It's Time to Get Real About Becker Muscular Dystrophy (Becker): Using Qualitative Research to Understand the Lived Experience and Give Voice to an Underserved Community

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1. Objective

The objective of this study was to develop a strategy to mobilize the Becker muscular dystrophy (Becker) community by capturing and communicating the practical and psycho-social experiences, needs, and challenges of individuals living with Becker and their caregivers across their lifespan.

2. Background

Becker is a serious, debilitating, and potentially life-threatening neuromuscular disorder with a high unmet medical need. The community is underserved as there are:

- No approved therapies for Becker
- Only two interventional trials are actively recruiting, demonstrating the limited research exclusively dedicated to this indication
- No Becker-specific advocacy organizations to promote research and provide patient -focused resources.

Increasingly, the FDA encourages the patient voice to be included in drug development, to help establish the therapeutic context for risk/benefit decision making, improve study design, and enhance trial enrollment and communications. (1,2) Understanding the patient voice is crucial to effective communications and community mobilization, particularly in a community that is underserved and disenfranchised.

3. Design

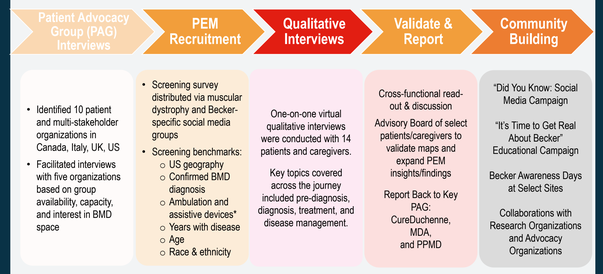
The design of the patient experience mapping (PEM) study consisted of five stages, with the final community building stage still ongoing (Figure 1). Demographics show that the 14 individuals who participated represented a wide range of age and ambulatory ability. (Table 1) Interviews focused on the journey from pre-diagnosis to diagnosis to treatment and management of Becker.

Table 1: *Demographics of interview participants.*

	Age range (yrs.)	Range of age at dx (yrs.)	Fully ambulatory	Requires mobility aid	FT Wheelchair
Pediatric n = 7	8-17	2-9	4	3	0
Adult n = 7	30-62	5-30	1	2	4

3. Design (Continued)

Figure 1: *Methodology.*



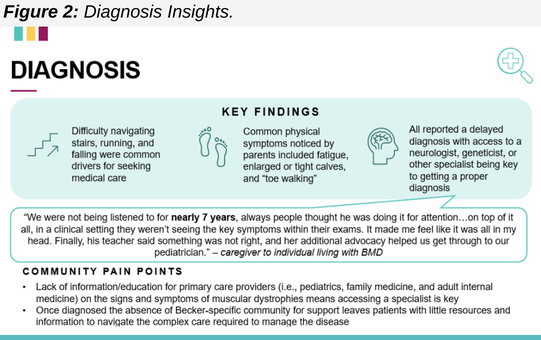
**participants were selected with the intent to represent a wide range of experiences and stages of disease*

4. Results

Results of the PEM study yielded distinct themes further characterizing the misunderstood and underserved nature of Becker. Diagnostic delays were noted (Figure 2) along with the repercussion of receiving a Becker versus Duchenne diagnosis (Figure 3). Along with mobility limitations,, all participants reported comorbidities with cardiac complications being most concerning. All noted long-term mental health impacts, partially driven by financial worries, that come with living with a disease that has an unknown progression. (Figure 4). While these challenges and experiences demonstrated the significant impact of living with Becker, resources and information about Becker are lacking in the community often leaving these individuals feeling unheard, unseen and under resourced.

Highlights of PEM Results

Figure 2: *Diagnosis Insights.*



4. Results (Continued)

Figure 3: *A Becker versus Duchenne diagnosis impacts provider communication and access.*

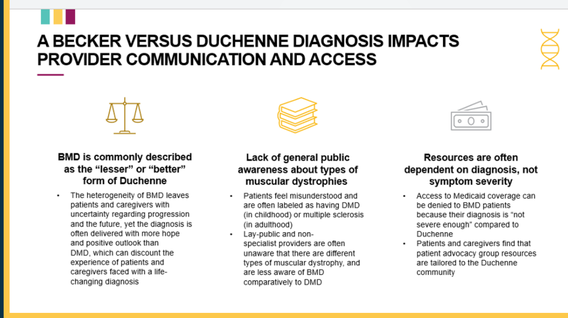
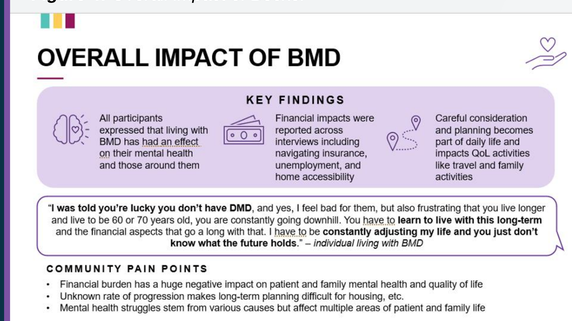


Figure 4: *Overall impact of Becker*



Awareness & Education Campaign

From these results, a multi-pronged, multi-stakeholder awarenes and education campaign was developed with three goals:

- Assure the Becker community that their unique needs are being heard
- Provide much needed information on Becker
- Encourage the community to come together to create change.

The campaign consisted of articles/flyers disseminated through various channels, (Figure 5) and a series of vignettes on social media focusing on impacts identified in the qualitative research (Figure 6).

The intent of the campaign is to assure the Becker community that their unique needs are being heard, provide much needed information on Becker and encourage the community to come together to create change.

4. Results (Continued)

Figure 5: *Sample Call-To-Action Education Flyer*

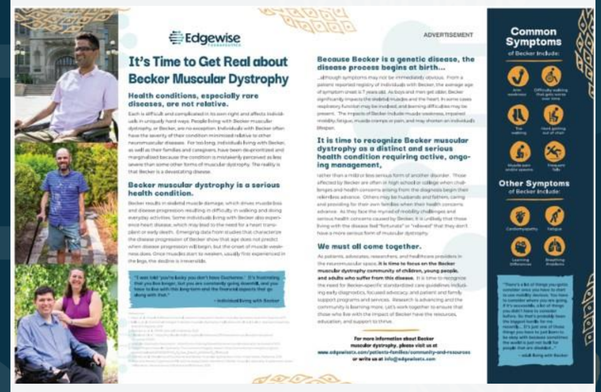


Figure 6: *Sample Vignettes from Social Media Campaign*

Community Engagement

Stemming from these initial campaign tactics, an in-person Becker Education and Engagement Day is in planning and will be held simultaneously across 4 sites in the US in early December. This event is a multi-stakeholder effort with collaboration by industry partners, advocacy, academic sites and KOLs. The goal of the Becker Education and Engagement Day is to provide disease education and treatment management, highlight ongoing scientific advances, and give individuals living with Becker an opportunity to come together in person and meet each other and their care providers, thus inspiring a hopeful community.

5. Discussion

These patient experience results show that qualitative data can be powerful and should not be considered anecdotal. When the patient voice and disease misperceptions are visually and emotively communicated, all stakeholders in an underserved community will engage in efforts to serve the community. The lived experiences of the Becker community must be communicated and amplified to create the change the community needs.

References

1. U.S. Food and Drug Administration: https://www.fda.gov/drugs/development-approval-process-drugs/fda-patient-focused-drug-development-guidance-series-enhancing-incorporation-patients-voice-medical. Accessed August 2023
2. Guide to Patient Involvement in Rare Disease Therapy Development; A Publication of the Rare Disease PFDD Compendium Workshop Series, The Everylife Foundation for Rare Diseases, 2022.

Presented at NORD Breakthrough Summit, Washington, DC, October 2023