BECKER MUSCULAR DYSTROPHY

FDA PATIENT **LISTENING SESSION**

JANUARY 14TH, 2026

Becker Muscular Dystrophy (Becker, BMD) FDA Patient-Led Listening Session

January 14, 2026

FDA Attendees

Office of the Commissioner (OC) – 3 offices

- OC/OEA/PES -- Office of External Affairs/Public Engagement (organizer)
- OC/OCMO/OOPD – Office of the Chief Medical Officer/Office of Orphan Products Development
- OC/OCMO/OPT – Office of the Chief Medical Officer/Office of Pediatric Therapeutics

Center for Biologics Evaluation and Research (CBER) – 3 offices

- CBER/OCD - Office of the Center Director
- CBER/OTP/OCE/DCEGM/GMB2 – Office of Therapeutic Products/Office of Clinical Evaluation/Division of Clinical Evaluation General Medicine/General Medicine Branch 2
- CBER/OTP/PSPS - Office of Therapeutic Products/Policy and Special Projects Staff

Center for Drug Evaluation and Research (CDER) – 7 offices

- CDER/OND/OCHEN/DCN – Office of New Drugs/Office of Cardiology, Hematology, Endocrinology and Nephrology/Division of Cardiology and Nephrology
- CDER/OND/ON – Office of New Drugs/Office of Neuroscience
- CDER/OND/ON/DNI – Office of New Drugs/Office of Neuroscience/Division of Neuroscience I
- CDER/OND/ON/DNII – Office of New Drugs/Office of Neuroscience/Division of Neuroscience II
- CDER/OND/ORDPRUM/DRDMG - Office of New Drugs/Office of Rare Diseases, Pediatrics, Urology and Reproductive Medicine/Division of Rare Diseases and Medical Genetics
- CDER/OPQ/OPQAI/DPQAII – Office of Pharmaceutical Quality/Office of Product Quality Assessment I/Division of Product Quality Assessment I
- CDER/OTS/OB/DBI – Office of Translational Science/Office of Biostatistics/Division of Biostatistics I

Center for Devices and Radiological Health (CDRH) – 9 offices

- CDRH/OPEQ/OHTI/DHTIB – Office of Product Evaluation and Quality/Office of Health Technology I/Division of Health Technology IB

- CDRH/OPEQ/OHTI/DHTIC – Office of Product Evaluation and Quality/Office of Health Technology I/Division of Health Technology IC
- CDRH/OPEQ/OHTIII - Office of Product Evaluation and Quality/Office of Health Technology III
- CDRH/OPEQ/OHTIII/DHTIIIB - Office of Product Evaluation and Quality/Office of Health Technology III/Division of Health Technology III
- CDRH/OPEQ/OHTIII/DHTIIIC - Office of Product Evaluation and Quality/Office of Health Technology III/Division of Health Technology IIIC
- CDRH/OPEQ/OHTV/DHTVA - Office of Product Evaluation and Quality/Office of Health Technology V/ Division of Health Technology VA
- CDRH/OPEQ/OHTV/DHTVB - Office of Product Evaluation and Quality/Office of Health Technology V/Division of Health Technology VB
- CDRH/OPEQ/OHTVI/DHTVIB - Office of Product Evaluation and Quality/Office of Health Technology VI/Division of Health Technology VIB
- CDRH/OSPTI/OEID/DPCD – Office of Strategic Partnership and Technology Innovation/Office of Equity and Innovative Development/Division of Patient Centered Development

Non-FDA Attendees

- National Institutes of Health (NIH)/National Center for Advancing Translational Sciences (NCATS)
- Reagan Udall Foundation for the FDA

Becker Muscular Dystrophy Community Testimonials

- Presenter 1: Mother of 2 young sons, ages 7 and 10 years, both of whom are ambulatory with significant cognitive symptoms that are associated with Becker. Main topics: Lack of therapies, uncertainty about disease progression, clinical trial opportunities for patients of all ages.
- Presenter 2: Father of 3 sons, ages 15, 18, and 30 years, all ambulatory with progressing mobility challenges. Main topics: Need for accurate and timely diagnosis, lack of therapies, lack of resources and support.
- Presenter 3: Male teen, age 15 years, ambulatory. Main topics: Impact on activities, clinical trial preferences, desire to preserve function
- Presenter 4: Male, age 26 years, ambulatory. Main topics: Cognitive impacts, progressive loss of mobility, preservation of independence
- Presenter 5: Male, age 36 years, ambulatory. Main topics: Muscle pain and fatigue, cardiac functional decline, lack of coordinated care.
- Presenter 6: Male, age 42 years, ambulatory with assistive device. Main topics: Loss of mobility, clinical trial participation, building a more informed patient community.
- Presenter 7: Male, age 49, ambulatory with assistive device. Main topics: Clinical trial experiences and preferences.

- Presenter 8: Male, age 58, non-ambulatory with late-stage cardiomyopathy and Ventricular Assist Device. Main topics: Muscle weakness, cardiac functional decline, lack of research opportunities

Listening Session Objectives

- Recognition of Becker muscular dystrophy as a serious, irreversible, and life-threatening disease with distinct challenges and numerous unmet needs
- Advancement of clinical and regulatory consideration of Becker muscular dystrophy, including patient preferences and objectives in research and therapy development
- Recognition of urgent needs for approved therapies and improved clinical care

Summary of Topics Discussed

Gaps in drug development and treatment options

- There are no FDA-approved therapies for Becker muscular dystrophy
- The absence of Becker-specific clinical trials underscores the urgent need for research and therapeutic development, including interventions that address mobility impairment and the causes of Becker-related cardiomyopathy.

Patient and Caregiver Experience

- People living with Becker have numerous and significant unmet needs.
- There is insufficient awareness and understanding of Becker among health care providers.
- Late diagnosis and misdiagnosis are common in Becker.
- Becker is characterized by muscle pain, fatigue, progressive and irreversible muscle weakness, cognitive and behavioral differences, and cardiomyopathy, resulting in variable but often substantial impacts on mobility, mental health, independence, and life expectancy.
- The clinical course in Becker is heterogeneous and unpredictable; however, once progression affects mobility, disease advancement becomes more pronounced.
- Coordinated, multi-disciplinary care – particularly for adults -- is frequently fragmented and difficult to navigate, requiring substantial time and effort to establish.

Patient-Centered Priorities and Preferences

- Priorities for treating Becker are slowing or halting disease progression, reducing muscle pain and fatigue, and improving cardiac health.
- Becker clinical trials should include participants of all ages and functional abilities.
- Decentralized and/or hybrid clinical trial designs should be considered in Becker to increase participation and adherence.

- Recent initiatives to build an integrated Becker community, encompassing patients, families, researchers, and healthcare providers, have increased awareness of and engagement with specialized care and research opportunities.

Key Takeaways

Meeting overview

The FDA Listening Session on Becker muscular dystrophy was held on January 14, 2026. The group of patients and family members participating in the Listening Session was not affiliated with any non-profit patient or advocacy group, although Parent Project Muscular Dystrophy was supportive of the initiative. The patients and families connected via social media and Becker Education and Engagement Days, annual events focused on Becker and organized by a consortium of advocacy, academic, medical, and industry stakeholders. During the Listening Session, nine presenters, including eight patients and family members and Dr. Katherine Mathews from the University of Iowa, an expert in Becker muscular dystrophy, provided testimony in a 90-minute virtual format, which included personal narratives, slide presentations, and videos.

Genetic Characterization and Clinical Background

Becker muscular dystrophy is a recessive X-linked dystrophinopathy, affecting predominately males, caused by pathogenic variants in the **DMD** gene, which encodes dystrophin, a key structural protein required for sarcolemmal stability in skeletal and cardiac muscle. The ***DMD*** gene is among the largest in the human genome, and mutations in the gene can result in one of two forms of muscular dystrophy: Duchenne muscular dystrophy or Becker muscular dystrophy. The phenotypic distinction between the two can be explained by the reading-frame rule. According to this principle, pathogenic variants that disrupt the translational reading frame (out-of-frame deletions or mutations) typically result in premature truncation of the dystrophin protein to the extent that little to no functional protein is expressed, leading to the Duchenne phenotype. In contrast, in-frame mutations preserve the reading frame, allowing translation of a shortened but partially functional dystrophin protein, classically associated with Becker muscular dystrophy.

This rule can help predict phenotype in many cases, but it is not absolute. The clinical consequences of an in-frame mutation depend not only on preservation of the reading frame, but also on the specific dystrophin domains affected, the stability and localization of the truncated protein, and the level of dystrophin expression achieved in muscle tissue. As a consequence, disease severity varies widely in Becker muscular dystrophy, ranging from minimally symptomatic individuals diagnosed incidentally in adulthood to individuals with early loss of ambulation, significant respiratory compromise, and severe cardiomyopathy. Importantly, cardiac involvement in Becker muscular dystrophy may be disproportionate to skeletal muscle weakness and may represent the dominant clinical manifestation.

This variability reflects the functional importance of the deleted regions of dystrophin. Deletions involving hinge regions may be well tolerated, whereas those affecting actin-binding domains or regions critical for interaction with the dystrophin-associated glycoprotein complex may result in a more severe phenotype despite preservation of the reading frame. Most phenotype-genotype research in Becker muscular dystrophy has focused on those mutations that result from exon-skipping transcripts in Duchenne. More research is needed to elicit a thorough understanding of all the pathogenic variants in Becker muscular dystrophy.

Characteristics of Duchenne and Becker Muscular Dystrophies At-A-Glance

FEATURE	DUCHENNE MUSCULAR DYSTROPHY	BECKER MUSCULAR DYSTROPHY
Gene	DMD	DMD
Inheritance	X-linked recessive	X-linked recessive
Typical Mutation Type	Out-of-frame deletions, duplications, nonsense mutations	In-frame deletions or duplications
Reading frame	Disrupted	Preserved
Dystrophin Production	Absent or nearly absent	Reduced, truncated, partially functional
Age at symptom onset	Early childhood (typically < 5 years)	Childhood to adulthood; highly variable
Disease Severity	Severe and progressive	Broad spectrum from mild to severe
Ambulation	Loss typically in early adolescence	Variable; may persist into adulthood or be lost earlier
Cardiac involvement	Common and progressive	Common; may be severe even with mild skeletal muscle disease
Diagnostic classification	Defined by genetic mechanism	Defined by genetic mechanism, not clinical course
Common misperception	Always severe	Always mild (incorrect)

Comparison Table: Table comparing characteristics of Becker and Duchenne Muscular Dystrophy. Both are X-linked recessive disorders caused by DMD gene mutations. Becker features in-frame deletions with preserved reading frame and reduced but partially functional dystrophin, while Duchenne has out-of-frame mutations with disrupted reading frame and absent dystrophin. Becker shows variable symptom onset from childhood to adulthood with broad disease severity spectrum, while Duchenne presents in early childhood with severe progressive disease. Both conditions involve cardiac complications.

Disease Burden

- ### Loss of Mobility
  - **Presenters 6 and 7** (42 and 49 years old) provided videos during their testimonies that showed challenges with navigating steps, rising from a seated position, getting into and out of automobiles, dressing, and walking**.**

- **Presenter 1** provided videos showing how the disease affects her 7 and 10-year-old sons' ability to walk, run, climb, and dance.
- **Presenter 3** said two of his three diagnosed sons, ages 18 and 30, were beginning to experience significant mobility challenges, with one requiring a mobility device for distances and one who changed jobs due to increased mobility loss.
- **Presenter 4** said his mobility challenges are unpredictable and vary from slight to moderate. He said maintaining mobility is critical to maintain his job and living accommodations.
- **Presenter 7** added that even slight changes in the weather prohibit him from leaving his home because wind, snow, and ice make his gait unsteady and unsafe.
- **Presenter 6** said falls and various issues resulting from falls, including requiring assistance to get up, as well as the potential for fractures and serious injury, were burdens associated with his progressive loss of mobility. He recently underwent intensive rehabilitation following a fall that resulted in a hip fracture. He achieved his goal of remaining ambulatory following the fracture, but said the experience exacerbated his fears and anxiety around progressive loss of mobility.

Cardiomyopathy

- **Dr. Mathews** presented data about cardiac functional decline in Becker muscular dystrophy, which is characterized by degeneration and fibrosis of the heart muscle leading to dilated cardiomyopathy, heart failure, and in some instances, sudden cardiac death. She cited one study[1] of 225 patients across all ages and severity of skeletal muscle involvement that found over 30% had abnormal Left Ventricular Ejection Fraction (LVEF), a key measure of heart function, and of those, 40% were under 30 years old.
- **Presenter 5** said he recently learned that his LVEF had decreased by 10 percent since his last check-up, and he is now in "acute systolic heart failure." He said that hearing the words heart failure at age 36 hit hard.
- **Presenter 8** (age 58) underscored the importance of educating health care providers about cardiac aspects of the disease. He said that he was in his mid-30s and had been diagnosed for over a decade when he learned through his own research that the disease could have serious cardiac implications. By the time appropriate testing was done, he already had significant cardiac functional decline, as measured by an LVEF in the low 30s. He has since endured what he characterized as many difficult physical and

> "While it is hard to say which Becker symptoms affect me the most, it is my cardiac status that most acutely threatens my health and life."
> *-- Presenter 8*

[1] **Natural history of Becker muscular dystrophy: a multicenter study of 225 patients**
Akinori Nakamura[1], Tsuyoshi Matsumura[2], Katsuhisa Ogata[3], Madoka Mori-Yoshimura[4], Eri Takeshita[5], Koichi Kimura[6], Takahiro Kawashima[7], Yui Tomo[8], Hajime Arahata[9], Daigo Miyazaki[10], Yasuhiro Takeshima[11], Toshiaki Takahashi[12], Keiko Ishigaki[13], Satoshi Kuru[14], Akiko Wakisaka[15], Hiroyuki Awano[16], Michinori Funato[17], Tatsuharu Sato[18], Yoshiaki Saito[19], Hiroto Takada[20], Kazuma Sugie[21], Michio Kobayashi[22], Shiro Ozasa[23], Tatsuya Fujii[24], Yoshihiro Maegaki[25], Hideki Oi[8], Hisateru Tachimori[7,26], Hirofumi Komaki[5]

emotional realities due to his cardiac health, including serious tachycardia events, two complex ablation procedures, extended time on a ventilator, and the need for an Implantable Cardioverter Defibrillator (ICD). In 2019, his progressing heart failure resulted in persistent shortness of breath, bloating, and extreme fatigue, and he now lives with an external Ventricular Assist Device (VAD). He has been deemed ineligible for a heart transplant. Presenter 8 said, "While it is hard to say which Becker symptoms affect me the most, it is my cardiac status that most acutely threatens my health and life."

- **Muscle Pain and Fatigue**
  - Muscle pain and fatigue were described, with **Presenters 5, 6, and 8** saying their mobility in adolescence was normal, but they could not keep up with their peers in sports and experienced frequent to constant muscle pain and fatigue.
  - **Presenter 5** (age 36) said he lives with daily back and leg pain, significant fatigue, inflammation, muscle cramps, and tightening of his heel cords to the extent that his gait and ability to stand are affected. He said, "At this point in my life, I've come to accept that I live every day in constant pain. I dream of a day when I could wake up without pain."

> "At this point in my life, I've come to accept that I live every day in constant pain. I dream of a day when I could wake up without pain."
>
> *-- Presenter 5*

  - Knee pain and instability were cited by **Presenters 4** (age 26) and **7** (age 49), who described pain and fatigue associated with leg stability, saying they cannot predict when their knees are going to "give out" and result in falls while walking or navigating stairs or curbs.
- **Cognitive Impacts**
  - **Dr. Mathews** discussed common neurocognitive disorders that can be associated with Becker muscular dystrophy, including learning disabilities and psychiatric problems such as obsessive-compulsive disorder, anxiety, and autism-like behaviors. She said that for some individuals and families, this aspect of the disease can be extremely problematic**.**
  - **Presenter 1** said she worried that her boys' cognitive issues could mean they would be excluded from participation in clinical trials.
  - **Presenter 4** described how cognitive differences affect him as an adult, including problems with focus, organization, and impulse control.
- **Barriers to optimal clinical care**
  - **Dr. Mathews** said a multidisciplinary clinical care approach is optimal for disease management, but that this model is often unattainable, particularly for adult patients. The transition from pediatric to adult care is a common difficulty for patients with muscular dystrophy.

- **Presenter 3** said he did not have local care providers with sufficient knowledge about Becker muscular dystrophy, and traveling back and forth for appointments takes him away from school and typical teen activities.
- **Presenter 4** said he was not able to access consistent and comprehensive care due to time, travel, and expense limitations. He said he was specifically concerned about not having access to regular cardiac monitoring.
- **Presenter 5** said that he had good clinical care as a pediatric patient, but transitioning to adult care was like "losing a safety net." He said his most recent annual checkup spanned three months, required visits to multiple clinicians at multiple locations, and required a loss of 10 work hours, excluding travel time.
- **Presenter 6** said he regretted not pursuing consistent and specialized care when he was younger because he did not realize how severe the disease would become in his 30s. Now in his early 40s, he said he still finds the unpredictability of progression to be one of the hardest aspects of living with the disease. He said, "I had no idea how severe Becker muscular dystrophy would be until it hit like a ton of bricks in my mid-30s. I wish someone had told me what to anticipate."

"I had no idea how severe Becker Muscular Dystrophy would be until it hit like a ton of bricks in my mid-30s. I wish someone had told me what to anticipate."
-- Presenter 6

- **Presenter 8** said he had to be proactive in seeking out providers who had knowledge of Becker muscular dystrophy, specifically about the potential severity of disease-related cardiomyopathy. He said managing his cardiac issues now requires constant monitoring, frequent medication adjustments, excellent health insurance, and a "willingness to attend appointment after appointment."

Clinical and Research Implications of Late Diagnosis and Misdiagnosis

- **Dr. Mathews** included a discussion about the critical role of genetic testing in distinguishing Becker from other forms of muscular dystrophy, specifically Duchenne muscular dystrophy and the Limb-Girdle muscular dystrophies**.**
- **Presenter 1** (mother of 7 and 10-year-old sons) described how genetic testing for her sons' cognitive issues led to their diagnosis of dystrophinopathy, which subsequently led to her father's diagnosis of Becker muscular dystrophy. Several other affected members in this family have since been identified. She described her family history with Becker muscular dystrophy as one of "missed and dismissed symptoms, misdiagnoses, physical and mobility struggles, heart failure, and heartbreak."
- **Presenter 2** (the father of three recently diagnosed sons) provided a real-world example that reinforced the need for early and comprehensive genetic testing. His father-in-law's misdiagnosis of Limb-Girdle muscular dystrophy led to incorrect genetic testing for other members of the family, and years of misunderstandings about muscle weakness and pain experienced by his sons.

- **Presenters 2, 4, 7, and 8** attributed their diagnostic odyssey to a lack of knowledge about Becker muscular dystrophy among health care providers.
- **Presenter 8** said he may have missed out on early cardiac interventions because of a lack of knowledge about how the disease affects heart function.
- **Presenters 3 and 6** were correctly diagnosed as young children, one due to the diagnosis of an older brother, and one due to muscle weakness and confirmed with muscle biopsy and genetic testing.

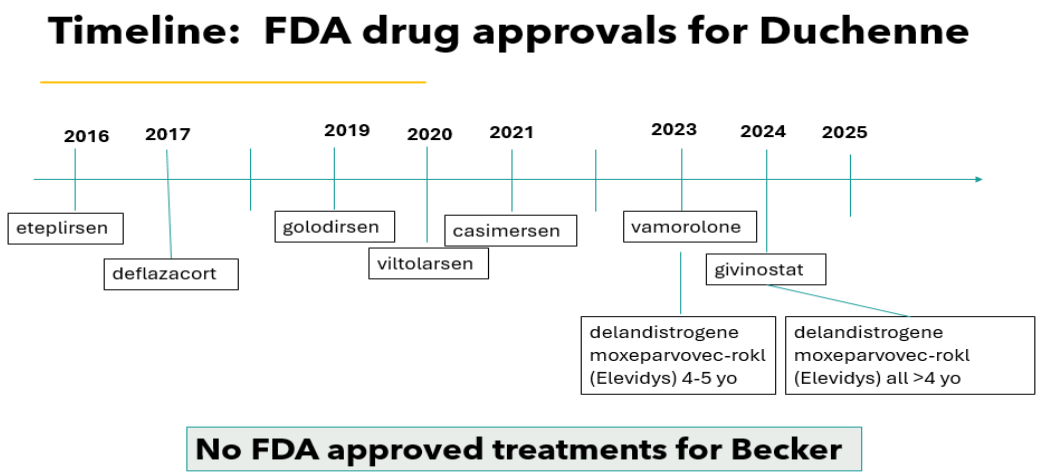
The testimonies underscored the significant need for early and accurate diagnosis. Despite advances in molecular diagnostics, Becker is still frequently misclassified based on clinical presentation rather than genetic mechanism. The age of onset, slower progression, or prolonged ambulation are often associated with to Becker muscular dystrophy, while earlier onset or rapid progression lead to a Duchenne diagnosis, but this approach is genetically incorrect.

Genetic classification should be based on the nature of the DMD variant and resulting dystrophin expression, not on age at diagnosis or functional milestones. Individuals with in-frame mutations may experience severe disease that clinically resembles Duchenne muscular dystrophy, while rare individuals with out-of-frame mutations may demonstrate milder phenotypes due to alternative splicing, exon skipping, or low-level dystrophin production.

Incorrect genetic classification also has significant implications for patient care and research, including access to mutation-specific therapies, eligibility for clinical trials, cardiac surveillance strategies, and interpretation of natural history data. Reliance on clinical severity alone risks excluding individuals with Becker muscular dystrophy from therapeutic opportunities and contributes to gaps in evidence for adults and more severely affected Becker muscular dystrophy populations. Accurate genetic characterization, coupled with quantitative assessment of dystrophin expression, when possible, is essential to ensure appropriate diagnosis, equitable access to emerging therapies, and improved understanding of dystrophinopathies across the lifespan.

More Research and Clinical Trial Opportunities are Needed

Currently, there are no therapies approved for use in Becker in the United States, and patients and families have numerous unmet needs. Opportunities for participation in research and clinical trials are also very limited, both in number and scope. Presenters at the Listening Session described a variety of concerns and desires for more opportunities and greater innovation in participation. During her testimony, Dr. Mathews presented the following timeline of FDA approvals for Duchenne muscular dystrophy, but noted that nothing has been approved for patients living with Becker muscular dystrophy.



Timeline Graphic: Timeline of FDA drug approvals for Duchenne muscular dystrophy from 2016 to 2028, showing approvals for eteplirsen (2016), deflazacort (2017), golodirsen (2019), viltolarsen (2020), casimersen (2021), vamorolone (2023), and delandistrogene moxeparvovec-rokl/Elevidys for ages 4-5 (2023) and givonostat (2024), and delandistrogene moxeparvovec-rokl/Elevidys for all patients over 4 (2024). Note indicates no FDA approved treatments exist for Becker muscular dystrophy.

- **Presenter 1,** the mother of two boys who have been diagnosed with dystrophinopathy, said she has not pursued a definitive diagnosis because family history indicates that the boys have Becker muscular dystrophy, and that diagnosis will exclude them from clinical trials and therapies currently only available to patients with Duchenne muscular dystrophy. She said it has been frustrating and emotionally difficult to see many research opportunities for patients with Duchenne, but very few for Becker. “It’s like Becker is just forgotten as a less serious and less-worthy-of-treatment form of muscular dystrophy”.
- **Presenter 1** also expressed concerns about both lower and upper age limits for clinical trial participation, saying her sons have been excluded because they are too young and her father, who also has the disease, has been excluded because he is too old. In addition to missing out on the hope offered by clinical trials, she said she worries that participation exclusions could result in insurance coverage delays or denials once therapies are approved and become commercially available.
- **Presenter 3** said he would be interested in participating in clinical trials if they were local to him and did not require significant time away from high school and social activities.
- **Presenter 5** cited career demands and time-consuming medical appointments as reasons why he is unable to participate in clinical trials**.**

- **Presenters 4, 5, and 6** all discussed how decentralized and hybrid trial designs would be useful for this patient population**.**
- **Presenter 6** said more natural history studies are needed in Becker, and decentralized data collection would incentivize more patients to participate in such studies.
- **Presenter 7** had experience in several studies and trials that used outcome measures based on ambulation, but now he is concerned that his declining ability to walk would exclude him from future participation. He called for trial endpoints that reflect additional activities important to patients in their everyday lives. Presenter 7 said trials with cohorts of Duchenne and Becker patients could accelerate progress, as long as results were considered in the context of the separate cohorts. As an example, he discussed his experience in a gene therapy trial and said, "The trial had multiple cohorts, including one for Duchenne, and it was abandoned when it did not show results for Duchenne. I still wonder why no one saw the value in continuing with the Becker cohort."

> "The trial had multiple cohorts, including one for Duchenne, and it was abandoned when it did not show results for Duchenne. I still wonder why no one saw the value in continuing with the Becker cohort."
> *-- Presenter 7*

- **Presenter 8** said he would be interested in research participation if more progressed patients were eligible and was particularly interested in studies that address disease-specific cardiomyopathy.
- **Presenters 2, 5, and 7** called for more inclusion and consideration of Becker cohorts in certain clinical trials for Duchenne.
- **Presenter 5** said, "Too often, Becker patients are left out of research that could make a real difference in how long and how well we live."

Treatment Objectives and Preferences

Slowing or stopping disease progression was the primary goal of treatment for all of the patient and family presenters at the Listening Session.

- **Presenter 1** said she wanted her boys to have access to a treatment while they are still young because "time is muscle."

> "I would be very interested in taking a therapy for Becker if it slowed disease progression and did not have bad side effects."
> *-- Presenter 3*

- **Presenter 3** said, "I would be very interested in taking a therapy for Becker if it slowed disease progression and did not have bad side effects."
- **Presenter 4 (**age 26) said he considered slowing disease progression to be the most important attribute of a therapy, and added that

he would be motivated to seek out and organize better medical care if there were therapies available. He said, "If there were treatments to preserve function, pursuing coordinated care would feel purposeful instead of overwhelming."

- **Presenter 5** said his treatment objectives would be slowing disease progression and alleviating muscle pain and fatigue**.**
- **Presenter 7** associated slowing disease progression with preservation of ambulation, the ability to open doors and open packages, use the bathroom, and dress without assistance.
- **Presenter 8** said that with the challenges he now faces, slowing or stopping further progression could give him more years with his wife and four children, as well as the ability to participate in activities with them.

Looking Forward: Community Building Brings Hope

The Listening Session included a discussion about recent efforts to build a more connected and informed community of Becker muscular dystrophy patients, families, healthcare providers, researchers, and industry partners**.**

- **Presenter 6** described his motivation for becoming a founding member of Becker Education and Engagement Day, a now 4-year-old initiative that has been instrumental in bringing about much-needed disease awareness and support. He said developing community is critical for research, and FDA should be aware that there is now an active and organized community of Becker muscular dystrophy patients and families**.**
- **Presenter 8**, the father of three daughters who are genetic carriers, said in his concluding remarks that he wants Becker muscular dystrophy to be considered as a specific disorder with unique challenges and considerations. He said, "This has created new hope that better treatment and care options will be available very soon."

Open Discussion

Following the testimonies, FDA staff asked the group about the use of corticosteroids as a therapy option for Becker muscular dystrophy.

- **Presenter 1** said she has discussed the option with care providers, and there is agreement that corticosteroids could exacerbate behavioral issues for her boys, who have cognitive impacts from the disease.
- **Presenter 3** said he is currently taking a low dose of corticosteroids because they make his legs feel stronger and have helped him gain some desired weight. He said a higher dose resulted in puffy facial features and excessive weight gain that made him feel self-conscious around his friends.
- **Presenter 5** said he has not used corticosteroids, and his doctor has not recommended them due to the risks of side effects**.**

- **Presenter 7** said he has used corticosteroids at various times but is not currently taking them. He said his doctor does not think they would be beneficial given his age (49).
- **Dr. Mathews** said she sometimes prescribes corticosteroids for adult patients if they are concerned about an impending functional loss, but long-term corticosteroid treatment is a highly personal decision in Becker muscular dystrophy. She also said high-dose steroids can cause steroid myopathy, which would be detrimental to this population.

FDA staff also asked the group about what risks they would accept in order to take a new therapy.

- **Presenter 6** said he would want to know about potential risks to liver and cardiac health when considering a new therapy, but was willing to try just about anything. He said there are many in the community who were eager to accept the risks associated with participation in clinical trials.
- **Presenter 7** said he would accept most risks, including the possibility of death, for a promising treatment.
- **Presenter 8** talked about risks associated with interventions for late-stage heart failure, saying he has a love-hate relationship with them but is grateful for them overall. He said treatment risk is not as hard to live with as the uncertainties of further cardiac decline.

Conclusion

Becker muscular dystrophy is a distinct diagnosis, characterized genetically with variable and progressive loss of function, and carries a significant risk of cardiomyopathy. Becker muscular dystrophy currently has no therapies, and patients have significant unmet needs.

Disclaimer

Discussions in FDA Rare Disease Listening Sessions are informal. All opinions, recommendations, and proposals are unofficial and nonbinding on FDA and all other participants. This report reflects the patient organizers' account of the perspectives of patients and caregivers who participated in the Becker muscular dystrophy Patient-led Listening Session with the FDA. To the extent possible, the terms used in this summary to describe specific manifestations of Becker muscular dystrophy, health effects and impacts, and treatment experiences, reflect those of the participants. This report is not meant to be representative of the views and experiences of the entire Becker muscular dystrophy patient population or any specific group of individuals or entities. There may be experiences that are not mentioned in this report.

Financial Disclosures

None of the patients nor the physician received compensation for their participation in the Listening Session. Listening-only participants from Parent Project Muscular Dystrophy did not receive compensation for the Listening Session. Partner organization AdvocacyWorks

Consulting received financial support from industry for helping to identify and prepare patient and family participants.

All of the participants at the Listening Session thank the FDA for providing this platform for patients and families impacted by Becker muscular dystrophy.