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

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

DECREASED HEART FUNCTION
CARDIOMYOPATHY
LEADS TO HEART FAILURE
WEAKENS DIAPHRAGM
REQUIRES VENTILATION IN TEENS
LEADS TO PNEUMONIA
LOSS OF MUSCLE MASS
WEAKNESS
INFLAMMATION
FIBROSIS

Digestive issues
Constipation
Gerd

BRITTLE AND WEAK

Duchenne affects more than just muscle

Dystrophin found in:
Skeletal muscle
Cardiac muscle
Smooth muscle
Brain
Retinas
Kidney

Manifestations of Duchenne
Clinical Trials in Duchenne

- Exon-Skipping
- Gene Therapy
- CRISPR/Cas9
- Stop-Codon Readthrough

Steroid Replacement

- Anti-Fibrinolics

Inflammation & Fibrosis

- Calcium Regulation

Ryanoine Receptors

- Calcium Homeostasis

Muscle Growth and Protection

- Myostatin Inhibition
- Follistatin Upregulation via Gene Therapy
- Selective Androgen Receptor Modulators
- Utrophin Upregulation

Dystrophin Restoration/Replacement

- Stem Cells

Cardiac

- Blood Flow

Mitochondria

- nNOS Upregulation
- Mitochondrial Biogenesis
- Mitochondrial Enhancers

Project Muscular Dystrophy

Join the fight. End Duchenne.
What Does Cortisol do?

- Hypothalamus-pituitary-adrenal (HPA) Axis

1. Stress
2. Hypothalamus releases CFR
3. Pituitary gland releases ACTH
4. Body's cortisol level increases
5. Body has extra energy to deal with stress
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

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PJ Nicholoff Steroid Protocol

Background/Assessment

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