

# Update Of Endocrine & Bone Standards Of Care For DMD



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**Duchenne UK Information Day,  
Newcastle, 22<sup>nd</sup> September 2018**

# SeCondary Osteoporosis & its Therapy Duchenne Musclare Dystrophy

## SCOT-DMD

**Muscular  
Dystrophy UK**  
Fighting muscle-wasting conditions



**CHIEF  
SCIENTIST  
OFFICE**



**Scottish Muscle Network**



## THE DIAGNOSIS AND MANAGEMENT OF DUCHENNE MUSCULAR DYSTROPHY

# A GUIDE FOR FAMILIES

UNITED KINGDOM



Adrenal suppression and crisis are a potentially life threatening complications of long-term steroid use (See Figure 5). It is important to know that you may be at risk of an adrenal crisis if your steroids are stopped suddenly or if doses are missed because of illness or other reasons for more than 24 hours. All patients taking chronic daily steroids should have a plan in place that outlines what to do in case of missed doses or during times of major illness or severe trauma, when extra doses, or "stress doses" of steroids may be needed. Information about preventing, recognising, and managing adrenal insufficiency, as well as when and how to use stress doses of steroids, are included in the PT Nuffield steroid Protocol, which can be found here: [www.psnuffieldtrust.nhs.uk](https://www.psnuffieldtrust.nhs.uk)

### SYMPTOMS OF AN ADRENAL CRISIS

You should receive information about the signs and symptoms of an adrenal crisis:

- Severe fatigue
- Headache
- Nausea/vomiting
- Low blood sugar
- Low blood pressure
- Passing out

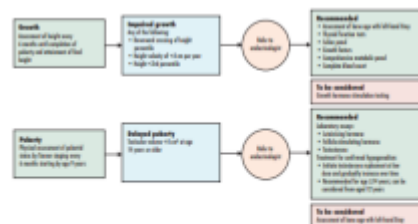


Figure 5. Assessment and Management of Growth and Puberty while taking Corticosteroids

## 8. BONE HEALTH (OSTEOPOROSIS) MANAGEMENT (Figure 6)

Bone health is important in both the ambulatory and non-ambulatory phases of Duchenne. People living with Duchenne at all ages have weak bones, especially if they are taking steroids. Steroids cause bones to have a lower bone mineral density, increasing the risk of fractures. Broken bones compared to the general population. Muscle weakness and decreased mobility are also risk factors for weak bones.

Dual energy X-ray absorption (DEXA) is a non-invasive test that measures bone mineral density of the long bones (usually the leg or arm). Thinner bone is less healthy and is more susceptible to fractures. Measuring your bone mineral density is important in monitoring overall bone health. It is recommended you have DEXA scans at least every 2 to 3 years.

People living with Duchenne, especially those taking steroids, are at risk for vertebral compression fractures. Vertebral compression fractures occur when vertebrae (the bones of the spine) sustain small fractures, causing them to become misshapen and to collapse on one another. These fractures, and resultant vertebral collapse, can cause pain. Vertebral compression fractures can be seen on a lateral (side view) spinal X-ray, even without having back pain. Vertebral fractures may be treated with bisphosphonates, especially if pain is present. It is recommended you have lateral spine X-rays every 1 to 2 years or more frequently if you experience back pain.



TREAT-NMD website

Duchenne UK social media

# IMPLEMENTING THE LATEST STANDARDS OF CARE GLOBALLY



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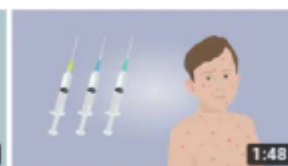
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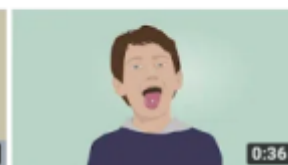
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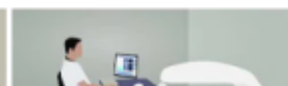
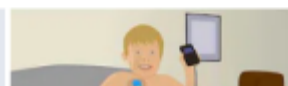
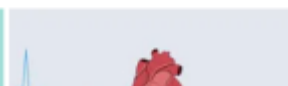
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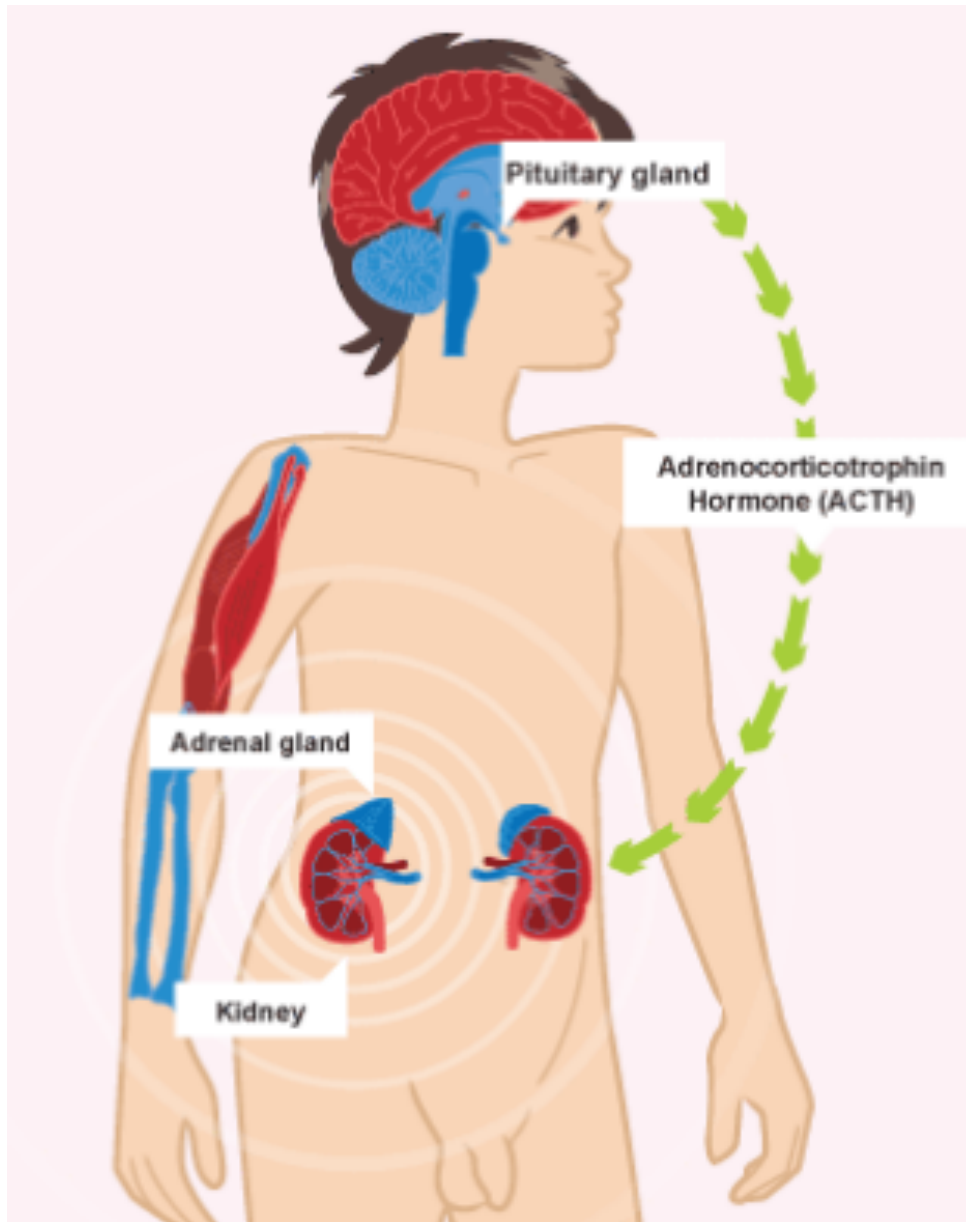
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# **ADRENAL SUPPRESSION FROM LONG TERM USE OF STEROID**



# Steroid & Adrenal Glands



The pituitary gland the master regulator of hormone control in the body

Adrenal glands make steroid hormones

Steroid medicines (eg Prednisolone or Deflazacort) are much higher than the amount produced by the body

This leads to switching off of the master regulator (ie ACTH)

Information about

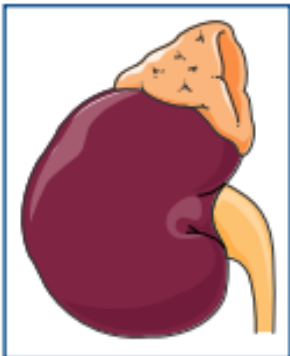
## Adrenal Suppression from Long Term Use of Steroid in Duchenne Muscular Dystrophy (DMD)

All patients who take steroids for longer than 6 months have adrenal suppression (sleepy adrenal glands)

**Access to hydrocortisone as injections to be administered at home during severe illness (when unable to take steroids by mouth)**

### Intramuscular injection video

Here you will see a video demonstrating an intramuscular injection of hydrocortisone

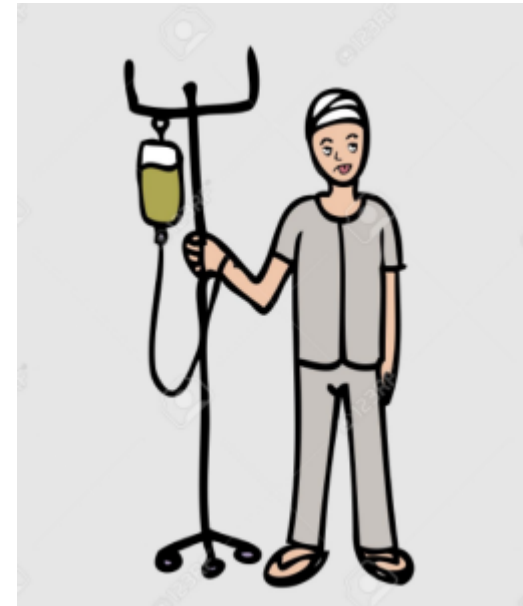
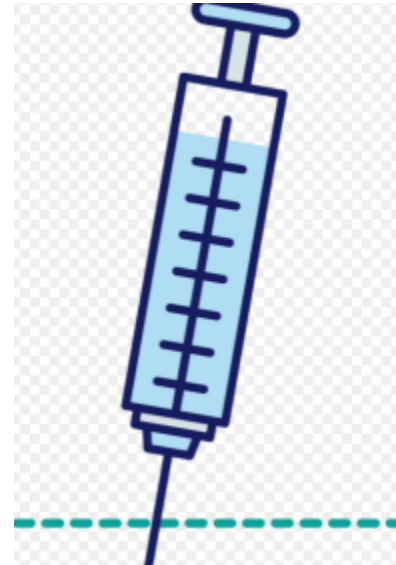
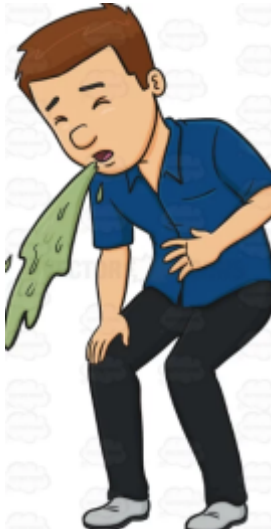


<http://www.smn.scot.nhs.uk/patients-and-families/dmd/>

Have emergency plans in place (steroid card, hospital alerts etc)

If serious vomiting illness or unable to tolerate oral steroids (eg major operations)

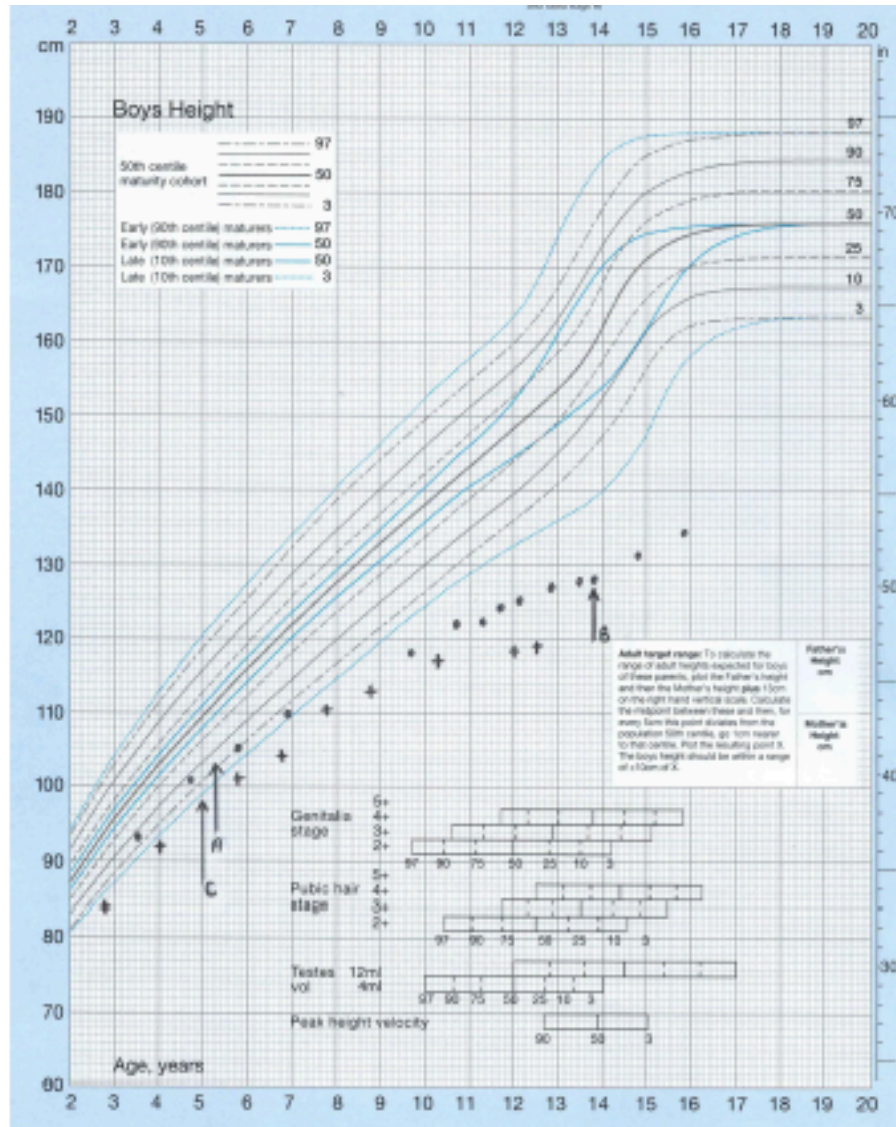
- Need plan for extra steroid to be given either as an injection or as a drip







# GROWTH ISSUES

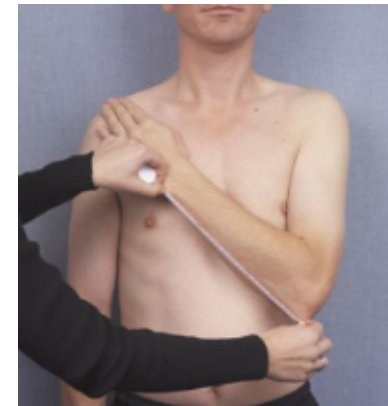


About 25% of boys with DMD are short before starting steroids.

Steroid cause growth failure

Need height measurement for interpretation of body mass index, lung function, blood pressure etc

**Height monitoring 6 monthly**



# Growth hormone treatment

Not recommended routinely

Not licenced indication

May improve growth rate but remain short

Daily injections

Side effects





# PUBERTY

Information about

## Puberty and Hormones in Duchenne Muscular Dystrophy (DMD)

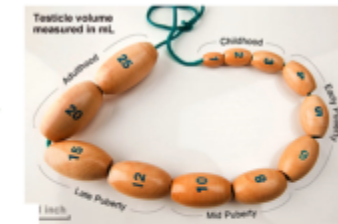


Almost all boys on steroid treatment (especially daily steroid) will not show signs of puberty

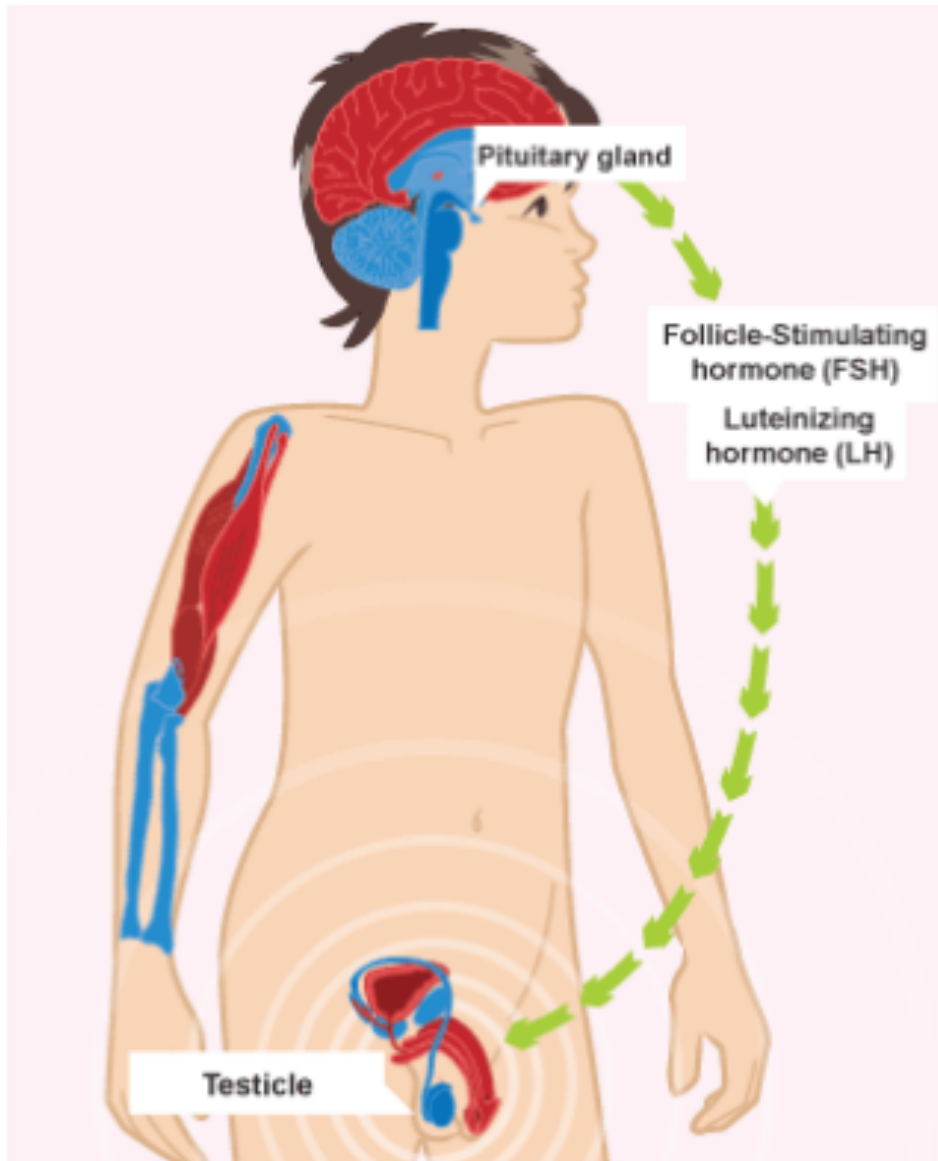
- Important for psychological well-being
- Bone/muscle effect

**Examination for puberty 6 monthly from age 9 years**

**Have to be examined from age 13 years**







Steroid medicine like Prednisolone and Deflazacort turn off the hormone switch that starts puberty.

LH and FSH leads to testicles size to increase

Testosterone leads to hair and genital development



Injections

Creams/gel

Tablets

If no signs of puberty by 14 years, can consider treatment

# **BONE HEALTH/OSTEOPOROSIS**



## **Fractures are very common**

- At least 50% of boys with DMD will have a fracture
- Compression fracture of the back common and underestimated if diagnosed based just on back pain

**Spine x-rays: Baseline then 1-2 yearly (steroid); 2-3 yearly not on steroid**

**Vitamin D levels: Baseline then annually**

**DXA (bone density scan): Baseline then annually**

Prioritise spine x-ray above DXA scan

- Bone density results should not be used as a guide to start bone protective medicine
- Presence of vertebral fracture is indication for consideration of bone protective medicine



# Vertebral fractures in boys with DMD



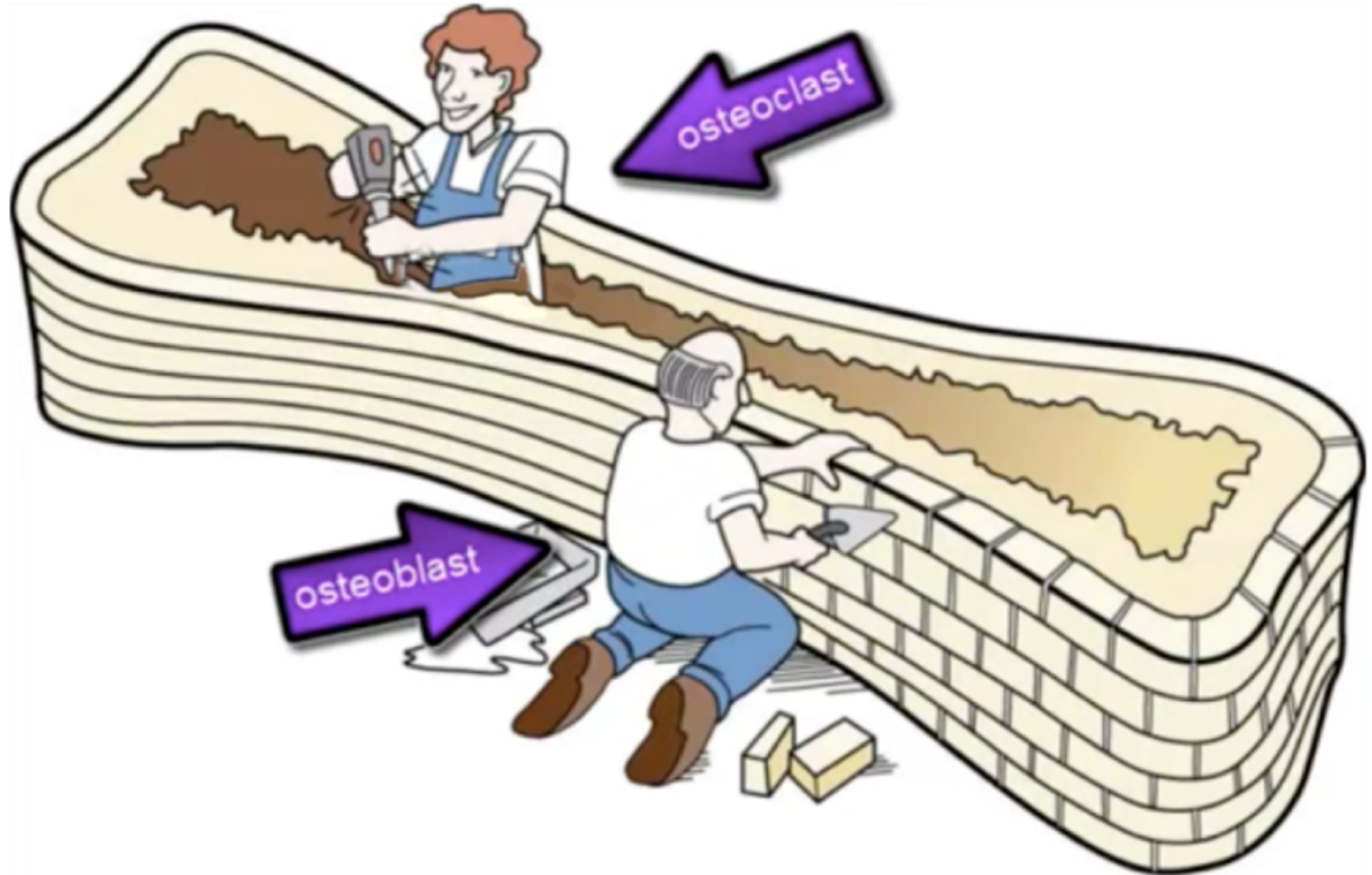
Normal spine



Mild vertebral fractures



Multiple severe  
vertebral fractures



Useful in post-menopausal osteoporosis as problem is due to increase breakdown of bone

In steroid treated patients like DMD most likely due to reduction in amount of bone formed

Can be given as tablets  
- Indigestion



Can be given as medicines into drip



No good published evidence of the effectiveness of bisphosphonates in DMD

Probably increases bone density but unclear if prevent fracture.

Very good for bone pain

No evidence for preventative use of bisphosphonate

# Who should get bisphosphonates?

No evidence of its use as a preventative medicine (and concern about possible side effects)

## Indication are:

Painful vertebral fractures

Moderate or severe vertebral fractures (even without pain)

MILD: Genant 1



MODERATE: Genant 2



SEVERE: Genant 3



Other situations to discuss on a case by case basis carefully

- Lots of long limb fractures







**Interventions to prevent and treat corticosteroid-induced osteoporosis and prevent osteoporotic fractures in Duchenne muscular dystrophy (Review)**

Bell JM, Shields MD, Watters J, Hamilton A, Beringer T, Elliott M, Quinlivan R, Tirupathi S, Blackwood B

No good quality studies to recommend treatment of osteoporosis in DMD  
- Trials urgently needed

EUROPEAN  
NEURO  
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1 - 3 June 2018

**Workshop nr. 236**

**Bone protective therapy in Duchenne Muscular Dystrophy:** Determining the feasibility and standards of clinical trials (BONE). Organisers: Prof Volker Straub, Dr. Jarod Wong, Prof Leanne Ward and Dr. Ros Quinlivan.



## Shuko Joseph

CSO Academic Fellowship with AD and MD-UK

“SeCondary Osteoporosis & Its Therapy In Duchenne Muscular Dystrophy”



Glasgow/Leeds

## Claire Wood

MRC Academic Fellowship with MD-UK

“Characterisation of skeletal development and the use of anabolic agents in murine models of DMD”



Edinburgh/Glasgow/Newcastle



“Unfortunately the care standards of boys with DMD can vary quite substantially. That’s why it’s important to read these new guidelines, which will give you the information you need to fight for the best care for your child at your medical Centre. The information is based on what clinicians and physiotherapists have known and developed over the years. Some advice, for instance on cardiac care, may not necessarily be known by the cardiac team looking after your son, so it’s important to have the right information so that you can ask the right questions when you go to your clinic appointments.”

Emily Crossley, Co-founder and Co-CEO of Duchenne UK



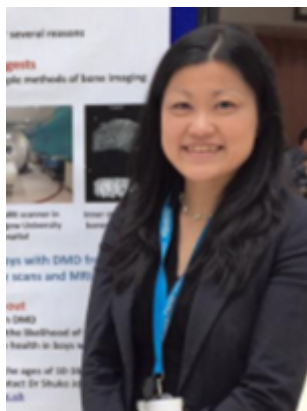
"If there is only one guide you ever read about Duchenne it should be this. It will allow you to arm yourself with all the information you need to make sure you get the best care for your child. You don't have to read it all in one go. Take your time, read a section at a time. Highlight points and make notes of anything you don't understand or concerns you that your child isn't getting access to. Reach out to your doctor, care advisor or us to discuss the guide and get any additional information you need."

Alex Johnson, Co-founder and Co-CEO of Duchenne UK





# Acknowledgement



S Joseph



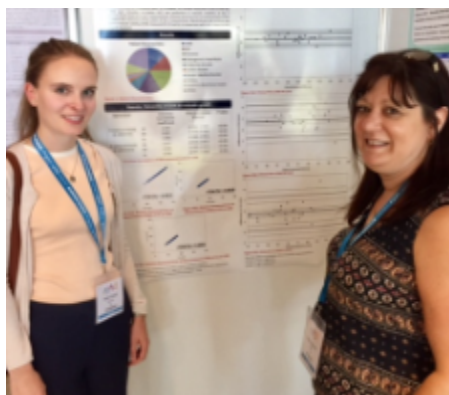
J Dunne



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Research radiographers & MRI Physics



F Ahmed





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