On the 15th June 2017, 92 delegates convened in Glasgow for a unique one-day symposium organised by the Developmental Endocrinology Research Group, University Glasgow (with support from Action Duchenne and Muscular Dystrophy UK) to discuss osteoporosis in boys with Duchenne Muscular Dystrophy (DMD). The symposium brought specialists and scientists from neuromuscular and bone backgrounds together to discuss recent understandings of bone health in DMD and the path forward.

As Dr Jarod Wong, organiser of the symposium, stated: “Fractures are extremely common in boys with DMD and can lead to devastating outcomes, including early loss of ambulation”. “Whilst there is still little robust evidence on how we should treat osteoporosis in DMD, we have learnt a lot more in recent years”, Dr Wong added. “The support of Muscular Dystrophy UK and Action Duchene in the improvement of health care of these boys, including bone health, is extremely important. We are very grateful to both groups and the Chief Scientist Office Scotland for funding Dr Shuko Joseph who is conducting a series of important research studies on bone health in boys with DMD in Scotland (email jarod.wong@glasgow.ac.uk for further information). The research will provide important results relating to the understanding of osteoporosis in DMD. It will consolidate the position of Glasgow as a research centre of excellence for neuromuscular conditions, especially in bone health and endocrine issues”.

HIGHLIGHTS FROM SPEAKERS’ TALKS

**Glucocorticoid in DMD: What is known and what remains unresolved?**

Dr Anna Sarkozy (Dubowitz Neuromuscular Centre, London)

1. The optimal steroid regimen in DMD is still unclear but outcome may be better in those who are treated with a higher dose and start treatment earlier.
2. A very small percentage (about 2% of boys with DMD) do not respond to steroid.
3. Side effect profile may be different with different types of steroid but we may need to learn more about predicting steroid response and side effects in DMD.

**Growth failure in chronic disease: Now and beyond**

Prof. Lars Säven Dahl (Karolinska Institute, Stockholm, Sweden)

1. Both steroid treatment and inflammation can lead to poor growth via their effects on the growth plate, which is located at the ends of long bones.
2. Proteins which lead to increased programmed cell death are increased in DMD and may contribute to poor growth.
3. New therapies targeting the growth hormone axis or other similar pathways may aid in rescuing bone growth in DMD.

**Muscle-bone interaction in health and disease**

Dr Sylvie Coupaud (University of Strathclyde, Glasgow)

1. Bone is closely related to muscle and its strength is related to the amount of muscle mass.
2. After immobile people after spinal cord injury, bones become much thinner but there is variability in pattern of bone loss. The pattern of bone loss in boys with DMD after loosing ambulation is still not fully clarified.
3. Low magnitude vibration therapy may be a method to improve muscle and bone health in people with immobility but its effect are not clear yet.

**Novel methods of imaging bone in young people**

Prof. Faisal Ahmed (Developmental Endocrinology Research Group, University of Glasgow)

1. Bone density from DXA does not seem to predict fractures in young people with chronic condition.
2. Modern generation DXA may be able to replace spine x-rays to detect spinal compression fractures.
3. There are many other newer ways of looking at detailed information of shape and the inner structure of bone including MRI and these may help us to determine the type of bone treatment to use in the future.

**Novel methods of assessment of muscle in neuromuscular conditions**

Prof. Volker Straub, (John Walton Neuromuscular Research Centre, Newcastle)

1. Clinical assessment of muscle function may not be reliable and repeatable.
2. Muscle MRI gives very useful information on disease progression in DMD including muscle volume, muscle inflammation and the amount of fat in the muscle.
3. It is very important to have reliable but also clinically important outcome measures to use in clinical trials in boys with DMD.
Osteoporosis in DMD: Mechanisms, clinical manifestation and the path forward  
Prof. Leanne Ward (Children’s Hospital East Ontario, Ottawa, Canada)  
1- Studies using bone biopsy in children with DMD show that these boys only have about 20% of bone volume of healthy boys and only about 50% of the amount of bone formation.  
2- Spine compression fractures are very common in DMD and many may be present without back pain.  
3- Routine screening with x-rays for spine compression fractures in DMD should now be recommended, and any evidence of moderate to severe spine compression fractures should lead to consideration of bone protective therapies.

An appraisal of therapies to improve bone health in DMD  
Prof. Margaret Zacharin (Royal Children’s Hospital, Melbourne, Australia)  
1- There are very few studies that investigate therapies to prevent the first fracture in boys with DMD or therapies to treat osteoporosis after first fracture.  
2- There is insufficient evidence from the four small published studies of bisphosphonate in DMD to recommend routine use as preventative therapy but results from a multi-centre trial maybe soon available.  
3- Addressing delayed puberty is very important to improve bone development and should be given more attention.

DMD: Activity and stretching in the ambulant child  
Ms Marina Di Marco (Scottish Muscle Network, Glasgow)  
1- Involving physiotherapists early in the management is important in the management of boys with DMD.  
2- Stretching is extremely important in DMD and can be carried out using a variety of methods including the use of active, passive and static techniques.  
3- Targeted exercises and ambulation aids may be used to improve posture, muscle strength and to maintain ambulation. These may also prevent the likelihood of falls.

Growth promoting therapies in children with chronic conditions  
Dr. Jarod Wong (Developmental Endocrinology Research Group, University of Glasgow)  
1- Growth and puberty are very closely related to bone development; therefore addressing them may be important in managing bone health in DMD.  
2- There is only limited evidence on the use of growth hormone in DMD and its use may be associated with side effects. In the clinical setting, growth hormone use in boys with DMD must be carefully considered.  
3- Delayed puberty is very common in DMD and low male hormone levels (testosterone) in adults with DMD may be common, necessitating long-term hormone treatment.

Novel and emerging therapies in neuromuscular conditions  
Dr. Michela Guglieri (John Walton Neuromuscular Research Centre, Newcastle)  
1- There are an increasing number of promising clinical trials for treatment of boys with DMD.  
2- Different drugs target different pathways which can lead to muscle problems in DMD.  
3- New therapies to modulate the steroid receptor may have the same efficacy as steroid medicines but have less/no side effects. Clinical trials in DMD are needed.

COMMENTS FROM SUPPORTERS OF THE SYMPOSIUM

Diana Ribeiro, Chief Executive Office of Action Duchenne: “Action Duchenne is delighted to see the progress of Dr. Shuko Joseph’s co-funded clinical research fellowship, in contributing to the clinical management of bone health for DMD. This was reflected in the important outcomes of the international bone health symposium co-funded with our partners Muscular Dystrophy UK and organised by the Office for Rare Condition in Glasgow. Collectively, we will play a pivotal role in progressing the clinical capability of Glasgow as an emerging research centre and management of bone health for the DMD community.

Dr Jenny Versnel, Director of Research and Business Innovation at Muscular Dystrophy UK: “This was an extremely informative symposium that brought together experts from several specialities to discuss the important topic of bone health in DMD. This builds on the work from Dr. Shuko Joseph’s clinical fellowship that we are funding together with Action Duchenne and the Chief Scientist Office in Scotland and will provide valuable contributions to the clinical management of bone health in DMD moving forwards.

The Developmental Endocrinology Research Group in Glasgow and the Scottish Muscle Network are extremely grateful for the support of both Muscular Dystrophy UK and Action Duchenne in helping make this important symposium happen.