

Coming to Consensus: *Updated Considerations for Cardiac Care*

**Parent
Project
Muscular
Dystrophy**

Carol Wittlieb-Weber, MD
June 26, 2026

Multidisciplinary Care



PP
MD

#PPMD2026

Care Considerations 2018

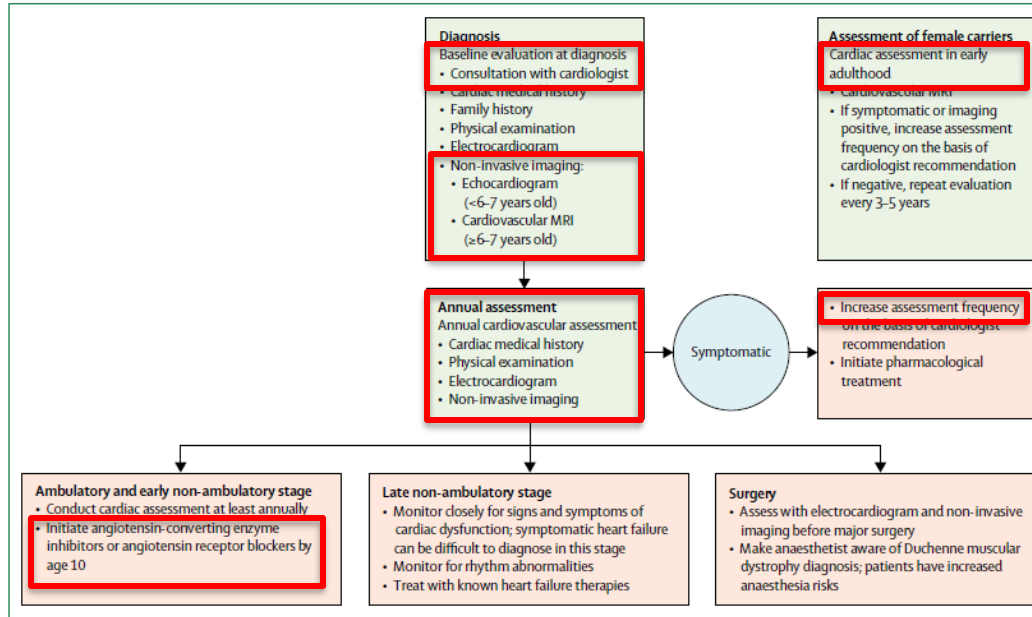


Figure 2: Cardiac monitoring, diagnosis, and treatment algorithm for patients with Duchenne muscular dystrophy

What's New?

Elevidys

delandistrogene
moxeparvovec-rokl

suspension for intravenous infusion



**PP
MD**

#PPMD2026

What's New?

[ABOUT DUCHENNE](#)[CARE](#)[ADVOCACY](#)[RESEARCH](#)[GET INVOLVED](#)[Duchenne Registry](#)[Donate](#)

NEWS:

MASSACHUSETTS BEGINS
NEWBORN SCREENING FOR DUCHENNE



JUNE 5, 2026

Massachusetts Begins Newborn Screening for Duchenne

As of today, Massachusetts has officially begun screening all newborns for Duchenne muscular dystrophy. This milestone comes after the passage of the state's Maternal Health bill (H. 4999), which was signed into law by Governor...

[LEARN MORE >](#)

PPMD JOINS FDA LEADERSHIP
FOR RARE DISEASE
ROUNDTABLE



JUNE 3, 2026

PPMD Joins FDA Leadership for Rare Disease Roundtable

On Wednesday, June 3rd, PPMD was invited to participate in a closed roundtable with senior leadership at the U.S. Food and Drug Administration (FDA). The meeting was convened by Amy Cornstock Rick, JD, Director of...

[LEARN MORE >](#)

NEWS:

DYNE SUBMITS BLA TO FDA FOR
Z-ROSTUDIRSEN (DYNE-251) IN DUCHENNE
AMENABLE TO EXON 51 SKIPPING



MAY 26, 2026

Dyne Submits BLA to FDA for z-rostudirsen (DYNE-251) in Duchenne Amenable to Exon 51 Skipping

Today, Dyne Therapeutics announced that the company has submitted a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for zeleciment rostudirsen (z-



#PPMD2026

What's New?



ABOUT DUCHENNE

CARE

ADVOCACY

RESEARCH

GET INVOLVED

Duchenne Registry

Donate

NEWS:

MASSACHUSETTS BEGINS
NEWBORN SCREENING FOR DUCHENNE

Project
Muscular
Dystrophy



JUNE 5, 2026

Massachusetts Begins Newborn Screening for Duchenne

As of today, Massachusetts has officially begun screening all newborns for Duchenne muscular dystrophy. This milestone comes after the passage of the state's Maternal Health bill (H. 4999), which was signed into law by Governor...

LEARN MORE >

PPMD JOINS FDA LEADERSHIP
FOR RARE DISEASE
ROUNDTABLE



JUNE 3, 2026

PPMD Joins FDA Leadership for Rare Disease Roundtable

On Wednesday, June 3rd, PPMD was invited to participate in a closed roundtable with senior leadership at the U.S. Food and Drug Administration (FDA). The meeting was convened by Amy Comstock Rick, J.D., Director of...

LEARN MORE >

NEWS:

DYNE SUBMITS BLA TO FDA FOR
Z-ROSTUDIRSEN (DYNE-251) IN DUCHENNE
AMENABLE TO EXON 51 SKIPPING



MAY 26, 2026

Dyne Submits BLA to FDA for z-rostudirsen (DYNE-251) in Duchenne Amenable to Exon 51 Skipping

Today, Dyne Therapeutics announced that the company has submitted a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for zeleciment rostudirsen (z-...



#PPMD2026

What have cardiologists learned?

Percent with LGE (%)

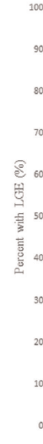


FIGURE 4 Plot of the percent early as 7 years. DMD, Duchenne wileyonlinelibrary.com]

Muscle & Nerve

WILEY

MUSCLE&NERVE

CLINICAL RESEARCH ARTICLE OPEN ACCESS

Prophylactic Use of Cardiac Medications and Survival in Duchenne Muscular Dystrophy

Kristin M. Conway¹ | Shiny Thomas² | Tahereh Neyaz¹ | Emma Cialfoni³ | Joshua R. Mann⁴ | Michelle Staron-Ehlinger⁵ | Gary S. Beasley⁵ | Paul A. Romitti¹ | Katherine D. Mathews⁵ | the Muscular Dystrophy Surveillance, Tracking and Research Network (MD STARnet)

¹Department of Epidemiology, College of Public Health, The University of Iowa, Iowa City, Iowa, USA | ²New York State Department of Health, Albany, New York, USA | ³Department of Neurology, School of Medicine and Dentistry, University of Rochester Medical Center, Rochester, New York, USA | ⁴Department of Preventive Medicine, School of Medicine and John D. Bower School of Population Health, University of Mississippi Medical Center, Jackson, Mississippi, USA | ⁵Stead Family Department of Pediatrics, Roy J. and Lucille A. Carver College of Medicine, The University of Iowa, Iowa City, Iowa, USA

Correspondence: Paul A. Romitti (paul-romitti@uiowa.edu)

Received: 25 April 2024 | Revised: 8 January 2025 | Accepted: 11 January 2025

PP
MD

#PPMD2026

MD
)

n age:
nts <10 years
ears
s-old

Cardiology Working Group

- **Lead:** Carol Wittlieb-Weber
- **Co-Lead:** Jon Soslow
- **Senior Consultants:** Chris Spurney, Linda Cripe
- **Frequency and timing of cardiac surveillance:** Emily Hayes, Teresa Wang, Dominic Fullenkamp
- **Standardized cardiac testing:**
 - Imaging: Kan Hor
 - Rhythm: Andreas Barth
- **Laboratory Monitoring:** Rachel Harris
- **Initiation and Optimization of Cardioprotective Therapy:** Frank Raucci, Paul Estes
- **Cardiac Monitoring in the Evolving Therapeutic Landscape:** Beth Kaufman, Deip Nandi
- **Managing Advanced Heart Failure:** Chet Villa, Larry Markham
- **Acute Cardiac Care:** Tyler Cunningham



#PPMD2026



Cardiology in the Young

cambridge.org/cty

Cardiac treatment for Duchenne muscular dystrophy: consensus recommendations from the ACTION muscular dystrophy committee

Original Article

Cite this article: Estes P, Auerbach SR, Bansal N, Harris R, Soslow JH, Birnbaum D, Conway J, Cripe LH, Nandi D, Hayes E, Gambetta KE, Hall EK, Hsu DT, Kaufman E, Rosenthal D, Kirmani S, Ploutz MS, Lal AK, Peng DM, Villa CR, Shugh S, Wittlieb-Weber and Shih R (2025). Cardiac treatment for Duchenne muscular dystrophy: consensus recommendations from the ACTION committee

Review article

Taking ACTION to detect myocarditis related to recombinant gene transfer therapy for Duchenne Muscular Dystrophy; Consensus recommendations for cardiac surveillance

Beth D. Kaufman¹, Aravindhan Veerapandiyar², Carol Wittlieb-Weber⁴, Paul Estes³, Neha Bansal⁸, Ashwin Lal⁹, Katherine Chet Villa^{1,3} and Deipanjan Nandi^{1,2}

Journal of Neuromuscular Diseases
Vol. 11(6): 1–10
© The Author(s) 2024
Article reuse guidelines:
sagepub.com/journals-permissions
DOI: 10.1177/22143602241303357
journals.sagepub.com/home/jnd



Pediatric Transplantation

WILEY


VIEWPOINT

Heart Transplantation and Ventricular Assist Device in Duchenne Muscular Dystrophy: A New Era

David N. Rosenthal¹, Antonio Amodeo², Russell J. Butterfield³, Ryan Butts⁴, Stephen Chrzanoski⁵, Linda Cripe⁶, John Day¹, Ryan Davies⁴, Tina Duong¹, Patrick Evers⁷, Kathryn Gambetta⁸, Rachel Harris⁹, Emily Hayes⁶, Beth Kaufman¹, Angela Lorts¹⁰, Gordon Mack¹¹, Samuel J. Mackenzie¹², Pradeep Mammen¹³, Larry Markham¹⁴, Jennifer McAlister¹⁵, Craig McDonald¹⁶, Stephanie J. Nakano¹⁷, Deipanjan Nandi¹⁸, Manchula Navaratnam¹⁹, Stanley Nelson¹⁸, John J. Parent¹⁴, Cara Piccoli¹, Marc Richmond¹⁵, Carolina Rocha¹, Hemant Sawhani¹⁰, Rachel Schrader¹⁹, Richard Shell⁶, Renata Shih²⁰, Svetlana Shugh²¹, Theodore Smart¹⁹, John Soslow²², Joseph Spinner²³, Tanja Taivassalo²⁴, Hari Tunugula²³, Christina VanderPluym²⁵, Aravindhan Veerapandiyar²⁶, Chet Villa¹⁰, Carol Wittlieb-Weber²⁷, Marwa Zafarullah¹, Seth Hollander¹



#PPMD2026

The background features large, faint, dark red letters 'PMP' in a bold, sans-serif font, centered on a solid red background. The letters are slightly transparent, allowing the red background to show through.

**Sneak peak at the *new* cardiac
recommendations**

Cardiology Visits

What has been recommended?

- First cardiology visit at the time of diagnosis

What's New?

- First cardiology visit at the time of diagnosis regardless of age
- <10 years: at least annual cardiac assessment
- >10 years: frequency of cardiac assessment may increase

Why?

- Outline clinical course and expected surveillance
- Discuss novel therapies and potential cardiac impact
- Discuss carrier status

Cardiac Imaging

What has been recommended?

- First cardiology visit at the time of diagnosis associated with cardiac testing

What's New?

- Cardiac testing with initial EKG/echo no later than age 5 years DMD, 10 years BMD
- Routine use of MRI should begin at an age when the patient is able to lie still, without sedation, and they can hold their breath

Why?

- With early diagnosis, cardiac testing may not be indicated at first visit
- Dystrophinopathy is a spectrum
- Different centers have different access to MRI

Cardiac Medications

What has been recommended?

- DMD: Initiate prophylactic ACEi or ARB by age 10

What's New?

- Prophylactic ACEi/ARB by age 8 is recommended
- Prophylactic MRA by age 10 years is recommended
- Beta blockade is recommended for the treatment of inappropriate sinus tachycardia

Why?

- We want to be 'proactive' in delaying the onset and progression of cardiomyopathy



#PPMD2026

Management of Carriers

What has been recommended?

- Cardiac assessment in early adulthood.

What's New?

- Mothers: Baseline cardiac evaluation with EKG, Holter monitor, and noninvasive imaging should be performed at the time carrier testing is confirmed
- Sisters: Baseline cardiac evaluation with EKG and noninvasive imaging should be performed around age 10-12 years or at time carrier testing is confirmed if older

Why?

- Female dystrophinopathy carriers represent a heterogeneous population with variable, but significant, risk for developing cardiomyopathy over the lifespan

Key Takeaways

New recommendations are coming!

Ask your child's heart doctor if they know about them!

Thank you!