Addressing the Need for Adult Care Models in Duchenne Muscular Dystrophy (Duchenne)

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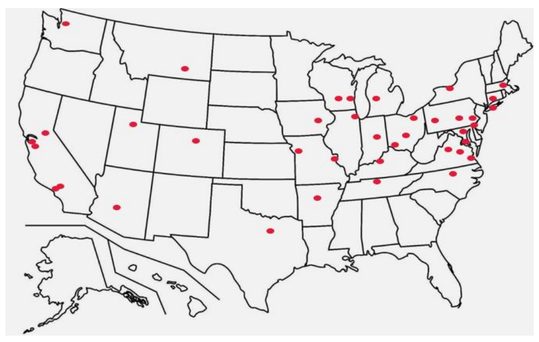
Background

- PPMD is an organization focused on ending Duchenne through research, care, advocacy and engagement[1]. As part of their commitment to optimal care, PPMD established the Certified Duchenne Care Center (CDCC's) program, aimed to ensure that children living with a dystrophinopathy have access to high quality, comprehensive, coordinated care. The program currently consists of 39 CDCC's across the US (Figure 1.0)[2].

Figure 1.0 PPMD Certified Duchenne Care Centers[2]



- Lifespan care is becoming increasingly important in dystrophinopathy management as advances in disease-modifying therapies have led to a growing adult population living with Duchenne and Becker muscular dystrophy (Becker).
- Understanding the challenges families face in accessing adult care, and those providers face in developing adult care models, is essential to improving next steps in providing optimal transfer of care.

Methods

- Exploratory interviews were conducted with two families and three healthcare providers identified by PPMD. Families had experience with the transition to adult care, and providers had expertise in caring for adults with dystrophinopathy and attempting to establish adult care clinics.
- Families shared their experiences accessing adult care and transitioning from pediatric to adult services, while healthcare providers discussed challenges in developing adult care models. Both groups offered insights and recommendations based on their experiences.
- Qualitative analysis of participant insights was conducted to identify recurring themes and generate considerations for the establishment and implementation of adult care centers.

Participants

Figure 2.0 Lived Experience Experts

- Family #1
  - Son born in 1986, diagnosed ~1989, currently age 39 years
  - Initial care encounter at pediatric hospital, no multidisciplinary care model, RN absorbed burden of care coordination
  - Various medical issues led parents to pursue transfer of care options on a system-by-system basis
  - Current status: transfer to adult care was competed at age 38, interested in better outcomes for all families and reducing the stress associated with finding and accessing care.
- Family #2
  - Son born in 2005, diagnosed ~2010, currently age 20 years
  - Initial care encounter at pediatric hospital, which moved to a multidisciplinary care model after a few years with help of PPMD
  - Relocation of family due to job change led to transfer of care options being sought by parents
  - Current status: transfer to adult care still in progress, currently looking for real-time options across multiple body systems

Parent Project Muscular Dystrophy

Challenges Faced by Families

- Family perspectives provided insights into the challenges that many individuals have, or may eventually face when their loved one living with Duchenne transfers to adult care. In both participant cases, identifying, managing and coordinating care has or will fall to the family.

Figure 3.0 Family Perspectives on Transfer to Adult Care

Fragmented Care
- Leads to fragmented, uncoordinated specialty care, placing the burden of communication and follow-up on families rather than a multidisciplinary care coordinator. This can overwhelm families and contribute to delayed or missed care.

Limited Expertise
- Limited Duchenne expertise among HCPs can lead to suboptimal care. Families often become "educators" for providers, which can reduce trust and contribute to delayed care until emergencies occur.

Access Constraints
- Limited appointment availability, scheduling barriers, and geographic or economic challenges can delay access to adult providers, therapies, and clinical trials, threatening continuity of care.

No Adult Model
- No multidisciplinary adult care structure or roadmap exists for navigating transfer from pediatric care. Leads to significant shortfalls in care for dystrophinopathy patients and families.

Barriers identified by Healthcare Providers

- Barriers identified by HCPs highlight the need for greater support through funding, data, training, care coordination and multidisciplinary approaches as adult care modes are established.

Figure 4.0 HCP Perspectives on Transfer to Adult Care

Economic Barriers
- Financial sustainability is a core barrier to maintaining an adult Duchenne clinic.
- Administrators prioritize financial performance; specialized neuromuscular clinics may operate at a loss.
- External funding and reimbursement constraints limit care coordination, comprehensive services, and programs.

Provider Experience Limitations
- Adults living with Duchenne represent a "new disease" without a natural history to guide providers.
- No established benchmark for typical disease progression at this life stage.

Changing Patient Needs
- Adult care providers report caring for patients during a complex stage of disease progression marked by evolving clinical needs and mental health challenges.
- As patients age, care shifts from neurology-focused management to addressing cardiac and pulmonary complications, challenging traditional pediatric care models.

Patient-Centric Care
- Care models must address a full patient spectrum, including cognitive impairments and those not pursuing higher education or independent living.
- Community involvement and advocacy are essential for young men with Duchenne.

Conclusions

Based on this small sample of exploratory interviews, there is an urgent need to establish adult care models that provide expertise in dystrophinopathy management, multidisciplinary supports, and improved care coordination and access.

Likewise, it appears that this is a significant undertaking, with more research needed to understand the natural history of the aging Duchenne population, educational needs of treating clinicians, and the ability of the healthcare system to incorporate these models in a sustainable way.

By considering these issues, the community is likely to identified models suitable for supporting long-term care that provides optimal outcomes for the growing population of adult patients with Duchenne.

Considerations / Next Steps

- The growing adult Duchenne and Becker population highlights the need for lifespan care, including adult-focused goals and expectations. Future research should examine differences in pediatric and adult priorities, including (but not limited to) health, education, employment, insurance, and independent decision-making.

Fig. 5.0 Future Considerations

Care Guidelines & Support
- Can the Duchenne Registry be expanded to include domains important to adult care and generate data on care transitions, quality of care, and outcomes to inform adult care guidelines and standards?
- What should comprehensive guidelines for transition and transfer to adult care include, and should they address both healthcare transfer and broader transition-to-adulthood needs?
- What is the optimal adult care model for dystrophinopathy, including the role of neuromuscular care as the medical home and strategies to improve patient medical and insurance literacy for independent healthcare management?

Medical Expertise
- How can existing natural history data, published evidence, provider expertise, and infrastructure support be leveraged to better understand and prepare for the long-term care needs of adults living with Duchenne into mid- and late adulthood?

Financial Outcomes
- How can existing transition and care delivery data be leveraged to quantify loss to follow-up, evaluate outcomes, and build a sustainable business case for multidisciplinary adult dystrophinopathy care, including the role of CDCCs and family-led support?

Philanthropy
- How can PPMD partner with families, adults living with Duchenne, and healthcare systems to advance advocacy, philanthropy, and the development of sustainable adult care models?
- What role can existing initiatives, pilot programs, CDCCs, and family leaders play in building and scaling adult dystrophinopathy care across regions?
- How can emerging therapies and innovative funding approaches, including equity-based models, be leveraged to support sustainable adult care infrastructure?

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References

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