

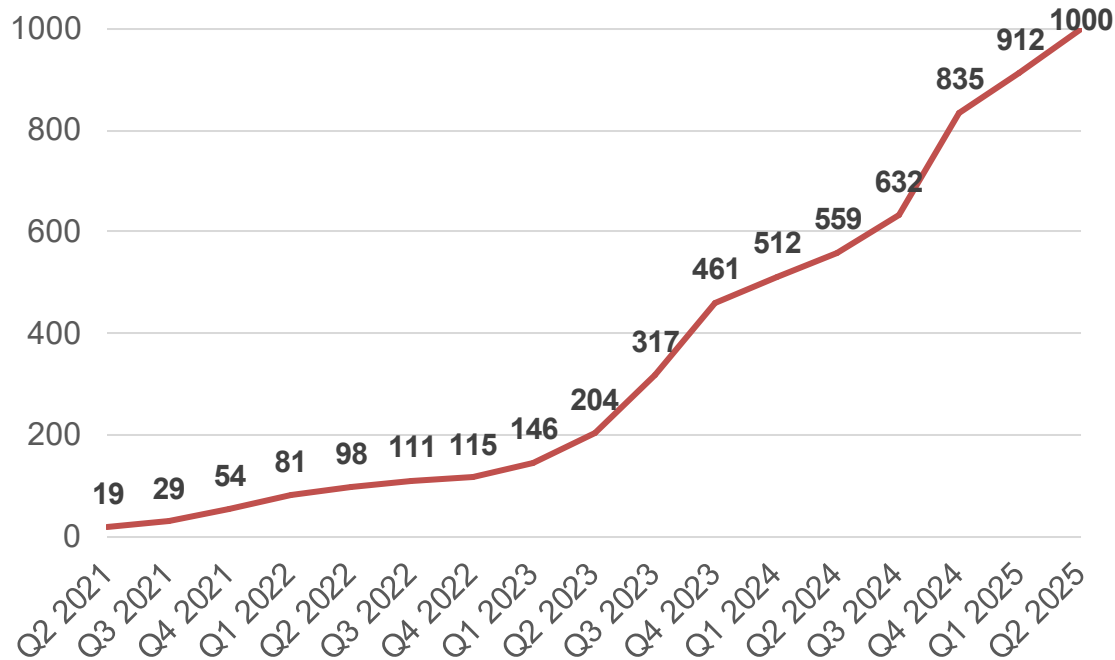
# First 1,000 Patients Enrolled in ACTION: What We Have Learned

**Parent  
Project  
Muscular  
Dystrophy**

Emily A. Hayes, MD  
June 26<sup>th</sup>, 2026

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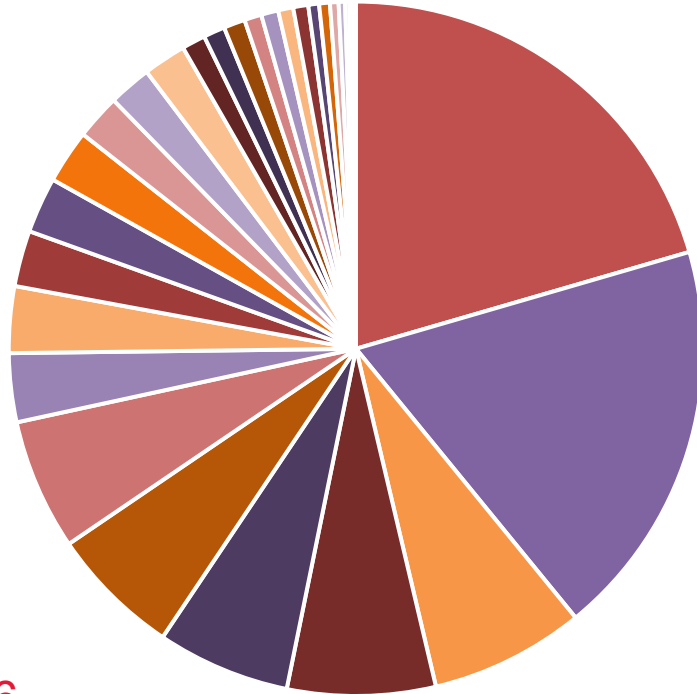
# Enrollment By Quarter



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# Enrollment By Participating Centers

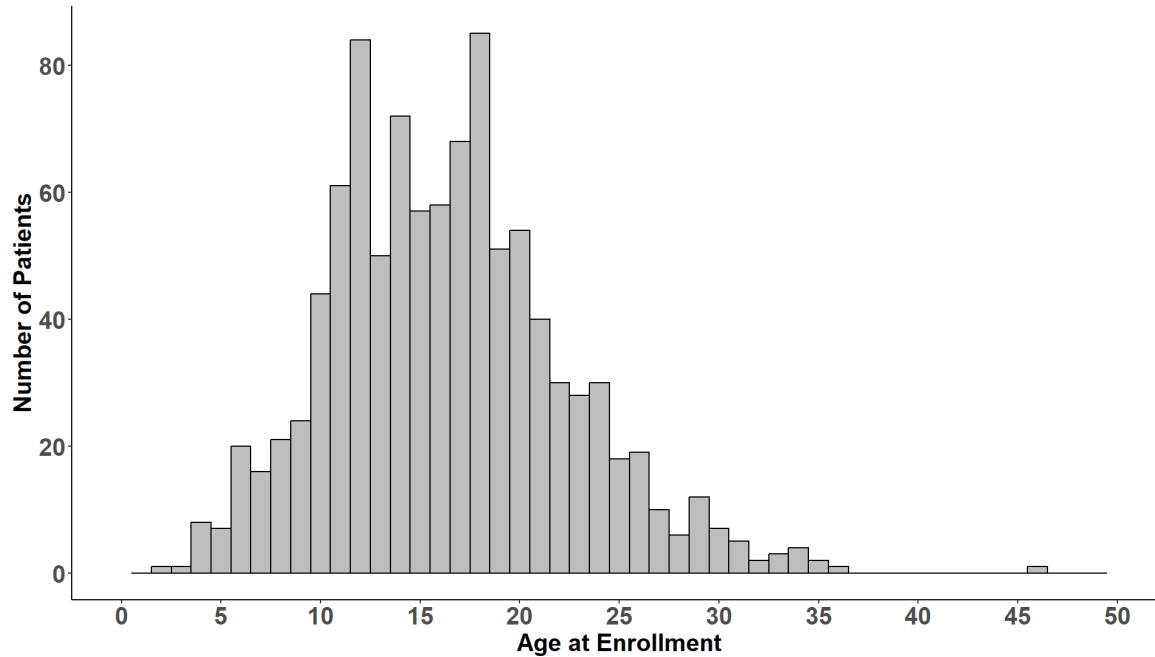


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# Age at Enrollment



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# Cohort Characteristics

Median Age of 16 years

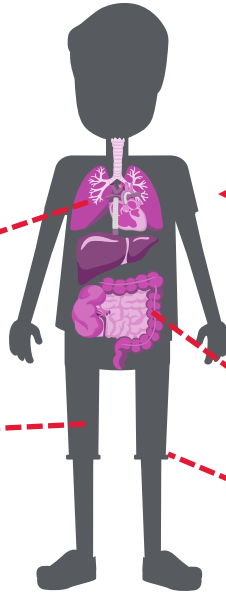
68% No respiratory support

44% Ambulatory

87% Duchenne  
muscular  
dystrophy

13% Gene therapy

79% steroids



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# Cardiac Medications

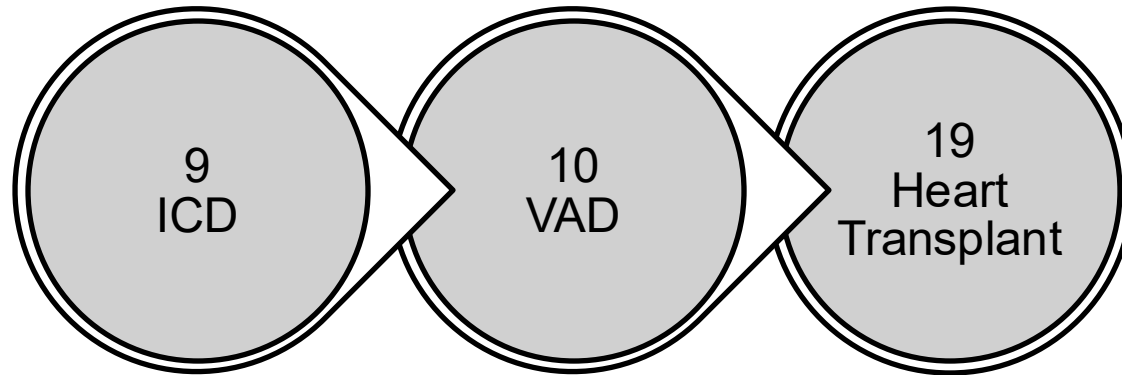
- 83% on ACE/ARB/ARNI
- 59% MRA
- 45% on Beta blocker



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# Advanced Cardiac Therapies



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# What ACTION Has Taught Us

- Cardiac medication use
- Characteristics of the first 500 patients
- VAD use in muscular dystrophy patients
- Additional analyses ongoing




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# ActionLearningNetwork.org

## Our Publications

Our Transparency Leads to ACTION

 **65** Manuscripts Published

 **106** Abstracts Accepted

 **43** Harmonized Protocols

### Manuscripts Published




**RESET**

2026 2025 2024 2023 2022 2021 2020 2019 2018

CardioMEMs CHD Consensus/Guidelines Fontan Heart Failure HeartMate 3™

Medicines **Muscular Dystrophy** VADs Berlin Heart EXCOR® Donor Utilization

Impella® Other Devices Other Pubs



### Abstracts Accepted

- ▶ 2026
- ▶ 2025
- ▶ 2024
- ▶ 2023
- ▶ 2022
- ▶ 2021
- ▶ 2020
- ▶ 2019

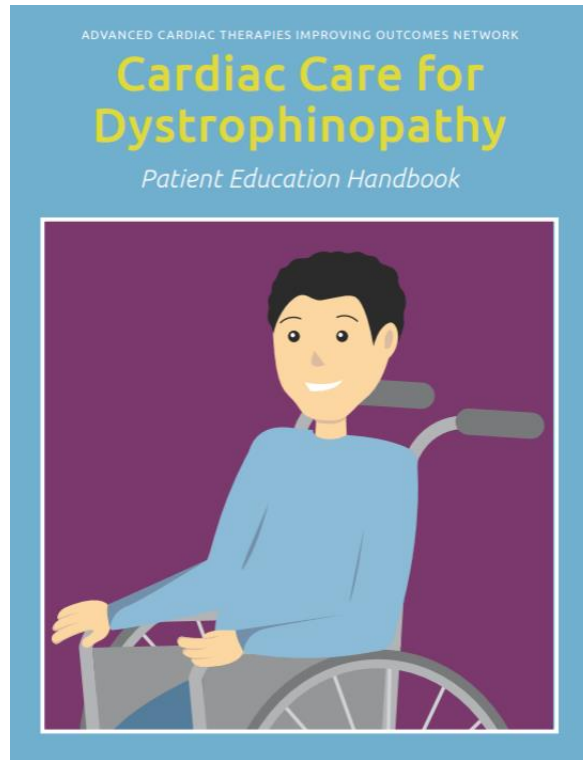


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# Beyond Research: Education & Community

- Educational handouts for patients and providers
- Coming soon: patient and parent handbooks



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# Importance of Registries

- Combines experiences across many centers
- Identifies best practices
- Accelerates research
- Improves future care



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# Questions ACTION Can Answer

- How does gene therapy affect the heart?
- Which patients benefit from advanced therapies?
- How does cardiac disease change with time and therapies?
- What predicts better outcomes?



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# Takeaways

- **ACTION-MD now includes >1000 patients from 30 centers**
- **Understand real-world use of cardiac medications, gene therapy, and advanced therapies**
- **Next phase will focus on understanding longer term outcomes and impact of therapies on heart health.**
- **Every participating patient and family contributes to improving care for the entire community – please enroll!**



**Questions?**

**Thank you!**