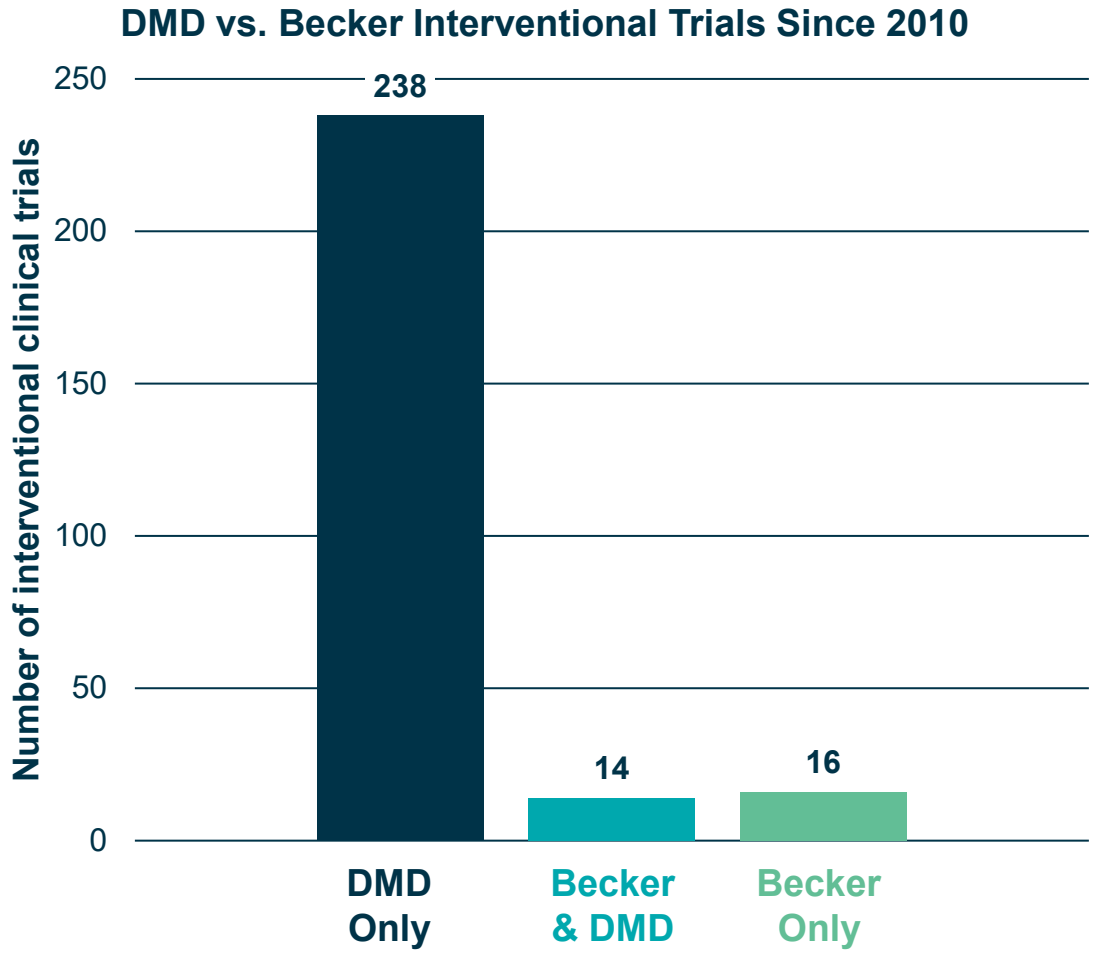
- No dedicated Becker patient advocacy organization
- Minimal education initiatives solely dedicated to the Becker community
- Patient community did not have a strong, differentiated identity, leading to feelings of being “ignored” and “forgotten”

Becker, Becker muscular dystrophy

Clinical Trials Since 2010

Recent search of clinicaltrials.gov showed that:

- First clinical trial inclusive of only Becker participants was initiated in 2010
- Since then, there have been 16 interventional studies dedicated to Becker
- In comparison, there have been many DMD trials as seen in the figure on the right

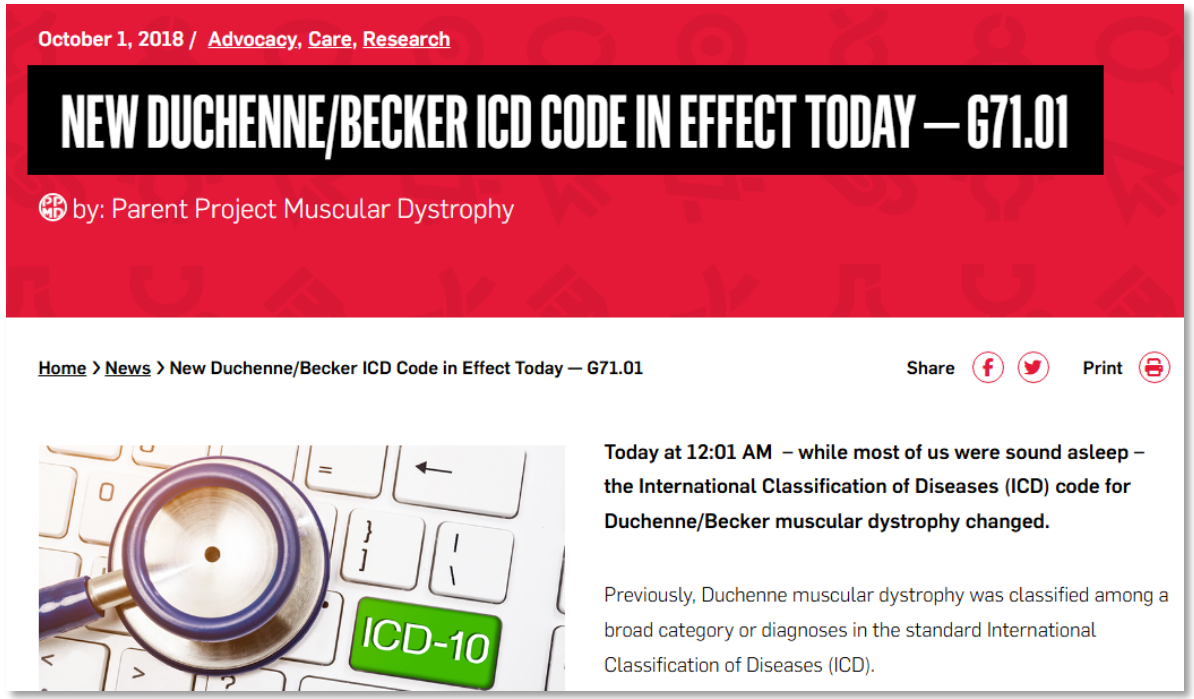


Becker; Becker muscular dystrophy; DMD; Duchenne muscular dystrophy
Clinicaltrials.gov. Accessed 27 Jan 2026.

Acceptance of ICD10 Code for Duchenne/Becker and Proposal of ICD11 Codes Driven by Healthcare and Advocacy Communities

Healthcare Providers

- With increasing awareness of muscular dystrophies, the healthcare and advocacy community came together to drive the acceptance of a new ICD10 code for Duchenne/Becker Muscular Dystrophy.[1]
- ICD11 codes for all muscular dystrophies, including one specific to Becker, are also used by the World Health Organization (WHO), but they are not currently implemented in the US[2]



October 1, 2018 / Advocacy, Care, Research

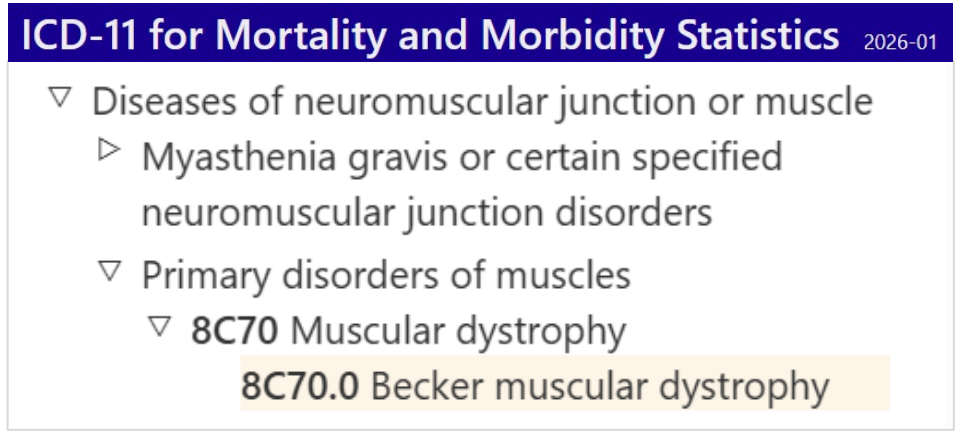
NEW DUCHENNE/BECKER ICD CODE IN EFFECT TODAY — G71.01

by: Parent Project Muscular Dystrophy

Home > News > New Duchenne/Becker ICD Code in Effect Today — G71.01 Share Print

Today at 12:01 AM – while most of us were sound asleep – the International Classification of Diseases (ICD) code for Duchenne/Becker muscular dystrophy changed.

Previously, Duchenne muscular dystrophy was classified among a broad category or diagnoses in the standard International Classification of Diseases (ICD).



Becker, Becker muscular dystrophy; ICD, International Classification of Diseases

1. New Duchenne/Becker ICD Code in Effect Today. Parent Project Muscular Dystrophy. Updated 02 Oct 2018. Accessed 27 Jan 2026. https://www.parentprojectmd.org/new-duchenne-becker-icd-code-in-effect-today-g71-01/. 2. International Classification of Diseases 11th Revision. ICD-11 Browser. Accessed 22 Feb 2026. https://icd.who.int/en/.

Community Building Starts with Data Collection

Patient and caregiver (n=14) interviews were conducted to develop an in-depth understanding of the experiences, needs, and challenges of living with Becker.

Key Findings

All expressed that living with BMD has had an effect on their mental health and on those around them

Financial impacts were common; navigating insurance, unemployment and home accessibility

Careful consideration and planning becomes part of daily life impacting QoL, travel, visiting family, etc.

Pain Points

Absence of Becker-specific community for support once diagnosed

Lack of information/education for primary care providers on signs and symptoms of Becker

- adult living with Becker

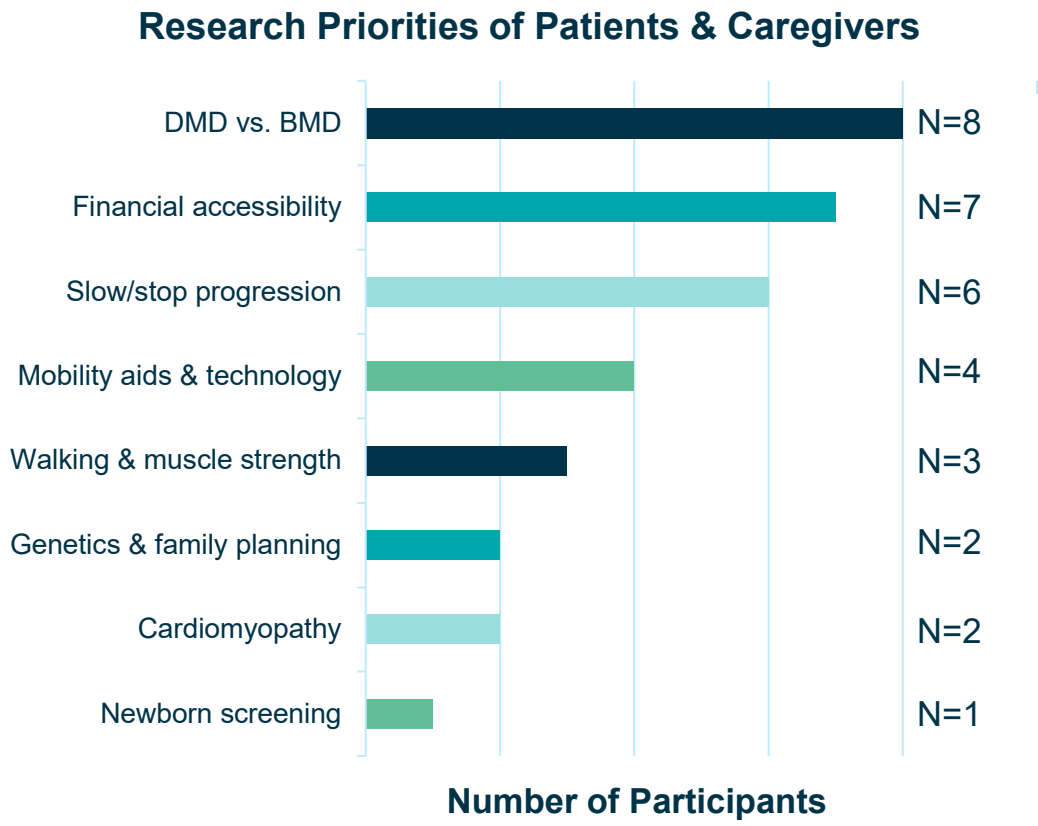
Becker, Becker muscular dystrophy; Duchenne, Duchenne muscular dystrophy

1. Bronson et al. Poster presented PPMD; Jun 2022; Scottsdale, Arizona. 2. Bronson et al. Poster presented at NORD; April 2023

“Mild Duchenne” Myth: Becker Deserves As Much Care and Support

Patient Community

The 14 participants were asked what area they wish had more investment in research & what successful management of Becker meant to them:



“**I was told you’re lucky you don’t have Duchenne, and yes, I feel bad for them, but also frustrating** that you live longer and live longer… You have to live with this long-term… .”

– adult living with Becker

“The hardest part [about living with Becker] is **losing the ability to do things I really enjoy doing**...”

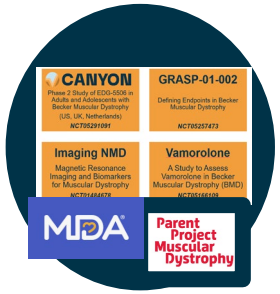
– adult living with Becker

“They are trying to turn Duchenne into Becker, but then they don’t focus on Becker.”

– caregiver

BMD, Becker muscular dystrophy; DMD, Duchenne muscular dystrophy
Data on File.

Pre-Competitive Collaboration Created to Operationalize our Collective Learnings, Spurring Progress In Becker Research, Education, and Patient Support



Study investigators and PAGs united to discuss recruitment challenges

- Do not “know they need help until they do”
- Ignored for years/lost faith
- Don’t understand natural history of disease and inevitable severity
- Naïve to clinical research
- Men less likely to seek healthcare

Potential solutions brainstormed – What was doable?

- Educate community
- Prioritize Becker and provide dedicated resources
- Develop Standards of Care for Becker

Becker Education and Engagement Day (BEED) was created

BEED was created to increase education about Becker and build a Becker community

Becker, Becker muscular dystrophy
Data on File.

US Becker Education and Engagement Day (BEED) Quickly Grew and Engaged a Disconnected Community

217% growth in attendance over the last 3 years

200+ influential speakers & key specialists active in Becker care

30+ hospitals & institutions involved

Elias and Jay, Becker Patient Advocates

Becker, Becker muscular dystrophy; BEED, Becker Education and Engagement Day

Becker Education and Engagement Day. Accessed 26 Jan 2026. https://beckereducationandengagement.com/

Today, Immense Progress for the Becker Community Has Been Made

Clinical Research

- Multiple natural history studies underway
- Interventional trials underway or in planning
- Endpoint development and validation

Healthcare Providers

- Increasing awareness and understanding of Becker, and as a distinct disease separate from Duchenne
- Initial work to develop standards of care for Becker

Patient Community

- Increasing Becker patient advocacy initiatives
- Increasing number of education initiatives solely dedicated to the Becker community
- A strong Becker patient community is starting to emerge

Becker, Becker muscular dystrophy; Duchenne, Duchenne muscular dystrophy

Despite the Progress, Unmet Needs Remain, Especially in Terms of Becker Management and Care

- A survey published in 2024 of 50 patients and caregivers was conducted to explore the lived experience and patient journey of those living with Becker[1]
- Survey topics included questions on various aspects of Becker, such as diagnostic journey, signs and symptoms, current care and management, quality of life impacts, etc.[1]
- Results highlighted many challenges and gaps, especially the need for consistent care and management of Becker.[1]

> ***"… I could have been diagnosed sooner."***
> — patient survey response[2]

Majority reported experiencing pain and fatigue, but **management for these were reported to be primarily self-driven**.[1]

98% of respondents reported receiving cardiac testing, however the types and frequency of testing varied greatly.[1]

> ***"[Becker] needs to be treated as its own disease as it is severe in its own way."***
> – patient survey response[2]

Becker, Becker muscular dystrophy

1. Bronson A, et al. Poster presented at WMS; 2024 Oct 8-12; Prague, Czechia. #708LBP. 2. Data on File.

Becker Care and Management Needs for Patients Emerged as a Major Theme at BEED

Based on community feedback, the recent 2025 BEED program included panel discussions about **building an appropriate care team**.

Neurology, cardiology, physical therapy, and occupational therapy were seen as major areas of Becker care.

Attendees expressed a desire to connect with providers who are knowledgeable about Becker to **receive consistent and appropriate care**.

The community wants to learn more about **current research and treatment/management options**.

> "This event was the only time I've ever been presented with a clear understanding of how Becker differs from Duchenne, **and what changes to care need to be made."**
>
> – Individual living with Becker & BEED attendee

Becker, Becker muscular dystrophy; BEED, Becker Education and Engagement Day

Key Takeaways

Looking back, support and research solely for the Becker muscular dystrophy community was minimal.

Immense progress has been made to increase awareness and understanding of the disease and to build a strong and connected Becker community.

We need to keep the momentum going and continue to fill in the gaps that we are hearing from patients today, such as with consistent care and management.

Multi-Disciplinary Care and Management of Becker

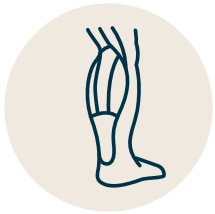
Amit Sachdev, MD, MS
Associate Chief Medical Officer, MSU Healthcare
Medical Director, Department of Neurology
Assistant Professor, Neuromuscular Medicine
Michigan State University

Edgewise THERAPEUTICS

Disclosures

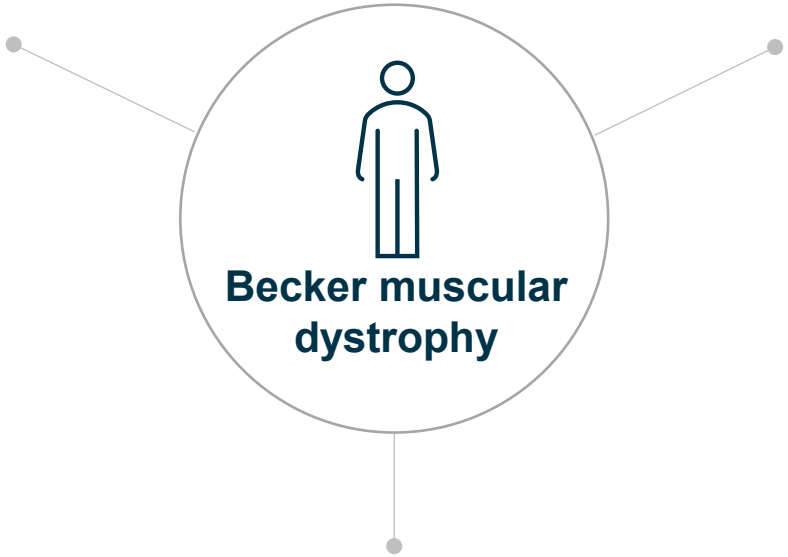
- Boards and Organizations
  - Trustee to the board of the Myasthenia Gravis Association of Michigan
  - MSMS Health Equity Task Force
  - MDHHS Behavioral Health Advisory Council (former)
  - Ingham County Health Department Health Equity Council
  - MHA – Keystone Center Board
- Granting
  - Eisai
  - Muscular Dystrophy Association Clinical Center of Excellence
  - UCB
  - Alexion
  - Catalyst Pharmaceuticals
  - Argenx
  - Dianthus
  - Regeneron
  - Remegen
- Consulting and Speakers Bureaus
  - Argenx
  - Alexion
  - Amgen
  - Johnson and Johnson
  - Takeda
  - CSL Behring
  - Catalyst
  - Edgewise

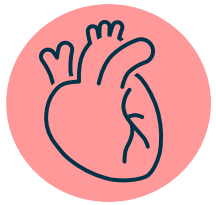
Becker Muscular Dystrophy is a Serious, Multi-Systemic Disease



Musculoskeletal

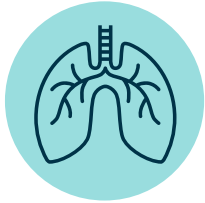
Ongoing contraction-induced muscle injuries leads to irreversible loss of function.[2]





Cardiovascular

Frequency of cardiac involvement in Becker is 60-70%.[1]



Other Systems

Becker patients can experience other symptoms, including those related to respiratory, bone, neuropsychiatric, gastric, and urinary systems.[3]

Becker, Becker muscular dystrophy

1. Ho R, et al. World J Cardiol. 2016;8(6):356-61. 2. Aartsma-Rus A, et al. J Med Genet. 2016;53(3):145-51. 3. Magot A, et al. J Neurol. 2023; 270(10):4763-4781.

2025 UK Natural History Study Found That the First Concerns for Becker Patients Included Muscle, Cardiac, and Neuropsychiatric Symptoms

Diagnostic Delay Based on Nature of the First Concern

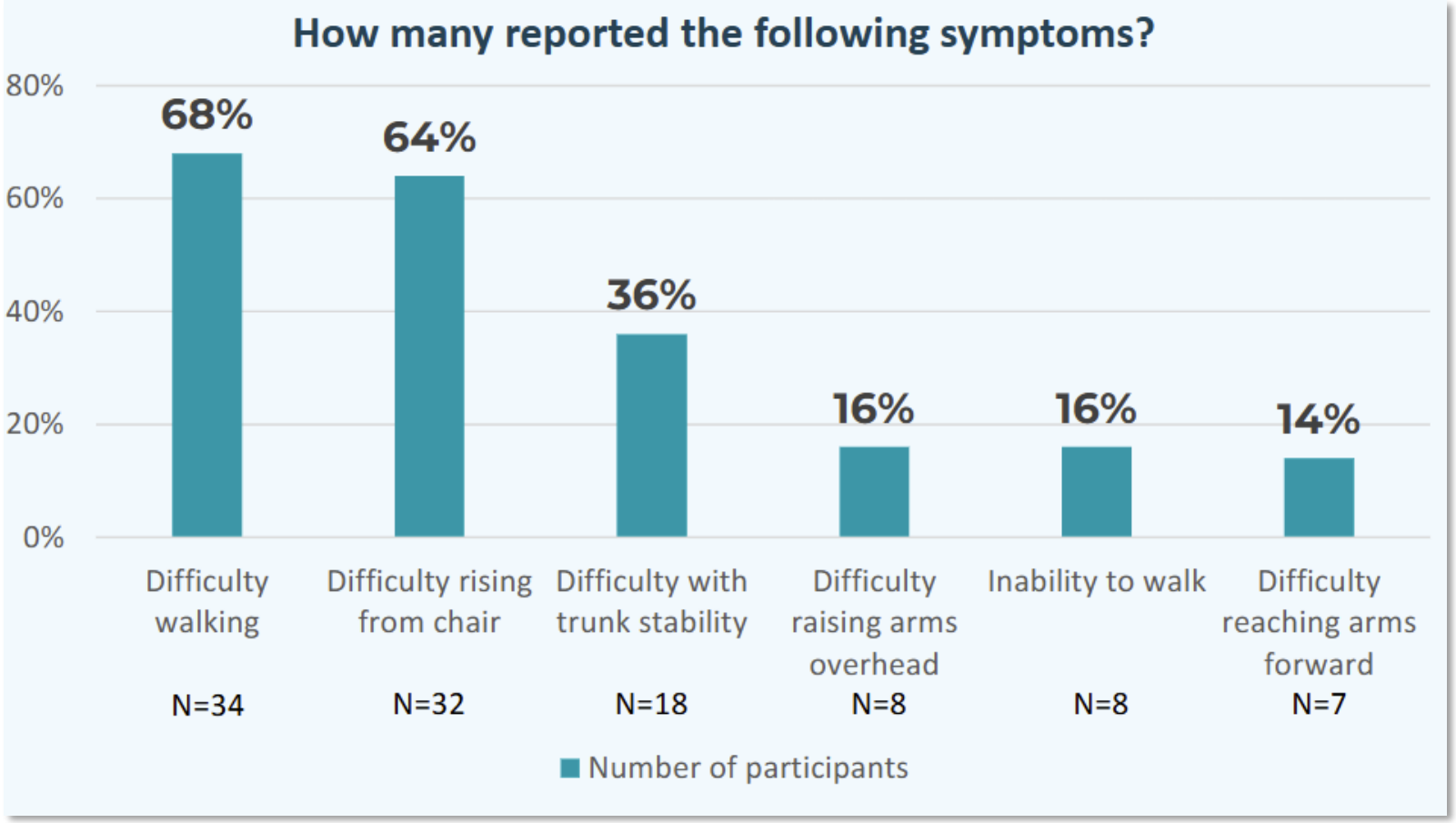
Nature of first concern	Number of patients (*N*)	Mean diagnostic delay (years ± SD)	Median diagnostic delay (years)	Number (%) of patients*
Incidental high CK finding	6	1.2 ± 1.8	0.7	11.3
Muscle signs/symptoms	31	2.9 ± 2.9	1.7	58.5
Cardiac signs/symptoms	5	3.3 ± 4.4	1.4	9.4
Neuropsychiatric issues/diagnoses	11	2.7 ± 3.2	0.8	20.8

*Percentage was calculated based on the total number of patients with first concern after January 2000, excluding family history, incidental molecular diagnosis and one patient whose genetic report was not available (53/134)

Becker, Becker muscular dystrophy; CK, creatine kinase; SD, standard deviation
Riguzzi P, et al. J Neurol. 2025;272(7):488.

Skeletal Muscle Weakness, Muscle Cramps, and Pain are a Key Presenting Symptom in Becker Muscular Dystrophy.[1]

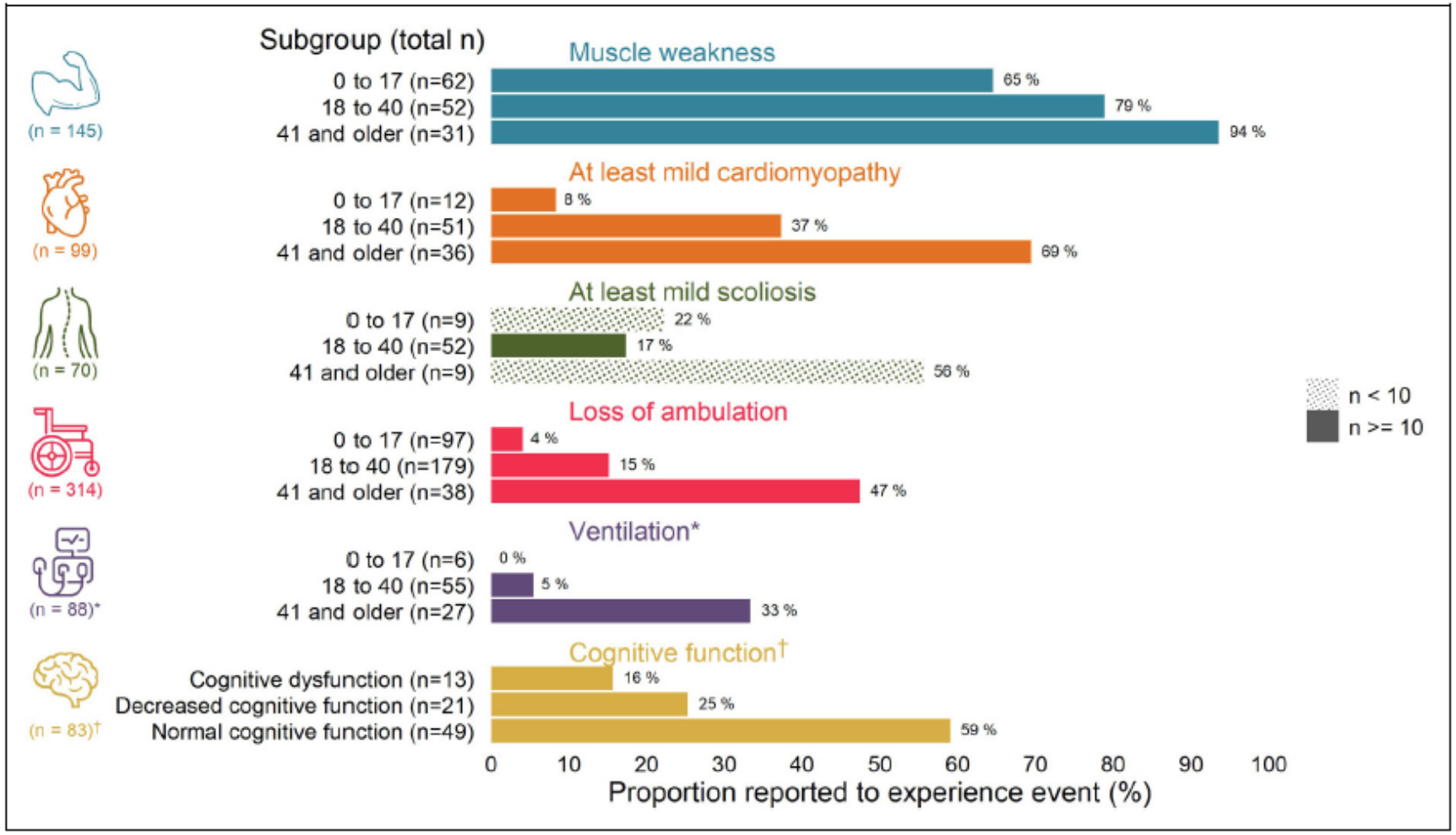
- Additionally **22%** reported that the pain they experience **never fully goes away**[2]
- **42%** noted that when it occurs, the **pain will last for several hours**[2]



1. Magot A et al. J Neurol. 2023;270(10):4763-4781. 2. Bronson A, et al. Poster presented at WMS; 2024 Oct 8-12; Prague, Czechia. #708LBP.

Literature Review Found that Muscle Weakness and Cardiac Involvement were the Most Frequently Observed Manifestations

Proportion of Patients with Becker Experiencing Outcomes by Age Group



Becker, Becker muscular dystrophy
Mickle AT, et al. J Neuromuscul Dis. 2026:22143602261420045. Online ahead of print.

Cardiac Involvement is the Main Cause of Deaths of Individuals Living with Becker Muscular Dystrophy

- **No correlation** between skeletal muscle involvement and the severity or time of onset of cardiac involvement[2,3]
- Can have **minimal skeletal muscle weakness but advanced cardiac disease**[1,2]
- If left untreated, may develop **heart failure**, which is the **main cause of death**[1]

Cardiac Involvement in a Natural History Cohort [4]

Cardiac Function Data	N	mean SD (min-max)
Cardiac involvement	80/153	-
Age at detection of cardiac involvement	77/80	33.1 ± 15.7 (11.1-75.3)
Age at last cardiac follow-up	149/163	32.9 ± 20.1 (1-84.9)
Pacemaker	8	-
Left Ventricular Assist Device Placement	4	-
Undergone Cardiac Transplant	7	-

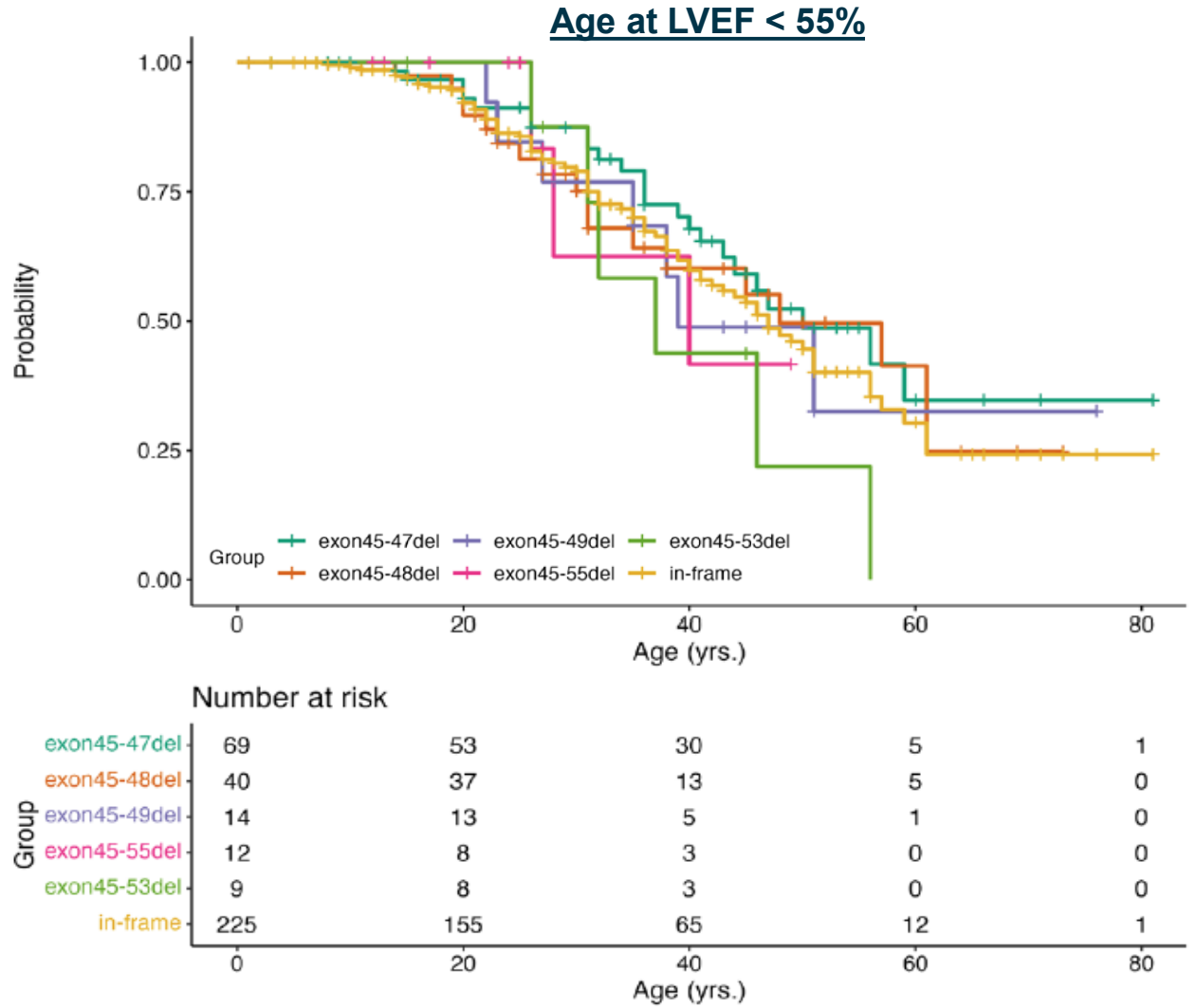
"My heart health is what actually threatens my health."
– patient survey response[5]

Becker, Becker muscular dystrophy; SD, standard deviation

1. Magot A, et al. J Neurol. 2023;270(10):4763-4781. 2. Ho R, et al. World J Cardiol. 2016;8(6):356-61. 3. Rajdev A, et al. Card Electrophysiol Clin. 2016;7(2):303-8. 4. Riguzzi P, et al. J Neurol. 2025;272(7):488. 5. Bronson A, et al. Poster presented at WMS; 2024 Oct 8-12; Prague, Czechia. #708LBP

No Clear Difference in Cardiac Deterioration Between Becker Mutation Groups

- N=225 Becker patients
- 22 Japanese centers
- Mean Age: 31.5 years old (range, 1-81)
- Heart disease **fourth common initial presentation**
- **No clear difference in age in development of LVEF <55% between deletion groups**

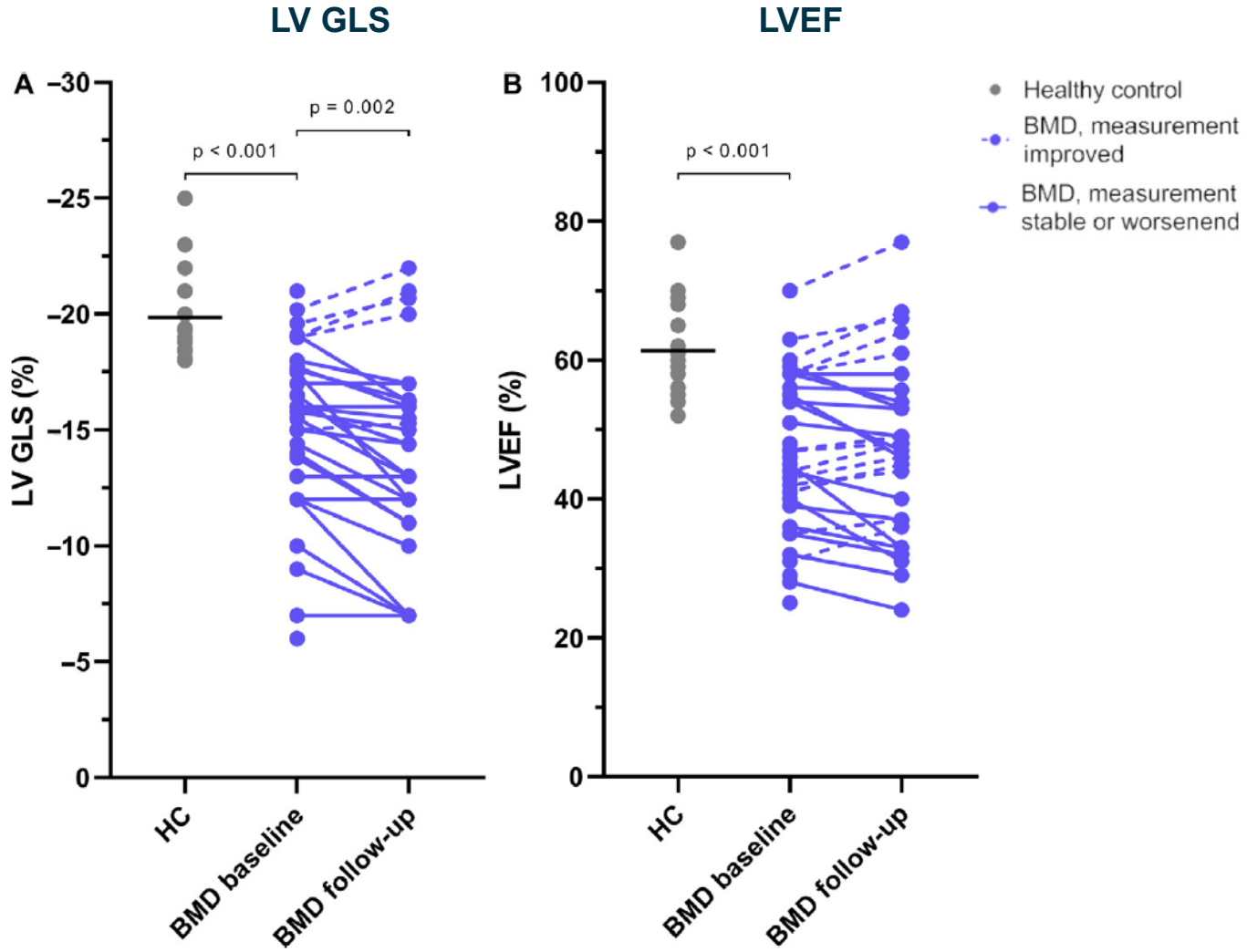


Becker, Becker muscular dystrophy; LVEF, left ventricular ejection fraction

1. Nakamura A, et al. Ann Clin Transl Neurol. 2023;10(12):2360-237

Cardiac Monitoring to Detect Early Myocardial Dysfunction is an Important Aspect of Becker Care and Management

- Becker (n=40; 29 at follow up) vs. controls (n=21)
- Mean follow-up: 2.0 ± 0.5 years
- LV GLS significantly worsened
    - **Mean deterioration: -1.3 ± 0.8%, p=0.002**
- LVEF remained **unchanged** (47.7 ± 10.8% to 47.0 ± 12.8%, p=0.455)
- **LV GLS more sensitive** to show progression of LV dysfunction over relatively short period vs. conventional echocardiographic measures (ie. LVEF)



Becker, Becker muscular dystrophy; LV, left ventricular; GLS, Global Longitudinal Strain; LVEF, left ventricular ejection fraction; HC, healthy controls

1. Van de Velde NM, et al. Am J Cardiol. 2022;162:170-176.

Becker patients can experience other symptoms, including those related to respiratory, bone, neuropsychiatric, gastric, and urinary systems[1]

Neuropsychiatric Issues in a Natural History Cohort[2]

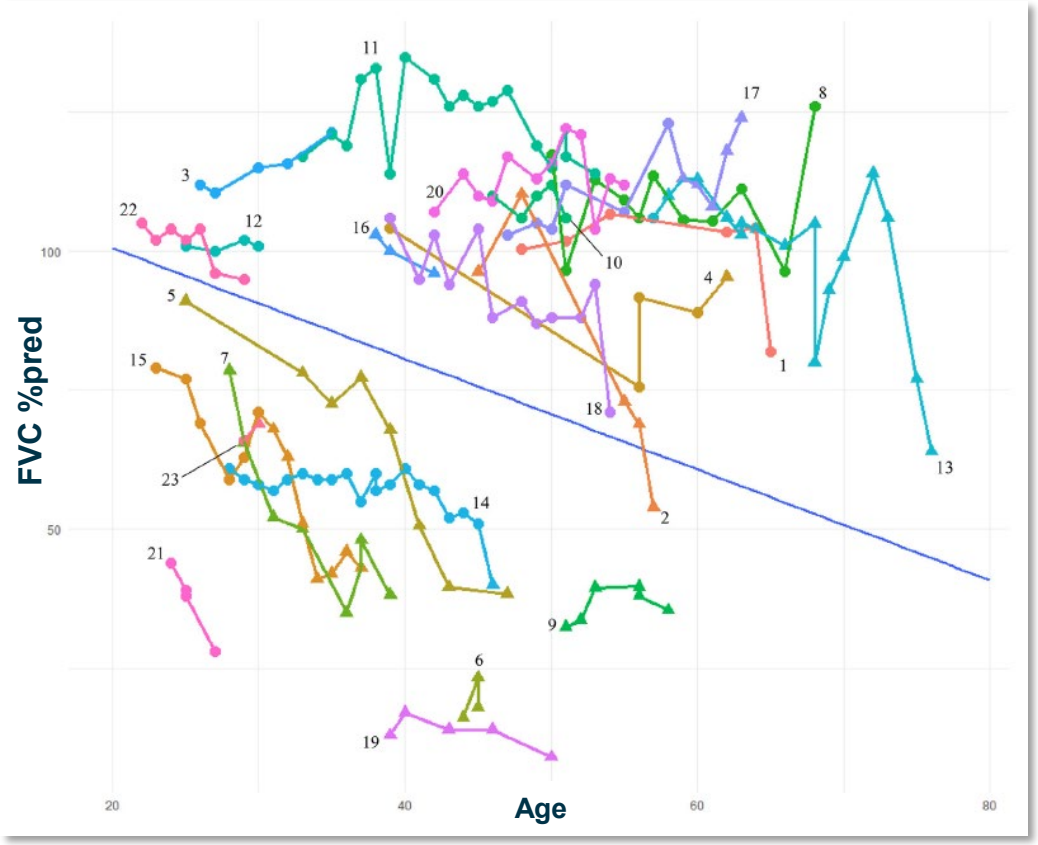
Table 4 Distribution of neuropsychiatric issues/diagnoses and psychiatric disorders within our cohort stratified by patients born before and after 01/01/1990

	Born before 01/01/1990	Born after 01/01/1990
Neuropsychiatric issues/diagnoses		
ADHD	1/86	6/77
Autism	1/86	16/77
Intellectual disability/specific learning disorders	2/86	4/77
No formal diagnosis	15/86	27/77
Psychiatric disorders*	39/86	18/77

ADHD attention deficit hyperactivity disorder

*Psychiatric disorders encompassed both diagnosed conditions and reported symptoms. Some individuals categorized under "psychiatric disorders" may have received a formal neuropsychiatric diagnosis or experienced neuropsychiatric issues during infancy

FVC % Predicted Decline Over Time in Adult Becker Patients[3]



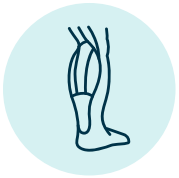
Becker, Becker muscular dystrophy; FVC, forced vital capacity

1. Magot A, et al. J Neurol. 2023;270(10):4763-4781. 2. Riguzzi P, et al. J Neurol. 2025;272(7):488 3. De Wel B, et al. Neuromuscul Disord. 2021; 31 (3):174-182.

Multidisciplinary Care and Proactive Management of Becker is Crucial for Patient Outcomes

Multidisciplinary Care

Opportunity for Improvement
The implementation of multidisciplinary care plan should be early in the course of MD in order to achieve the best outcome in quality of life. To carry out such plan requires care coordination. Care coordination of all modalities of care (irrespective of whether the



Neurologist: the lead clinician, taking overall responsibility for care of the Becker patient[1]

Other Specialties: cardiologist, physical therapist, pulmonologist, etc.

Proactive Management

Neuromuscular Monitoring
Assess for a Becker diagnosis and monitor for disease progression[1]

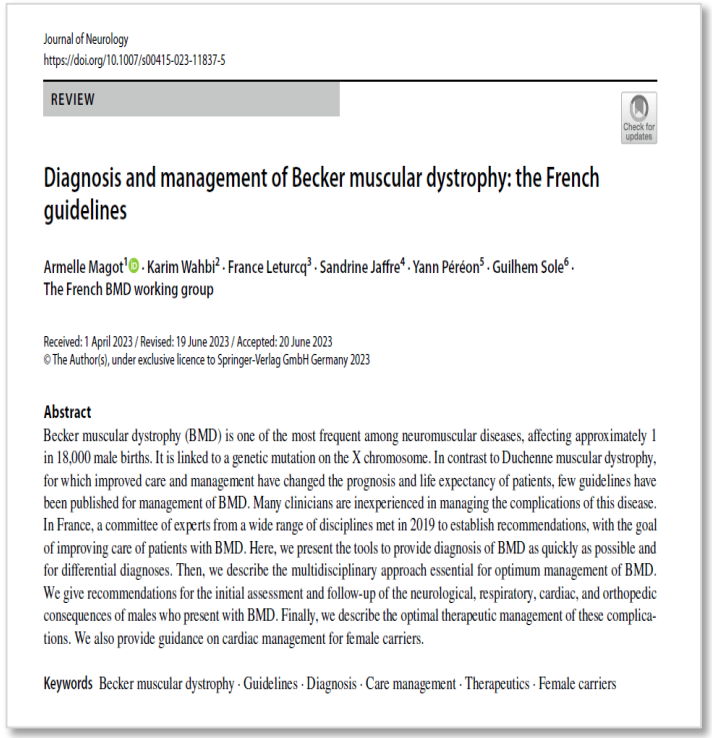
Cardiac Screening & Treatment
Routine monitoring by cardiologists and if needed, treatment[1,2,3]

Physical/Occupational Therapy
Referral to a physical or occupational therapist who is knowledgeable about muscular dystrophy[1,4]

1. Magot A, et al. J Neurol. 2023;270(10):4763-4781. 2. Riguzzi P, et al. J Neurol. 2025;272(7):488 3. American Academy of Neurology Muscular Dystrophy Quality Measure Set. 2014. 4. TreatNMD-Parent Project Italy. Diagnosis and Management of Becker Muscular Dystrophy Guide. 2021.

Some Guidelines Highlight Multidisciplinary Care and Proactive Management for Becker Muscular Dystrophy

2023 Becker Diagnosis and Management Guidelines
(the French BMD Working Group)



Journal of Neurology
https://doi.org/10.1007/s00415-023-11837-5

REVIEW

Diagnosis and management of Becker muscular dystrophy: the French guidelines

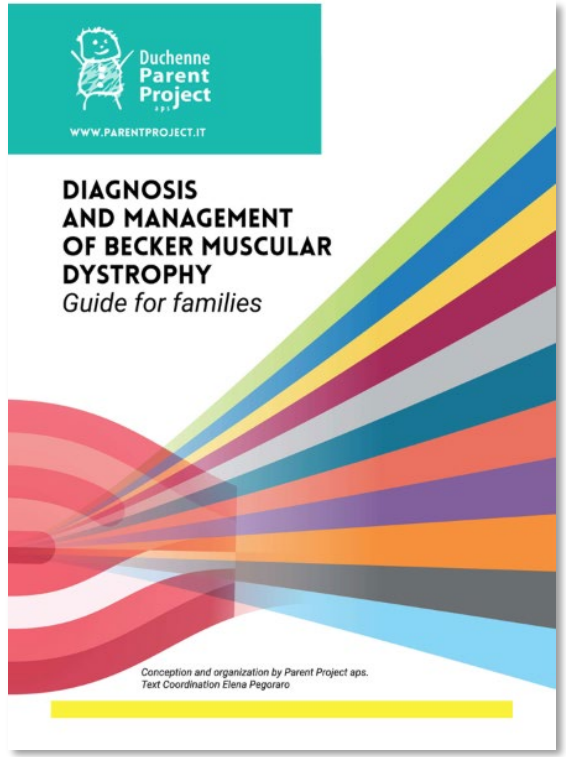
Armelle Magot[1] · Karim Wahbi[2] · France Leturcq[3] · Sandrine Jaffre[4] · Yann Péréon[5] · Guilhem Sole[6] · The French BMD working group

Received: 1 April 2023 / Revised: 19 June 2023 / Accepted: 20 June 2023

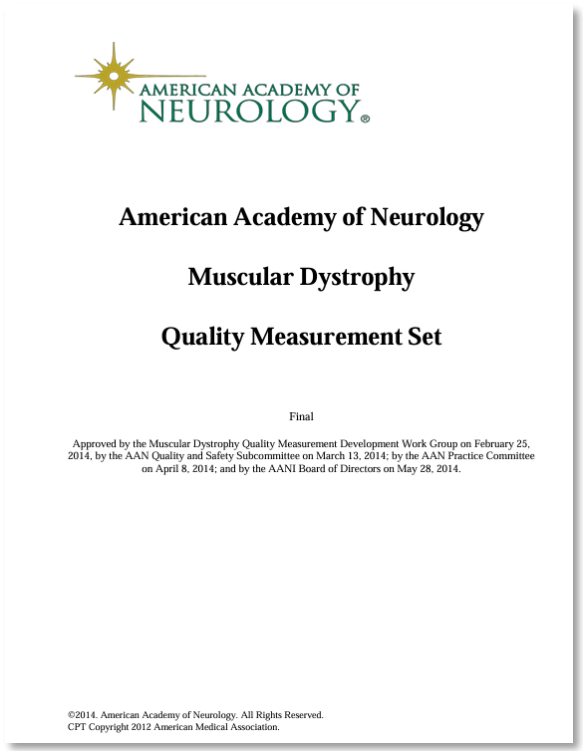
Abstract
Becker muscular dystrophy (BMD) is one of the most frequent among neuromuscular diseases, affecting approximately 1 in 18,000 male births. It is linked to a genetic mutation on the X chromosome. In contrast to Duchenne muscular dystrophy, for which improved care and management have changed the prognosis and life expectancy of patients, few guidelines have been published for management of BMD. Many clinicians are inexperienced in managing the complications of this disease. In France, a committee of experts from a wide range of disciplines met in 2019 to establish recommendations, with the goal of improving care of patients with BMD. Here, we present the tools to provide diagnosis of BMD as quickly as possible and for differential diagnoses. Then, we describe the multidisciplinary approach essential for optimum management of BMD. We give recommendations for the initial assessment and follow-up of the neurological, respiratory, cardiac, and orthopedic consequences of males who present with BMD. Finally, we describe the optimal therapeutic management of these complications. We also provide guidance on cardiac management for female carriers.

Keywords Becker muscular dystrophy · Guidelines · Diagnosis · Care management · Therapeutics · Female carriers

2021 Becker Guide for Italian Speaking Patients & Families
(TreatNMD and Parent Project Italy)



2014 AAN Muscular Dystrophy Quality Measurement Set
(American Academy of Neurology)



AMERICAN ACADEMY OF NEUROLOGY

American Academy of Neurology

Muscular Dystrophy

Quality Measurement Set

Final

Approved by the Muscular Dystrophy Quality Measurement Development Work Group on February 25, 2014, by the AAN Quality and Safety Subcommittee on March 13, 2014; by the AAN Practice Committee on April 8, 2014; and by the AANI Board of Directors on May 28, 2014.

Neuromuscular Assessments and Monitoring Can Help Identify Signs of Disease Progression

Monitoring and consistent follow-up assessments are recommended in guidelines to identify signs of progression.[1]

- The initial clinical exam:
  - Gait disorders
  - Weakness of the muscles
  - Reductions in muscle extensibility or joint contractures
  - Pseudohypertrophy of the muscles

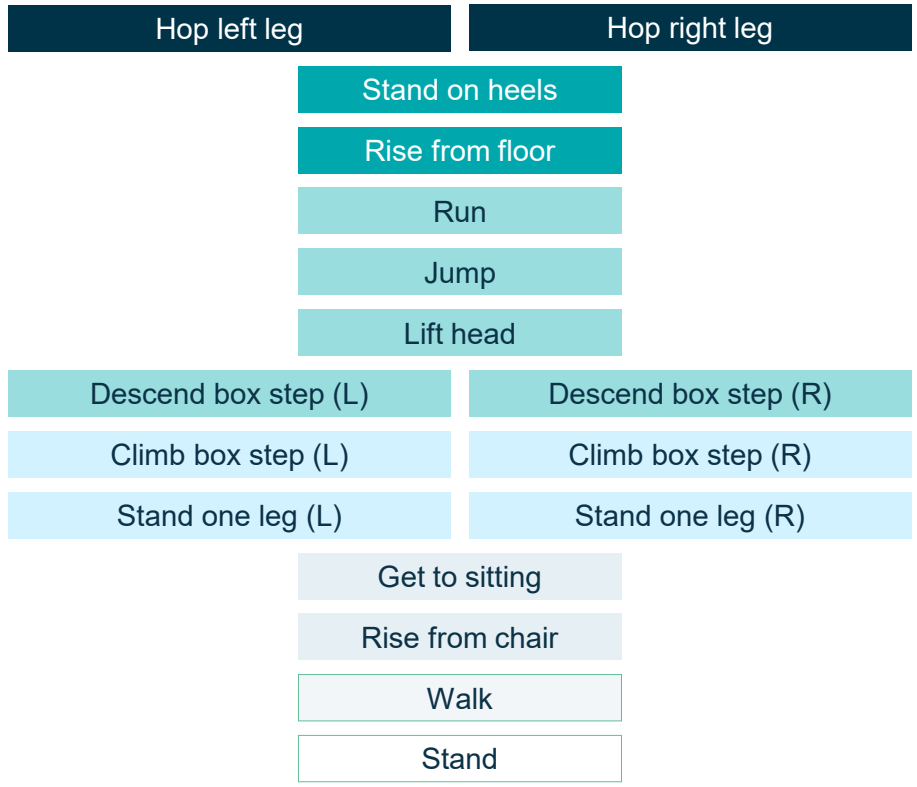
- Specialized functional assessments:
  - the 6-minute walk test
  - timed function tests
  - North Star Ambulatory Assessment

Learn more with the MDA on-demand webinar titled:

The North Star Ambulatory Assessment (NSAA) and its role in measuring impact in the Becker GRAND CANYON study

Speakers: Tina Duong, MPT, PhD and Joanne Donovan, MD, PhD

The North Star Ambulatory Assessment

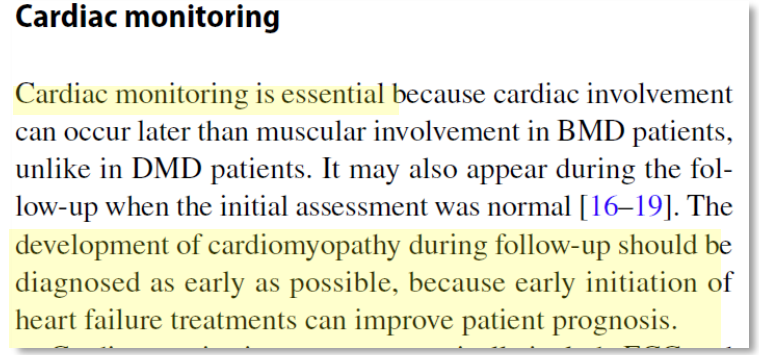


1. Magot A, et al. J Neurol. 2023;270(10):4763-4781

Cardiac Care and Monitoring Should Start After Diagnosis for Individuals with Becker Muscular Dystrophy

A cardiac assessment should occur right after diagnosis, regardless of symptoms, to detect subtle changes in myocardial tissue composition or left ventricular dysfunction.[1]

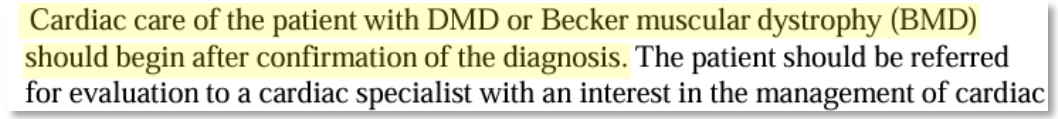
- Timely therapy can slow the rate of progression of cardiac dysfunction and, therefore, reduce the likelihood of heart failure and sudden death in the longer term[1]
- Cardiac monitoring should include echocardiogram[2]
- At a minimum, an echocardiogram every two years is recommended[2]



Cardiac monitoring

Cardiac monitoring is essential because cardiac involvement can occur later than muscular involvement in BMD patients, unlike in DMD patients. It may also appear during the follow-up when the initial assessment was normal [16–19]. The development of cardiomyopathy during follow-up should be diagnosed as early as possible, because early initiation of heart failure treatments can improve patient prognosis.

from the 2023 French BMD Working Group Guidelines



Cardiac care of the patient with DMD or Becker muscular dystrophy (BMD) should begin after confirmation of the diagnosis. The patient should be referred for evaluation to a cardiac specialist with an interest in the management of cardiac

from the 2014 AAN Measurement Set

1. Riguzzi P, et al. J Neurol. 2025;272(7):488 2. Magot A, et al. J Neurol. 2023;270(10):4763-4781 3. American Academy of Neurology Muscular Dystrophy Quality Measure Set. 2014.

Physical and Occupational Therapy Plays an Important Role in the Care of Patients with Becker Muscular Dystrophy

The aim of exercises performed during physiotherapy sessions is to optimize, maintain, or improve the patient's functional capacities.[1]

- Should be started as early as possible.[2]
- Twice a week for a period of 30-45 minutes seems to be the most beneficial.[1]
- Monitored by a professional **experienced with neuromuscular diseases**[3]

Learn more with the MDA conference Allied Health session:

Managing Diet & Exercise: In Clinic and Beyond - Exercise

Speaker: Tanja Taivassalo, PhD

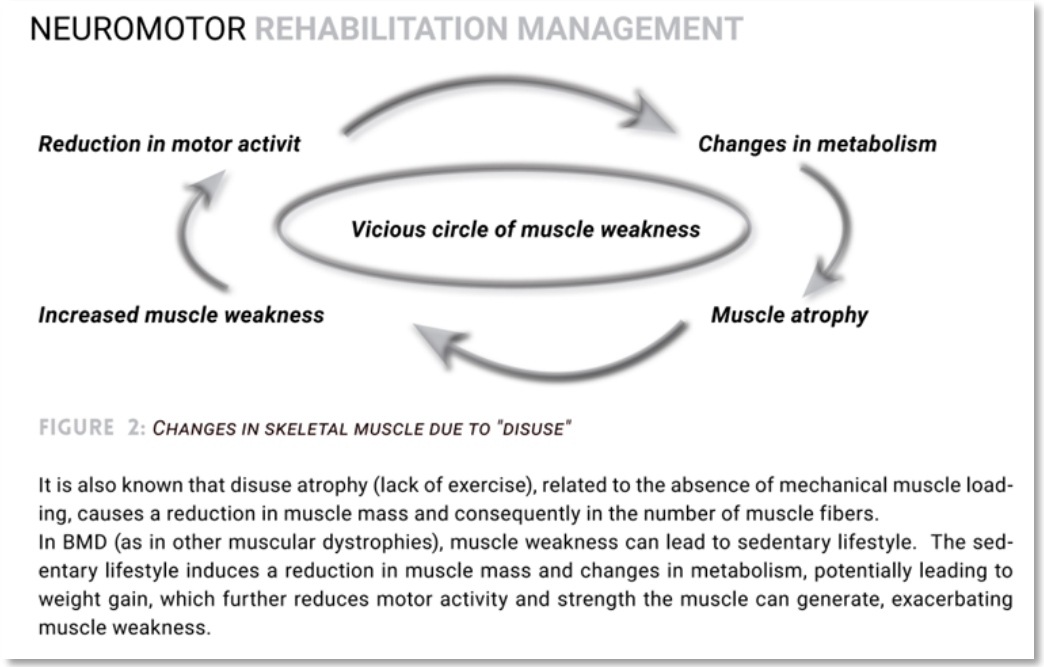
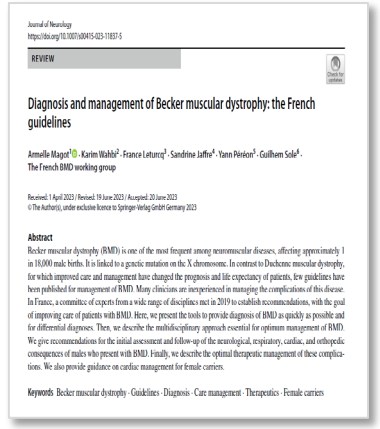


Figure from the 2021 Becker Muscular Dystrophy Guide by TreatNMD and Parent Project Italy

1. Magot A, et al. J Neurol. 2023;270(10):4763-4781 2. American Academy of Neurology Muscular Dystrophy Quality Measure Set. 2014 3. TreatNMD-Parent Project Italy. Diagnosis and Management of Becker Muscular Dystrophy Guide. 2021.

There is a Need for Becker-Specific Guideline Development in the United States to Improve and Standardize Care and Management

- With new data from many recent publications, updated guidance is needed that takes into account the latest findings
- Current guidance is based on other countries and are not specific to the United States
- Lack of guidelines specific to Becker muscular dystrophy
  - Current guidance, such as the AAN 2014 Measure set, incorporates recommendations for Duchenne muscular dystrophy and other neuromuscular disorders
- An unmet need for US guidelines dedicated solely to Becker muscular dystrophy remains evident


Journal of Neurology
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REVIEW
Diagnosis and management of Becker muscular dystrophy: the French guidelines
Armelle Magot[1] · Karim Wahbi[2] · France Leturcq[3] · Sandrine Jaffre[4] · Yann Pereon[5] · Guilhem Sole[6] · The French BMD working group
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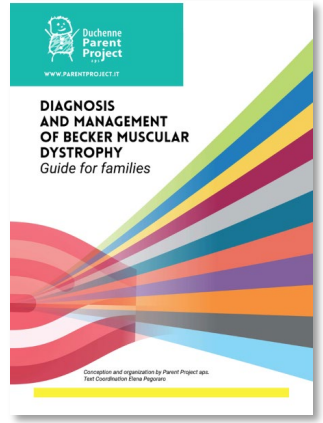
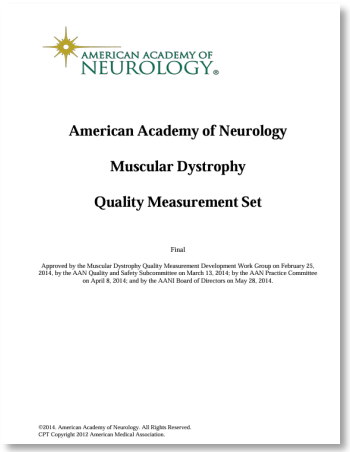
Abstract
Becker muscular dystrophy (BMD) is one of the most frequent among neuromuscular diseases, affecting approximately 1 in 18,000 male births. It is linked to a genetic mutation on the X chromosome. In contrast to Duchenne muscular dystrophy, for which improved care and management have changed the prognosis and life expectancy of patients, few guidelines have been published for management of BMD. Many clinicians are inexperienced in managing the complications of this disease. In France, a committee of experts from a wide range of disciplines met in 2019 to establish recommendations, with the goal of improving care of patients with BMD. Here, we present the tools to provide diagnosis of BMD as quickly as possible and for differential diagnoses. Then, we describe the multidisciplinary approach essential for optimum management of BMD. We give recommendations for the initial assessment and follow-up of the neurological, respiratory, cardiac, and orthopedic consequences of males who present with BMD. Finally, we describe the optimal therapeutic management of these complications. We also provide guidance on cardiac management for female carriers.
Keywords Becker muscular dystrophy · Guidelines · Diagnosis · Care management · Therapeutics · Female carriers

2023 Becker Diagnosis and Care Guidelines
(the French BMD Working Group)



2021 Becker Guide for Italian Speaking Patients & Families
(TreatNMD and Parent Project Italy)


AMERICAN ACADEMY OF NEUROLOGY
American Academy of Neurology
Muscular Dystrophy
Quality Measurement Set
Final
Approved by the Muscular Dystrophy Quality Measurement Development Work Group on February 25, 2014, by the AAN Quality and Safety Subcommittee on March 13, 2014; by the AAN Practice Committee on April 8, 2014; and by the AAN Board of Directors on May 28, 2014.
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2014 AAN Muscular Dystrophy Quality Measurement Set
(American Academy of Neurology)

AAN, American Academy of Neurology; Becker, Becker muscular dystrophy; US, United States

1. Magot A, et al. J Neurol. 2023;270(10):4763-4781 2. American Academy of Neurology Muscular Dystrophy Quality Measure Set. 2014 3. TreatNMD-Parent Project Italy. Diagnosis and Management of Becker Muscular Dystrophy Guide. 2021.

Key Takeaways

Multi-disciplinary and proactive care is important for the management of Becker muscular dystrophy.

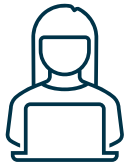
Although no pharmacological treatment is currently available for Becker, there are ways to proactive manage Becker.

Impactful proactive care options include neurology assessments, referrals to specialists, physical/occupational therapy support, routine cardiac monitoring, and if needed, cardiac treatment.

There is a need for guidelines dedicated to Becker muscular dystrophy in the United States to provide consistent care to patients.

Thank You for Attending!

We now welcome both virtual and in person questions from the audience.



For virtual viewers, please utilize the MDA platform to ask your questions.

For the in-person audience, please use the microphone to ask your question.

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Help us understand the current care and management of Becker by scanning and taking a quick survey!