

# Care Guidelines

**End Duchenne Tour  
September 14, 2019**

# Neuromuscular Care

- ▶ There are recommended standards of neuromuscular care.
- ▶ **Neuromuscular specialist (NMS)** - should be seen every **six** months so they can assess your muscle strength, function, and flexibility to keep you as active as possible.
- ▶ Exercise and activity are important. Ask your physical therapist and neuromuscular specialist which exercises and activities are safe for you.
- ▶ Stretching and wearing night braces help muscles stay flexible.
- ▶ Steroids are known to help slow down muscle weakness in Duchenne.
- ▶ It is important for you to tell your team how your doing so they can offer you the right treatment and help you stay as active and independent as possible.

# Neuromuscular Care

- ▶ Though the muscle weakness in Duchenne follows a predictable path, every person is different.
- ▶ It is important to pay special attention to the groups of muscles responsible for breathing, eating, and swallowing as well. The heart is a muscle so regular monitoring of heart function is very important.
- ▶ Corticosteroids are used to help maintain muscle strength for as long as possible. They are different from the anabolic steroids that are sometimes misused by athletes who want to become stronger. Examples of steroids used to treat Duchenne are prednisone and deflazacort (Calcort or Emflaza).

# Neuromuscular Care

- ▶ **Recommended Care**
- ▶ Many things can be. It is important for your team to know the following information, so they can help you stay as active and independent as possible:
- ▶ Strength
- ▶ Endurance (how long before you get tired out)
- ▶ Range of motion (how well you can move your joints)
- ▶ **Safe exercise**
- ▶ Exercising can help keep you healthy and active. However, many types of weight-bearing exercise can be harmful to your muscles and cause more damage. For example, swimming is a non-weight bearing form of exercise and is generally safe.
- ▶ Use of proper technology, bracing, mobility assistance can significantly improve the quality of life every day
- ▶ Many new therapies are on the horizon which will change the natural course of DMD

# D

## DIAGNOSIS

- If developmental delay or elevated liver enzymes, do a creatine kinase (CK) (ChildMuscleWeakness.org)
- If male patients have a high CK (CK>800), order full genetic testing for Duchenne Muscular Dystrophy
- Discuss carrier testing/reproductive options for mother and testing for other family members

# U

## UNDERSTAND BREATHING PROBLEMS

- Pulmonary function test at least every year starting as young as possible
- Discuss cough assist when cough peak flow is < 270 liters per minute or if cough becomes weaker (use during respiratory illnesses while ambulatory and daily and as needed after loss of ambulation)
- Discuss nighttime Bi-PAP as needed or when forced vital capacity (FVC) < 30%
- Keep immunizations (including pneumonia and annual flu) up to date
- Treat respiratory infections promptly and aggressively
- Do NOT give supplemental oxygen without monitoring CO2

# C

## CORTICOSTEROIDS

- Start early!
- Discuss the benefits and possible side effects of corticosteroids by age 3 years, or as young as possible
- Evaluate efficacy and manage side effects of corticosteroids at each neuromuscular visit
- Discuss the rationale for lifelong steroid management
- Never stop taking steroids abruptly
- Discuss the need for stress dosing of steroids for illnesses or surgeries

# H

## HEART

- Cardiology visit with imaging (cardiac MRI preferred; echocardiogram if cardiac MRI not available) every year from diagnosis or more often if needed
- Discuss cardiac medications if fibrosis is seen on cardiac MRI, for any decrease in cardiac function decreases from baseline, or for heart failure (SF or shortening fraction <28% or ejection fraction <55%) or by age 10 even if findings are normal

# E

## ENDOCRINE

- If taking steroids, check 25-OH vitamin D prior to starting steroids, then annually
- Supplement vitamin D as needed
- Encourage sun exposure
- Nutrition discussions of adequate calcium and vitamin D intake
- Discuss measurement of bone density and use of bisphosphonates
- Assess spine for scoliosis at each visit
- Monitor for puberty starting at 9 years of age for need for testosterone therapy

**N****OCCUPATIONAL THERAPY**

and other assistive devices (beds, arm assistance, lifts, etc.) from the time of diagnosis

 Fill & Sign**N****NUTRITION &  
GASTROINTESTINAL**

■ Monitor weight ■ Assess/discuss diet (healthy eating, calcium, vitamin D) ■ Evaluate swallowing/need for intervention ■ Treat GERD and constipation as necessary ■ See your dentist every 6 months

**E****EMERGENCY**

■ Have patients/parents carry a copy of their last visit/note summary (including medications and neuromuscular contact information) and a Duchenne emergency card with them at all times ■ Use caution with all anesthesia; avoid inhaled anesthesia ■ Never use succinylcholine

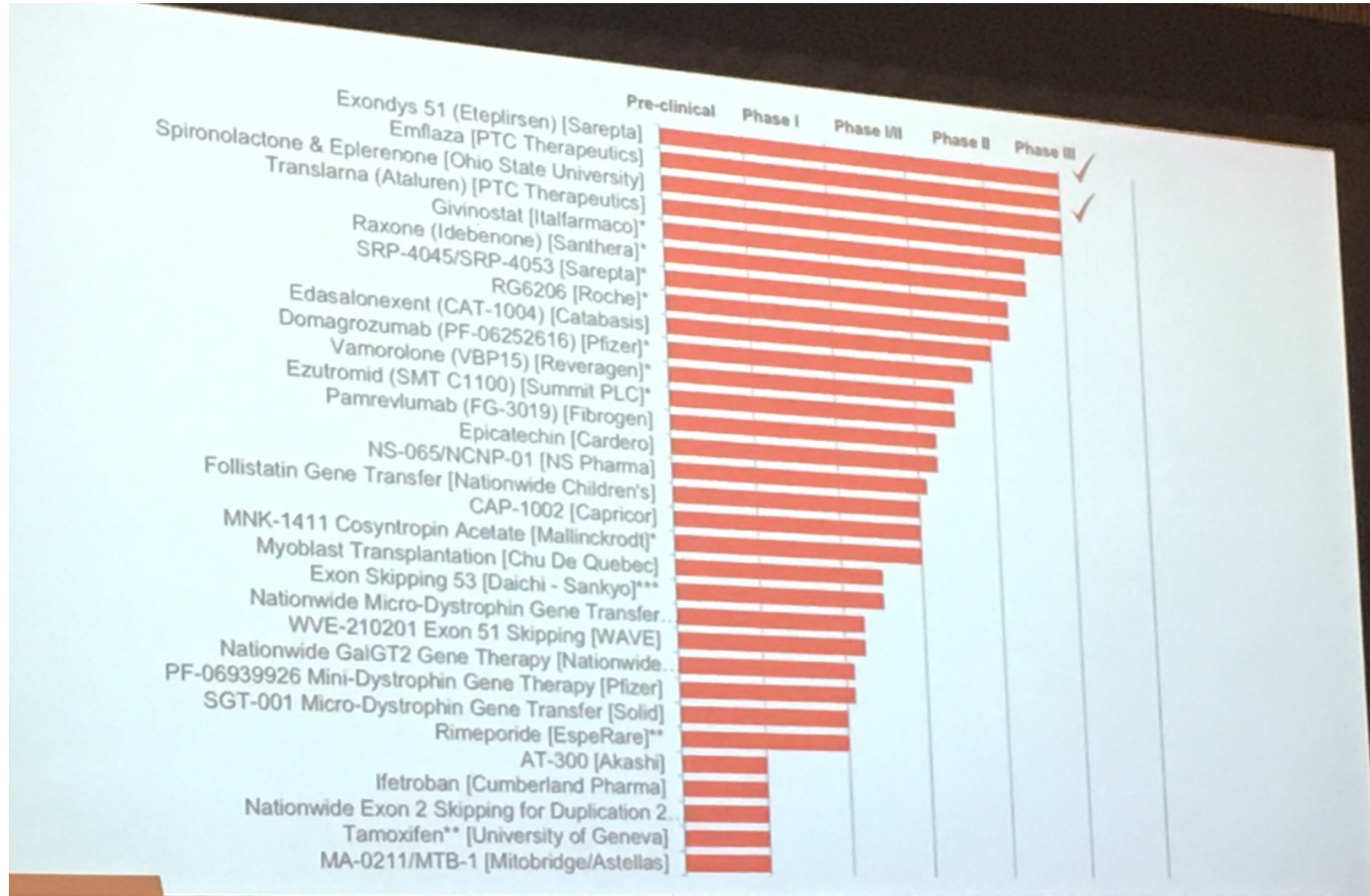
**M****MENTAL HEALTH**

■ Assess adjustment, coping, behavioral and emotional disorder and social isolation for the patient and family at each visit ■ Screen for learning disability (reading and math), language problems, attention deficit disorder (ADD), attention deficit and hyperactivity disorder (ADHD), autism and obsessive compulsive disorder (OCD)  
■ Neurocognitive evaluation done at diagnosis and prior to formal schooling; screening/management as needed  
■ Discuss the need for individualized/special educational plan

**D****DON'T DO IT ALONE**

■ Direct to trustworthy, reliable online resources ■ Organize follow up via a comprehensive neuromuscular center with expertise in caring for people living with Duchenne ■ Offer contact with organizations ([ParentProjectMD.org](http://ParentProjectMD.org), [TREAT-NMD.eu](http://TREAT-NMD.eu), [WorldDuchenne.org](http://WorldDuchenne.org))

# Clinical Trials and approved drugs



# Bragging rights....

- ▶ Some of the colleges young man from our clinic have gone on to attend and graduate from:
- ▶ University of Delaware, West Chester University, Princeton University, Villanova University, Delaware Technical Community College, King University, The College of New Jersey, Temple University, Edinboro University, Ursinus College and many more!



# Upcoming Events

- ▶ Please check PPMD site / Facebook page regularly for updates
- ▶ December 7<sup>th</sup> - Technology Day here at Nemours in conjunction with University of Delaware and Thomas Jefferson University
- ▶ PPMD National Conference June 2020 in Phoenix, Arizona

# THANK YOU!

- ▶ To everyone who made this day possible!