Duchenne Muscular Dystrophy:

Corticosteroid Treatment

PPMD Annual Conference

CONNECT

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Why Corticosteroids in DMD?

Prednisone shown to change the course of DMD
- reduced weakness
- preserved motor function

but ..... taken daily is best and
- side effects common
Corticosteroids in DMD

References:

- Manzur AY. et.al. Glucocorticoid corticosteroids for Duchenne Muscular Dystrophy. The Cochrane Library, 2004
- Bushby K. et.al. Consensus statement on the current role of glucocorticoid corticosteroids in Duchenne Muscular Dystrophy. UK North Star Network. Available at the ENMC.org website.
Summary:
- Corticosteroids help muscle strength
- Prednisone and deflazacort both work
- They slow the progression
- Start when boys are walking
- Best to give every day, once a day
- Balanced discussion of benefits and risks
- More studies are needed
More studies are needed

More than 10 different protocols

- 10 days on:10 off, high weekend dose, every other day, 20 days on:10 off, etc.

Yes..

- fewer side effects with weight, height, and behaviour

But..

- 10-15 year comparison with daily steroids needed
What are the benefits?

Short-term

- some, but not all have increased energy
- Long-term, 10-20 years
  - walking
  - breathing
  - heart
  - spine
  - arm strength
What are the Risks?

Variable and can include:

- weight gain
- behaviour
- Shorter….
What are the Risks?
What are the Risks?
What are the Risks?

Variable and can include:

- weight gain
- behaviour
- shorter
- bone health
- cataracts
- blood pressure, sugar in the urine,
- and more....
Time to Start

Arbitrary, 4 – 6 years of age

When parents tell us:

➢ stairs are more difficult
➢ falling more
➢ difficulty getting up from floor
➢ they are ready
Patterns of Weight Gain

Thin

- less than 25\textsuperscript{th} centile
- small muscles
- lordosis, sway back
- eat anything, anytime
- often tolerate prednisone
Patterns of weight gain

Heavy

- eating habits vary from a bird to an elephant
- rapid weight gain after 5 years
- off the chart by 10-12 years
- genetics might play a role
- very, very difficult to control
- steroids often get “blamed”
In between

- average weight around 4-6 years
- increases as mobility changes
- more than the 75th centile by 8-10 years
- diet can help, but…
- life style change for whole family
Preserved Muscle Function
Walking

With corticosteroids

- walk 3-5 years longer
Preserved Pulmonary Function
Pulmonary Function

At 18 years, boys on steroids should have:

- a better cough
- fewer admissions to hospital for pneumonia
- no increased lung infection

and very few should need nocturnal ventilation
Delayed Scoliosis
Scoliosis Surgery in DMD

Steroid treated:

- 10% of boys, compared to 90% not treated, done at 15-17 years
- can self feed
Preserved Cardiac Function
### Cardiac Function

<table>
<thead>
<tr>
<th></th>
<th>Control</th>
<th>Deflazacort</th>
<th>( P )</th>
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</thead>
<tbody>
<tr>
<td><strong>Age</strong></td>
<td>16 ± 2</td>
<td>14 ± 2</td>
<td>NS</td>
</tr>
<tr>
<td><strong>LVEF (&lt;45%)</strong></td>
<td>58%</td>
<td>5%</td>
<td>0.001</td>
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<tr>
<td><strong>% fractional shortening</strong></td>
<td>21 ± 8</td>
<td>33 ± 7</td>
<td>0.002</td>
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What about his bones?
What makes bones healthy?

- Genetics
- Physical activity
- Other things: calcium, vitamin D etc
What about my son’s bones?

- Even young have reduced bone density
- Worse with reduced walking
- ~25% have long bone fractures
- Vertebral (back bone) fractures don’t occur
More questions than answers

Most boys have osteoporosis

What will predict his risk of fractures?
Possibly worse with steroids

Risk of fractures, controversial
- long bones
- vertebrae

Can be treated, if indicated

More research needed
Can we start later?

Yes but…..

- Long-term benefits are not known
- Pulmonary function might benefit
- Heart? Spine?
- Research study underway
Can we stop the steroids?

Yes but….

- think twice
- wean slowly with physician guidance
- benefits lost rapidly and not regained if you restart steroids
Conclusions

Benefits of should outweigh side effects

Major benefits of preserved:

- walking
- pulmonary function
- cardiac function
- straight spine
- arm and hand function
Conclusions

Side effects:
- weight
- height
- cataracts
- osteoporosis
- delayed puberty
Conclusions

- hypertension
- diabetes
- renal stones
- liver problems
- increased vertebral fractures
Risk

Benefit
Getting the best for our boys by staying CONNECTED

Thank You!

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