

# Meeting Overview: Multidisciplinary Management of Weight Across the Lifespan in Dystrophinopathy:

Jaclyn Tamaroff MD, MSCI

Assistant Professor of Pediatrics

Division of Pediatric Endocrinology and Diabetes

Vanderbilt University Medical Center

**Parent  
Project  
Muscular  
Dystrophy**

Meeting Chair: Nadia Merchant, MD

Co-chair: Jaclyn Tamaroff, MD, MSCI

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# Disclosures

- **Consulting: Catalyst, Wave Life Sciences**
- **Research funding: Eli Lilly**

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# A Quick Note on Language

- This presentation discusses “body weight” and “obesity”.
- We recognize that language around weight is personal and that the word “obesity” may feel stigmatizing to some and medically useful to others.
- We are committed to person-centered, compassionate language and aim to honor patient and family preferences when discussing body size, weight, and health.

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# 2018 Consensus Guidelines

## Endocrine Management

Measure standing height every 6 months

Assess non-standing growth every 6 months

Assess pubertal status every 6 months starting by age 9 years

Provide family education and stress dose steroid prescription if on glucocorticoids

## Gastrointestinal and nutrition

Include assessment by dietician nutritionist at clinic visit (every 6 months); initiate obesity prevention strategies; monitor for overweight and underweight, especially during critical

Provide annual assessments of serum 25-hydroxyvitamin D and calcium intake

Assess swallowing dysfunction, constipation, gastro-esophageal reflux disease, and gastroparesis every 6 months

Initiate annual discussion of gastrostomy tube as part of usual care

## Bone Health Management

Assess lateral spine x-rays for occult fracture at diagnosis & at start of steroids, then every year on steroids, every 2 years if not

Consider bisphosphonates at first sign of fracture:

Genant grade 1 or higher vertebral fracture or first long bone fracture

Birnkrant et al,  
2018 PMID:  
29395990  
Birnkrant et al,  
2018 PMID:  
29395989

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# AAP Guidelines (2023)

FROM THE AMERICAN ACADEMY OF PEDIATRICS | CLINICAL PRACTICE GUIDELINE |  
JANUARY 09 2023

## Clinical Practice Guideline for the Evaluation and Treatment of Children and Adolescents With Obesity



Sarah E. Hampl, MD, FAAP ; Sandra G. Hassink, MD, FAAP; Asheley C. Skinner, PhD;  
Sarah C. Armstrong, MD, FAAP; Sarah E. Barlow, MD, MPH, FAAP; [Christopher F. Bolling, MD, FAAP](#);  
Kimberly C. Avila Edwards, MD, FAAP; Ihuoma Eneli, MD, MS, FAAP; Robin Hamre, MPH;  
Madeline M. Joseph, MD, FAAP; Doug Lunsford, MEd; Eneida Mendonca, MD, PhD, FAAP;  
Marc P. Michalsky, MD, MBA, FAAP; Nazrat Mirza, MD, ScD, FAAP; Eduardo R. Ochoa, Jr, MD, FAAP;  
Mona Sharifi, MD, MPH, FAAP; Amanda E. Staiano, PhD, MPP; Ashley E. Weedn, MD, MPH, FAAP;  
Susan K. Flinn, MA; Jeanne Lindros, MPH; Kymika Okechukwu, MPA

Address correspondence to Sarah Hampl, MD. E-mail: [shampl@cmh.edu](mailto:shampl@cmh.edu)

**FINANCIAL/CONFLICT OF INTEREST DISCLOSURES:** An Independent review for bias was completed by the American Academy of Pediatrics. Dr Barlow has disclosed a financial relationship with the Eunice Kennedy Shriver National Institute of Child Health and Human Development as a co-investigator.

*Pediatrics* (2023) 151 (2): e2022060640.

<https://doi.org/10.1542/peds.2022-060640> **Article history** 

How can we (and should we) apply these guidelines to individuals with dystrophinopathies?

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# What is Comprehensive Treatment?

“The essential components of comprehensive obesity treatment of children and adolescents include **treatment of the obesity as a chronic disease and evaluation and management of comorbidities.**”

“Ideally, primary care teams (**neuromuscular**) and pediatric weight management specialty (**endocrinologist and dietician**) teams will partner to provide COT for children and adolescents”

- Intensive, longitudinal treatment
- Medical Home
- Metabolic comorbidities
- Social Drivers of Health
- Addressing bullying and psychological distress
- Nonstigmatizing approaches
- Motivational interviewing
- Collaborative treatment goals → comorbidities, quality of life, self-image
- Weight management strategies across disciplines
  - Health behavior, pharmacotherapy
- Tailor treatment to ongoing needs of individual, family, community

HAMPL et al 2023.  
PMID: 36622115

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# Weight Management in DMD

Current recommendations:

**Evidence based guidelines for weight management in DMD do not exist**

Recommendations for traditional weight loss strategies  
(except for physical activity)

Structured daily eating, work with dietician, weight  
management clinic

Minimal data on medication management

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Weber et al, 2018. PMID: 30275248  
Casteels et al, 2010. PMID: 19496972  
Billich et al, 2024. PMID: 38353295  
Arikian et al, 2010. PMID: 21150251  
McPherson et al, 2018. PMID: 28272972  
Carter et al, 2005. PMID: 15779019



OPTIMIZING ENDOCRINE  
AND BONE HEALTH  
MANAGEMENT IN  
DUCHENNE MUSCULAR DYSTROPHY

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# Potential Associations with Elevated Weight in DMD



Quality of life concerns



Mostue Naume et al, 2023. PMID: 36579362  
Canapari et al, 2015. PMID: 24644236  
Billich et al, 2022. PMID: 36014811  
Van de Velde et al, 2019. PMID: 30400104  
Freytag et al, 2023. PMID: 35549789  
Henson et al, 2021. PMID: 34569261

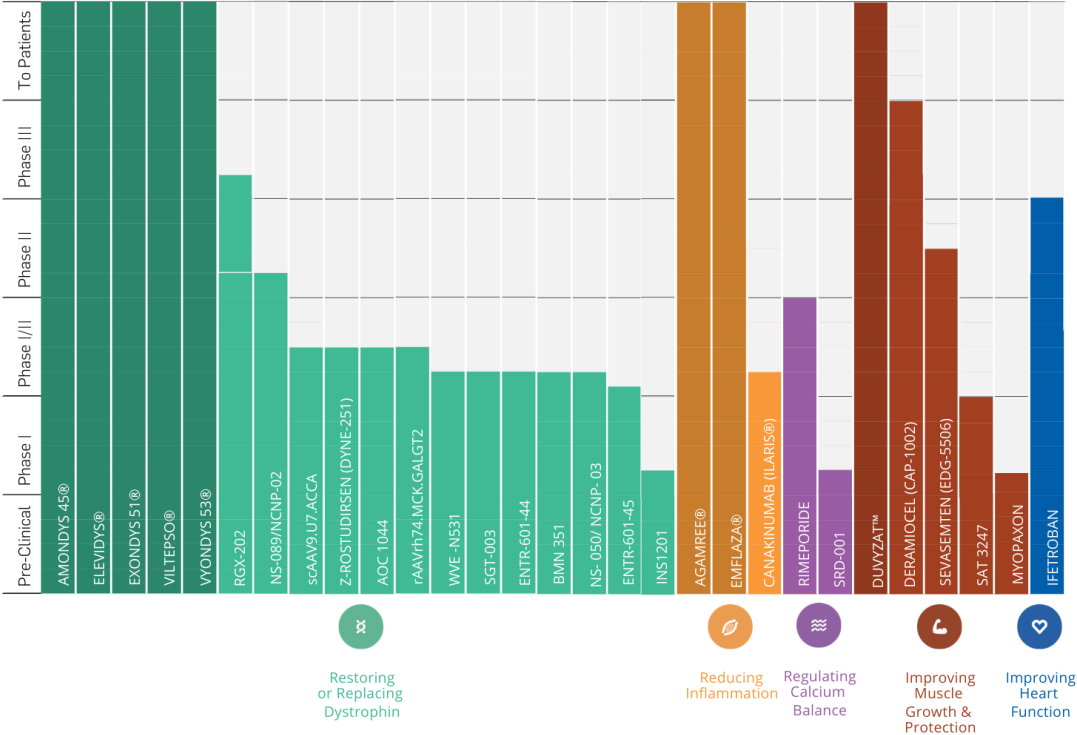
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# Changing Landscape of DMD Care

## DUCHENNE DRUG DEVELOPMENT PIPELINE

The Drug Development Pipeline is full of potential treatments that are being tested. These include therapeutic approaches that restore or replace dystrophin and those that treat Duchenne symptoms. The goal? To test combinations of these therapies to create the best "cocktail" for each patient.

Parent Project Muscular Dystrophy  
 JOIN THE FIGHT. END DUCHENNE.

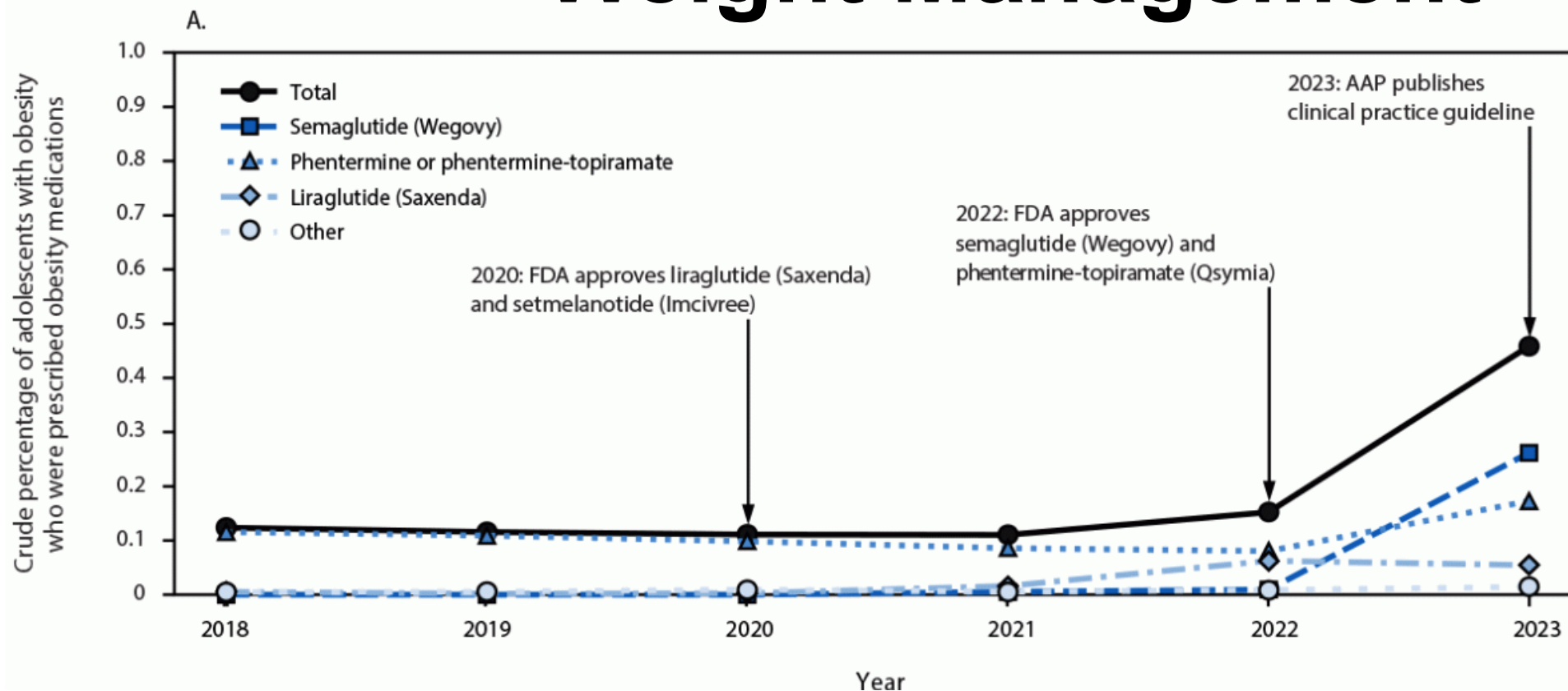


March 2026

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# Changing Landscape of Medications for Weight Management



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[https://wwwsw.cdc.gov/mmwr/volumes/74/wr/mm7420a1.htm?s\\_cid=mm7420a1\\_w#F1\\_down](https://wwwsw.cdc.gov/mmwr/volumes/74/wr/mm7420a1.htm?s_cid=mm7420a1_w#F1_down)

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# Conference Goals

Discuss **multidisciplinary** and **comprehensive** care of individuals with dystrophinopathy **across the lifespan.**

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Multidisciplinary  
Management of  
Obesity Across the  
Lifespan in  
Dystrophinopathy

May 14-15, 2026 | Dallas, TX

The Westin Dallas Downtown

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# Conference Goals

**Multidisciplinary:** build on ongoing work through OPTIMIZE DMD (more endocrine/neuromuscular focused)

**Comprehensive:** discuss new pharmacotherapy options but want to think broadly beyond medication management alone and the potential short and long-term impacts of medications.

**Across the lifespan:** importance of quality of life and focus on long term chronic conditions

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# Importance of Multidisciplinary Discussions Across Pediatrics and Adult Medicine

- Endocrinology
- Obesity medicine
- Registered dietitian
- Cardiology
- Gastroenterology/hepatology
- Neurology
- Psychology
- Physical therapy
- Pulmonology
- Social work
- Research

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# Conference Sessions

- **The Changing Dystrophinopathy Landscape and Why Obesity Matters**
- **The Systemic Impact of Obesity Across Body Systems**
- **Quality of Life Implications**
- **How Can Data Inform Clinical Practice?**
- **Medication Use in the Management of Obesity in Dystrophinopathy**
- **Moving the Needle Forward**



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# Conference Takeaways

1. This is an important topic
2. There is an interest in medication management in certain individuals
3. Research is needed!



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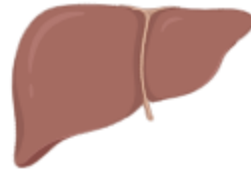
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# What Can we (potentially) Implement Now?

- **Earlier discussions**



- **Consider liver screening**



- **Medication management**



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# Earlier Discussions About Healthy Habits

Family-centered healthy lifestyle discussions

Start in toddler years

Regular DMD dietician and endocrine visits earlier

Continue to focus on transitions



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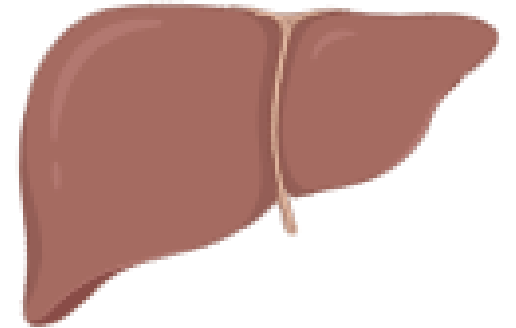
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# Liver Screening (MASLD)

**Consider screening for metabolic dysfunction-associated steatotic liver disease (MASLD) in those with obesity (10years+)**

- **Already may be getting HbA1c and lipids**
- **Consider a GGT (liver lab less impacted by muscle)**
- **If GGT significant elevated (over 100U/L) → consider imaging or GI evaluation**



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# Medications

## Multiple medication options:

- Metformin
- Topiramate/Phentermine
- Glucagon-like Peptide-1 Receptor-Agonists (GLP-1 RA)
  - semaglutide, liraglutide, tirzepatide



***None are routinely recommended***

**Very limited data in DMD**

**BUT they are starting to be used**

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# GLP-1 RAs



**Not routinely recommending but may have benefit for some individuals (unknown if DMD specific risks).**

- Multiple approved for weight loss and diabetes (T2D) in adolescents and adults
- Mechanisms: increase post-meal insulin, slow gastric emptying, decrease appetite, and more
- Currently approved GLP-1 RAs for weight (June 2026):

Generic	Brand names Weight (T2D)	How it is used	Obesity Approvals (FDA) (T2D approval)
Liraglutide	Saxenda (Victoza)	Daily SQ	Age 12+ (age 10+)
Semaglutide	Wegovy (Ozempic)	Weekly SQ	Age 12+ (age 18+)
Semaglutide	Wegovy (Rybelsus)	Daily oral	Age 18+ (age 18+)
Tirzepatide (GLP-1RA/ GIP)	Zepbound (Mounjaro)	Weekly SQ	Age 18+ (age 10+)

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# GLP-1 RAs: potential risks



**Not routinely recommending but may have benefit for some individuals (unknown if DMD specific risks).**

## Unknowns:

- Impact on muscle
- Impact on bone
- Changes in body composition
- Risk of GI upset or worsening constipation
- Impact on the heart
- Impact on the lungs
- Excessive weight loss
- Nutrient deficiency

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# GLP-1 RAs



**Not routinely recommending but may have benefit for some individuals (unknown if DMD specific risks).**

## **If using them:**

- Discuss with multidisciplinary team
- Routine cardiac monitoring (cardiology)
  - Cardiac imaging, ?ambulatory monitoring
- Follow function closely (neuromuscular, PT, PM&R)
  - Particular focus if ambulatory
- Dietician before and every 3 months (telehealth?)
- DXA before starting (bone and body composition)
- Optimize bone health (vitamin D, testosterone)
- Bowel regimen/discussion

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# Research Questions

- **Complications: MASLD, high cholesterol, abnormal glucose/insulin**
  - Incidence
  - How do we screen?
  - What to do with results?
  - What are the complications/risks?
- **Impact of obesity on body systems**
  - Cardiac, pulmonary, neuromuscular, bone
- **Family-centered care**
  - What do families want from testing/management options
  - Overcoming obstacles to care
- **Medication management**
  - Risks/Benefits
  - When do we consider starting
  - Registry/clinical data and randomized controlled trial

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# Thank you

## Multidisciplinary Management of Obesity Across the Lifespan in Dystrophinopathy

May 14-15, 2026 | Dallas, TX  
The Westin Dallas Downtown

- Jonathan Soslow, MD, MSCI
- Ashley Shoemaker, MD, MSCI
- Andrea Lee, MA, MLS
- Nicholas Joy, BS
- Kimberly Crum, RN
- DMD clinic at Monroe Carell Jr. Children's Hospital at Vanderbilt
- OPTIMIZE DMD Consortium
- **Nadia Merchant, MD**
- **Nora Renthal, MD, PhD**
- **Participants in the Multidisciplinary Management of Obesity conference**

### Current funding:

- AHA Second Century Early Faculty Independence Award
- NIH NIDDK 3R01DK118407-03S1 (PI: Shoemaker)
- VICTR: CTSA UL1TR002243
- Katherine Dodd Faculty Scholars Program

### Completed:

- K12HD087023 (NICHD, PI: DeBaun, Salazar)
- Fight DMD
- Turner-Hazinski Award
- NIH NIDDK F32DK128970
- NIH T32DK063688
- CHOP Bone and Body Composition Research Affinity Group
- Friedreich's Ataxia Research Alliance

# Parent Project Muscular Dystrophy



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