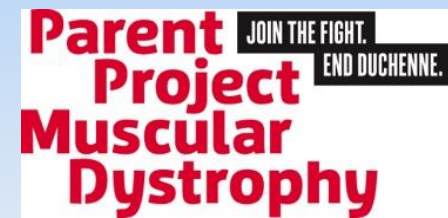


# Patient and Family Engagement: The PJ Nicholoff Steroid Protocol for Duchenne and Becker Muscular Dystrophy and Adrenal Suppression

Joel Feldman, MD, St Vincent

Brian Nicholoff, Father

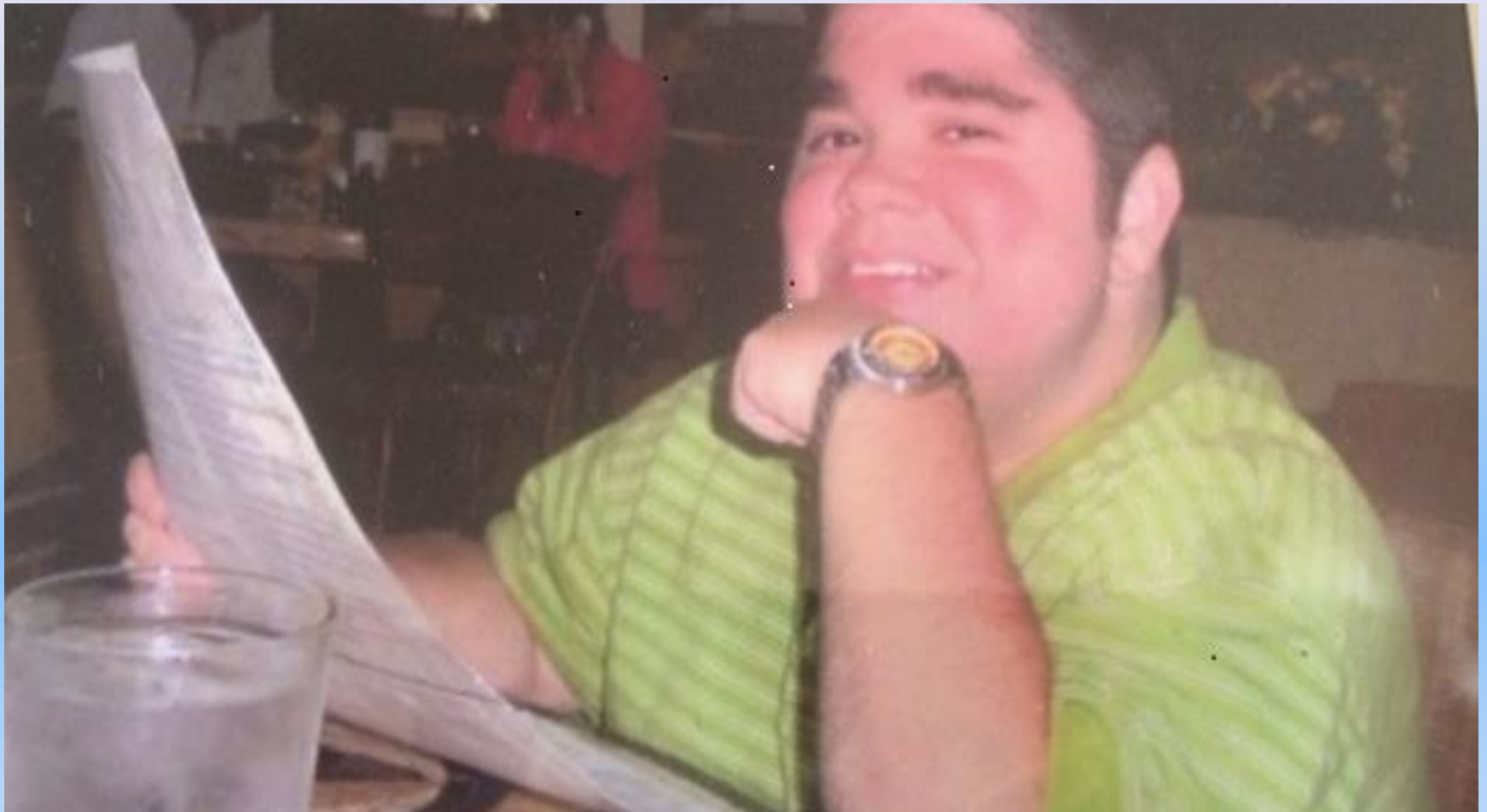
Kathi Kinnett, APRN, PPMD



# Objectives:

- Describe the benefits of patient and family engagement in the resolution of unexpected outcomes.
- Discuss the process of developing and implementing an embedded high-dose steroid protocol in the electronic health record.
- Identify tactics to engage community partners and professional organizations in the development of patient safety projects.

# PJ Nicholoff

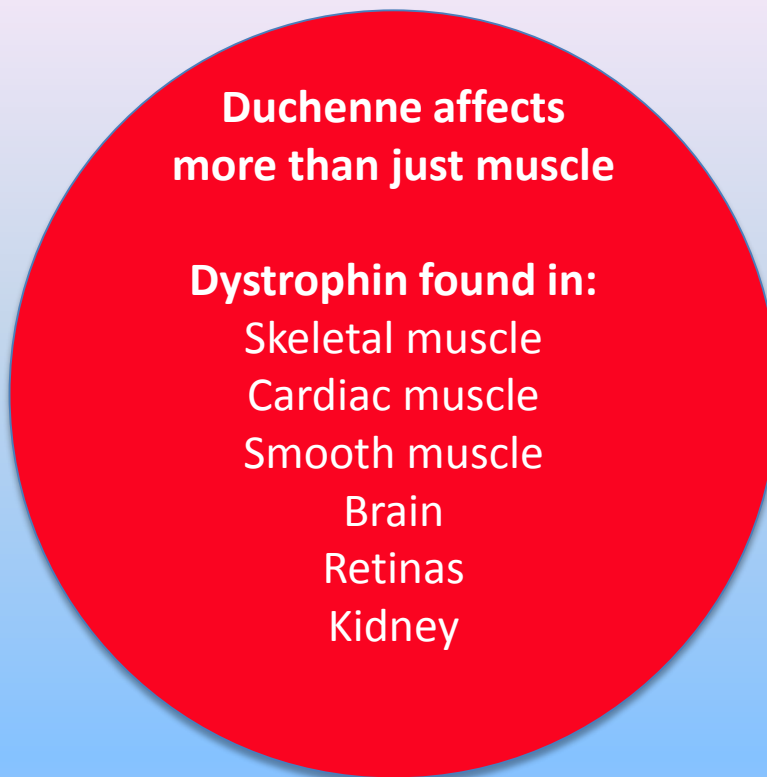




- **POSSIBLE LEARNING AND COGNITIVE DIFFICULTIES** (speech, learning, behavior, sensory, psychosocial))



- **WEAKENS DIAPHRAGM**
- **REQUIRES VENTILATION IN TEENS**
- **LEADS TO PNEUMONIA**



- **DECREASED HEART FUNCTION**
- **CARDIOMYOPATHY**
- **LEADS TO HEART FAILURE**



- **LOSS OF MUSCLE MASS**
- **WEAKNESS**
- **INFLAMMATION**
- **FIBROSIS**

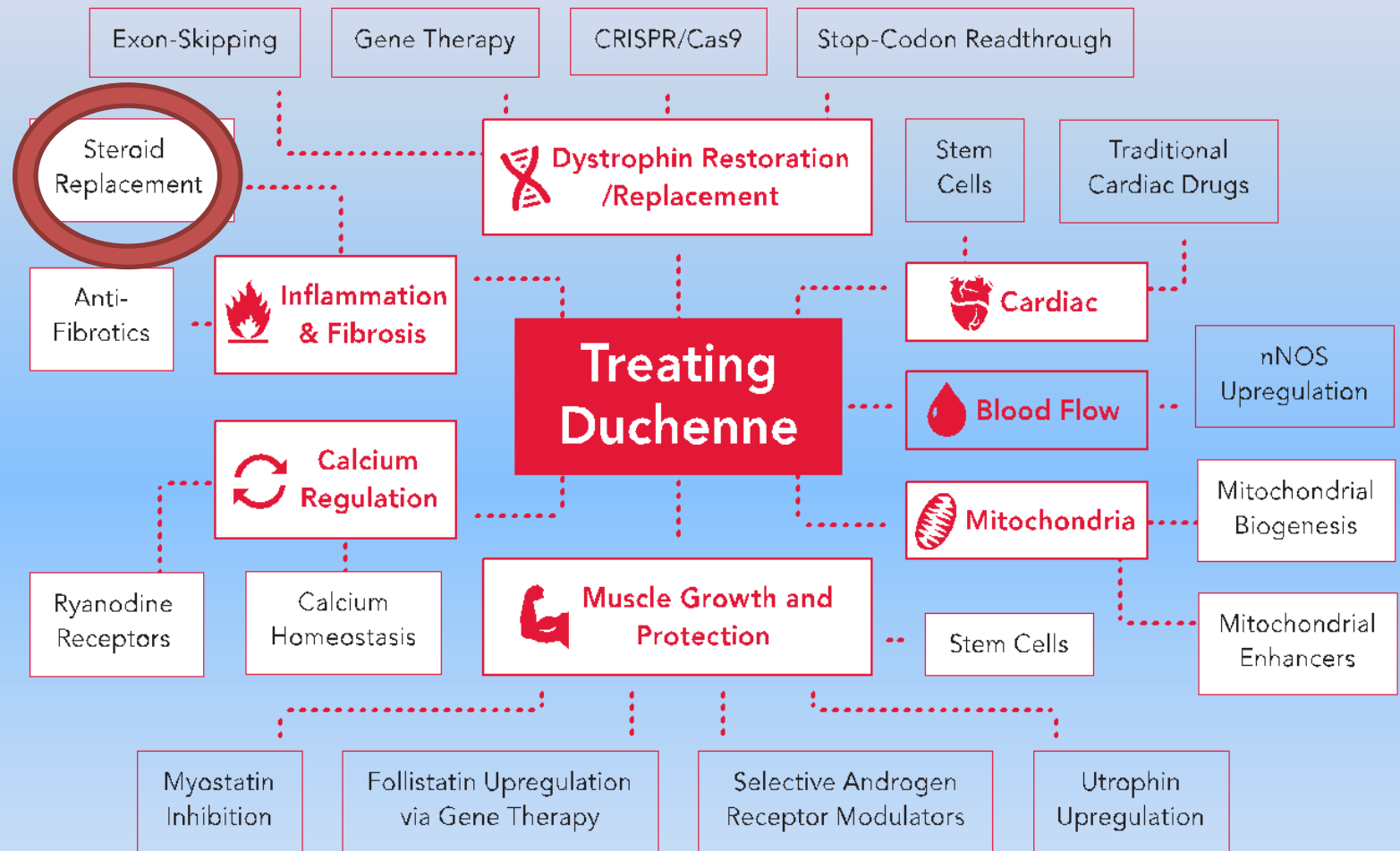


- **Digestive issues**
- **Constipation**
- **Gerd**



- **BRITTLE AND WEAK**

# Clinical Trials in Duchenne



# What Does Cortisol do?

- Hypothalamus-pituitary-adrenal (HPA) Axis



# Daily Steroid Dosing

- Daily steroids
  - Cortisol/cortisone coming from elsewhere
  - Temporarily inactivated adrenal glands
- Adrenal crisis
  - Acute adrenal insufficiency
  - Can be life threatening



# Emergency Care



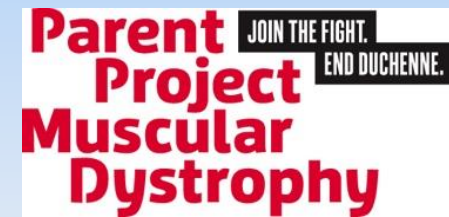
- Vomiting for 24 hours
  - Go to ED
    - Risk for Adrenal Crisis
  - Substitute corticosteroid by IV
    - Can figure out IV dose from oral dose
    - 6mg deflazacort = 5 mg prednisone
  - Remind staff that high liver enzymes (AST, ALT) are normal in Duchenne



# PJ Nicholoff Steroid Protocol

## Authors

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# PJ Nicholoff Steroid Protocol

Parent Project  
Muscular Dystrophy  
LEADING THE FIGHT TO END DUCHENNE

## PJ Nicholoff Steroid Protocol

Background/Assessment



Version 1. [PLOS Curr.](#) 2017 June 27; 9:  
[ecurrents.md.d18deef7dac96ed135e0dc8739917b6e](#).  
Published online 2017 June 27.  
doi: [10.1371/currents.md.d18deef7dac96ed135e0dc8739917b6e](#)  
Advanced Diagnostics and Biomarkers

PMCID: PMC5505768

PMID: [28744411](#)

## The PJ Nicholoff Steroid Protocol for Duchenne and Becker Muscular Dystrophy and Adrenal Suppression

[Kathi Kinnett](#) and [Garey Noritz](#)

# PJ Nicholoff Steroid Protocol

- Deflazacort/prednisone conversion
- Stress doses
- Symptoms of Adrenal Crisis
- Exams/tests to evaluate adrenal function
- Take this with you!



# Dissemination



- Submitted for publication
- Included in the 2017 updated CDC Care Guidelines
- PPMD (and other advocacy) websites
- Social media
- PPMD End Duchenne Tour stops
- Packets to all the PPMD Certified Duchenne Care Centers

