

# OPTIMIZE DMD

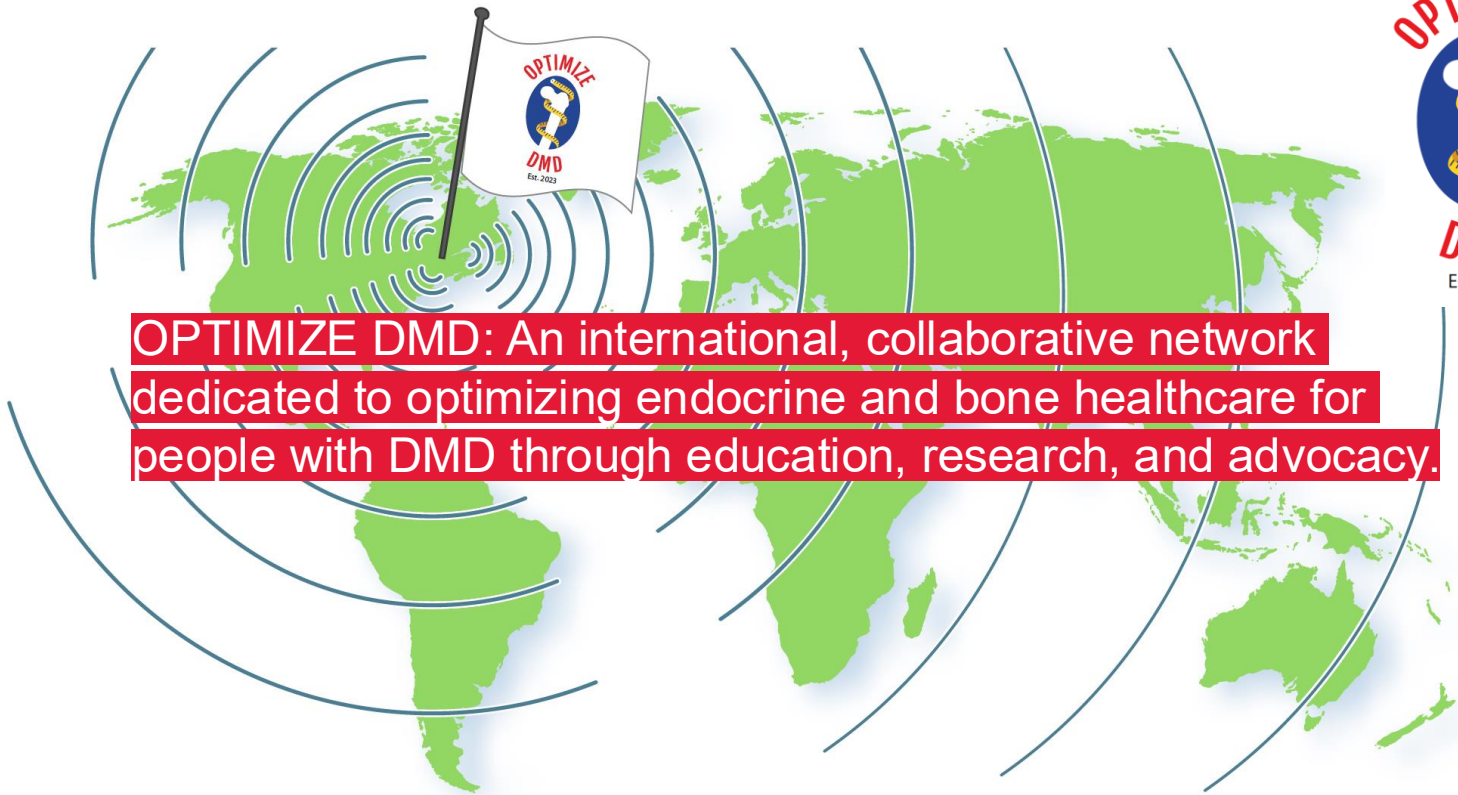
## Optimizing Endocrine & Bone Health Management in DMD

Nora E. Renthal, MD, PhD

Division of Endocrinology | Boston Children's Hospital

**Parent  
Project  
Muscular  
Dystrophy**





**OPTIMIZE DMD: An international, collaborative network dedicated to optimizing endocrine and bone healthcare for people with DMD through education, research, and advocacy.**

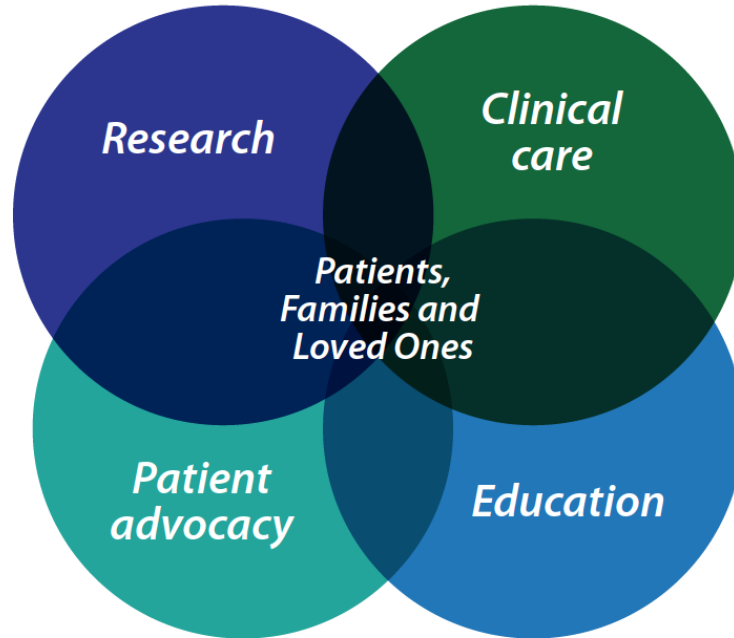


**OPTIMIZING ENDOCRINE AND BONE HEALTH MANAGEMENT IN DUCHENNE MUSCULAR DYSTROPHY**

**PPMD**

**#PPMD2026**

# *Optimizing the Present Care and Future Outlook for Patients and Loved Ones Living with DMD*

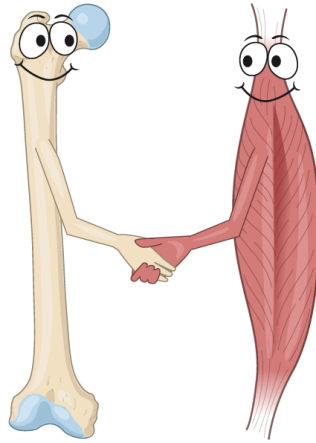


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## Overall Goal

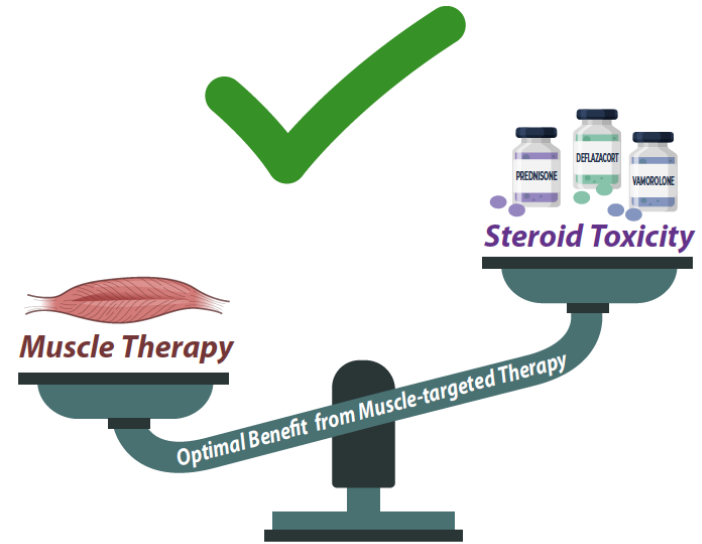
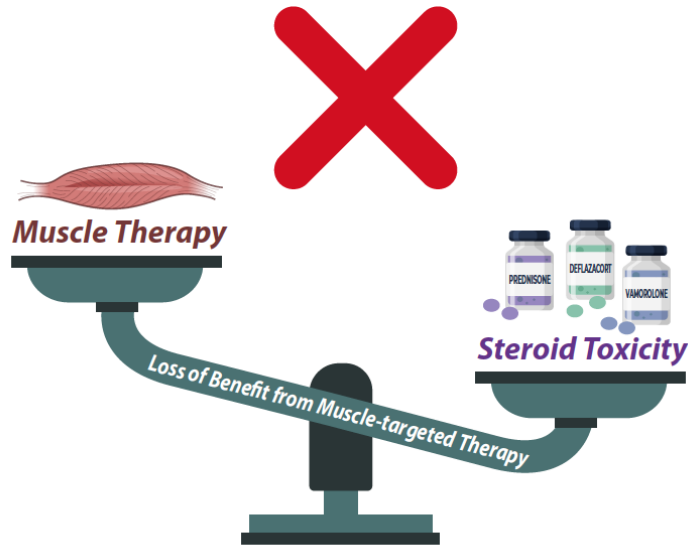
**OPTIMIZE Endocrine and Bone Health Outcomes  
so that Individuals with DMD can  
Maximally Benefit from their Disease-Targeted Therapy**



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# *Our Over-arching Goal is to Help Ensure the Benefits of Muscle-targeted Therapy are not Outweighed by Steroid-related Morbidity*



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# ABC's of ENDOCRINE HEALTH in DMD:



**A** **drenal Insufficiency**  
(secondary to long-term steroid exposure)

**B** **one**  
(osteoporosis & fractures)

**C** **arbs**  
(steroid-induced insulin resistance)

**D** **evelopment**  
(puberty, growth, adulthood & sexuality)

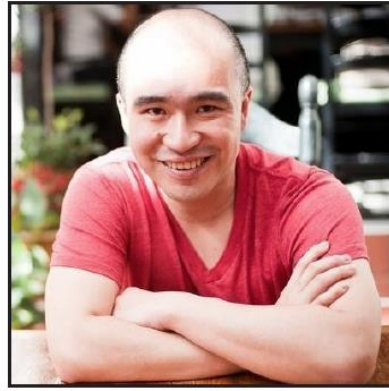
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## Founding Members of OPTIMIZE DMD



**Pat Furlong**  
USA



**Jarod Wong**  
UK



**David Weber**  
USA



**Leanne Ward**  
Canada

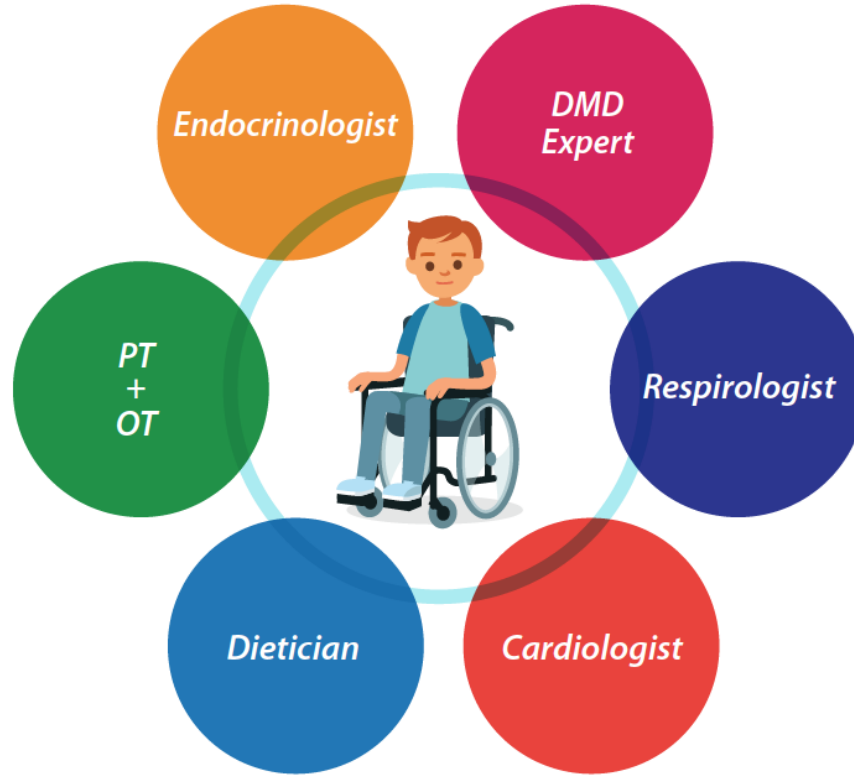
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OPTIMIZING ENDOCRINE  
AND BONE HEALTH  
MANAGEMENT IN  
DUCHENNE MUSCULAR DYSTROPHY

# Early Engagement of an Endocrinologist



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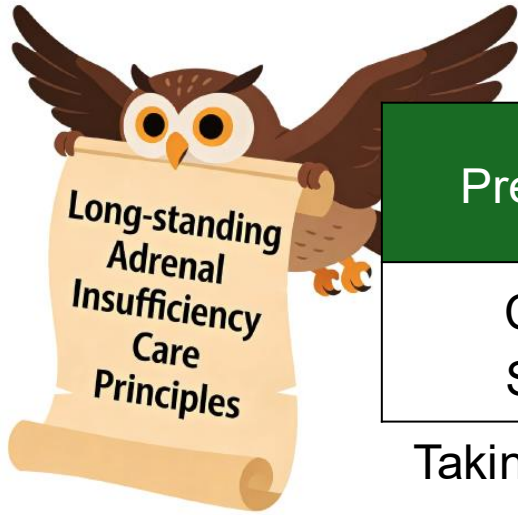
**D**evelopment  
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# Adrenal Insufficiency



Prednisone	Deflazacort (2017)	Vamorolone (2023)
Classic Steroid	Classic Steroid	Dissociative Steroid

Taking any of these steroids makes the adrenal gland “forget” how to make its own steroids.

# Adrenal Insufficiency

*Cortisol is Important for the Body's Ability to Handle "Medical Stress"*

*Fever*



*Vomiting Illness*



*Accidents*



*Surgery*



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# Adrenal Insufficiency Prevention

a co-prescription

**Rx** NAME \_\_\_\_\_  
ADDRESS \_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_  
Prescription:

Muscle-targeted  
GC therapy

\_\_\_\_\_  
SIGNATURE \_\_\_\_\_ DATE \_\_\_\_\_



**Rx** NAME \_\_\_\_\_  
ADDRESS \_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_  
Prescription:

Adrenal  
insufficiency-  
targeted  
GC therapy

\_\_\_\_\_  
SIGNATURE \_\_\_\_\_ DATE \_\_\_\_\_

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***Without “steroid stress dosing”  
patients have the following symptoms:***

***Fatigue***

***Nausea +  
Vomiting***

***Headache***

***Body aches  
+ pains***

***Low blood  
pressure***

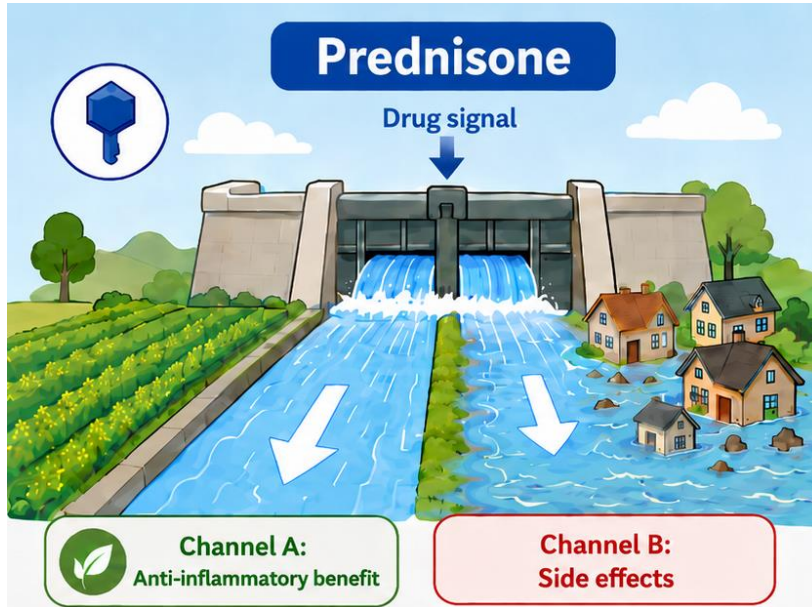
***Lack of appropriate steroid stress dosing can be life-threatening***



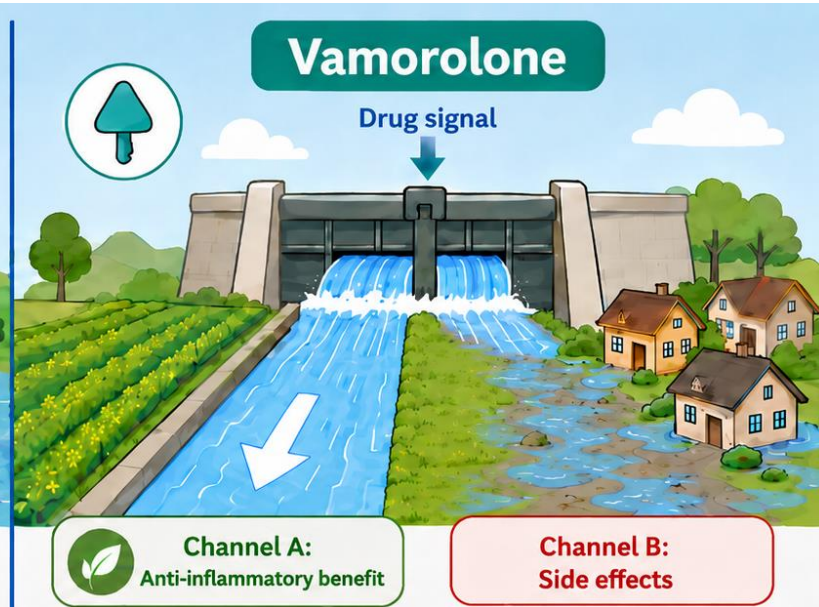
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## Classic Steroids



## Dissociative Steroid



**CANNOT BE USED FOR  
STRESS DOSING / SICK DAYS**

# Adrenal Suppression in Duchenne Muscular Dystrophy: Management Strategies Incorporating Novel Steroid Vamorolone

Anne Marie Sbrocchi,<sup>1</sup> Kathi Kinnett,<sup>2</sup> Maria-Elena Lautatzis,<sup>3</sup> Hugh J. McMillan,<sup>4</sup> Kathryn A. Selby,<sup>5</sup> Aravind Veerapandiyam,<sup>6</sup> David R. Weber,<sup>7,8</sup> Susan Apkon,<sup>9</sup> Diana X. Bharucha-Goebel,<sup>10,11</sup> Sonum Bharill,<sup>12</sup> Shipra Bansal,<sup>6</sup> Paula R. Clemens,<sup>13,14</sup> Melissa Fiscaletti,<sup>15</sup> Rana Halloun,<sup>16</sup> Carol Lam,<sup>17</sup> Nadia Merchant,<sup>18</sup> Laura McAdam,<sup>19</sup> Nat Nasomyont,<sup>20</sup> Stefan Nicolau,<sup>21</sup> Maria F. Ochoa Molina,<sup>22</sup> Kim Phung,<sup>23,24</sup> Meilan M. Rutter,<sup>20</sup> Mena Scavina,<sup>25</sup> Prasanth N. Surampudi,<sup>26</sup> Jaclyn Tamaroff,<sup>27</sup> Cuixia Tian,<sup>28</sup> Leanne M. Ward,<sup>23,24</sup> Claire L. Wood,<sup>29</sup> Sze Choong Wong,<sup>30,31</sup> and Alex Ahmet<sup>23,24</sup> on behalf of The OPTIMIZE DMD Consortium



STEROIDS

**RISK FOR ADRENAL CRISIS – DOWNLOAD PJ'S PROTOCOL**

**parentprojectmd.org**



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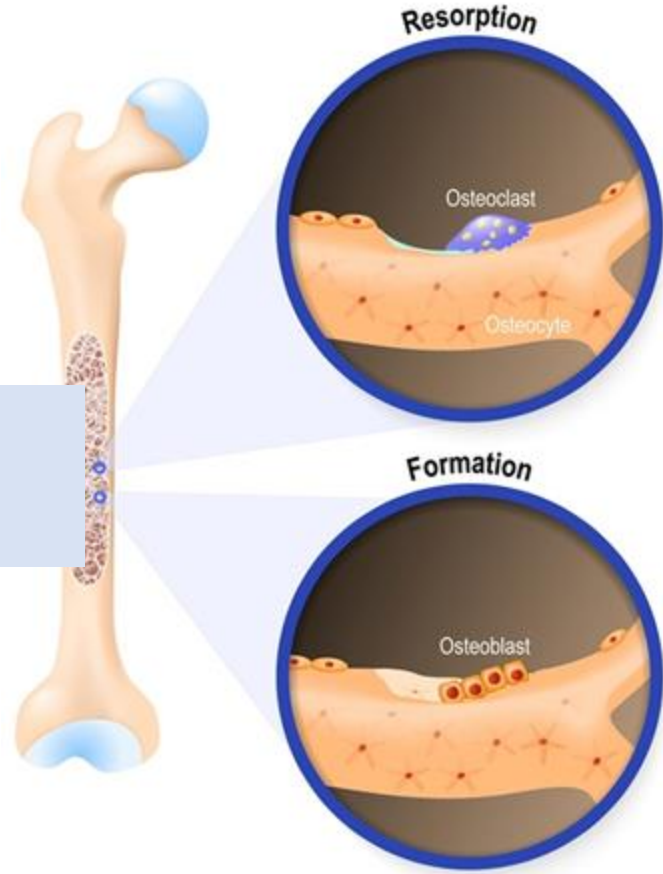
# Bone

(osteoporosis & fractures)

## Steroids: **Tough on** Bones

Uncoupled Bone turnover

Low formation, high resorption



# Treatment of Osteoporosis in DMD

## EVERYONE:

Nutrition: Calcium and vitamin D

## STANDARD CONSIDERATIONS:

Unequivocal indication for IV bisphosphonate therapy

- Single, low-trauma long-bone fracture or
- Vertebral compression fractures
- **Testosterone therapy as indicated**

## NEW CONSIDERATIONS:

Consideration for bisphosphonates **before the first fracture**

- **Progressive vertebral height loss on serial spine x-ray**
- **Low or declining BMD Z-score**



# 2026 – Publications in each area of Endocrine Care of DMD

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# Future Directions of the OPTIMIZE DMD Consortium

Focus on  
Knowledge Dissemination  
and  
Implementation

Nurture  
our  
Research  
Goals

Enrich  
Adult Care Pathways  
+  
Partners

Expand the  
"Ambassador Program"  
with PAGs and HCPs



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**THANK  
YOU!**

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