



Muscular Dystrophy UK's Adult North Star Network

Care recommendations for adults with Duchenne – a consultation

Background:

The North Star Network was set up in 2003 to help drive improvements in services and set national standards of care for children living with Duchenne muscular dystrophy.

A national database was established in October 2006 to collect data from children with Duchenne muscular dystrophy in all the major paediatric neuromuscular centres in the UK. The database has helped improve clinical care and led to major breakthroughs such as the use of steroids and new and emerging treatments for children with Duchenne.

Muscular Dystrophy UK is now working with neuromuscular specialists that see adults living with Duchenne to extend the North Star network and ensure that the same level of support is provided in adult care.

Purpose:

The purpose of creating the Adult North Star Network is to build up a picture of the progression of Duchenne muscular dystrophy in adults living with the condition. This work aims to improve the care and support available as well as knowledge and data that can support future research trials.

The network's main goals are:

- Bringing together all specialist health professionals who have patients who are adults with Duchenne muscular dystrophy to share best-practice
- Creating the first natural history database for adults living with Duchenne to better track how their condition is progressing and the effect of treatments.
- Producing the first specific recommendations of care guidelines for adults with Duchenne

Care recommendations:

The Adult North Star Network is creating the first ever care recommendations for adults living with Duchenne muscular dystrophy. These guidelines follow NICE accredited recommendations of care that have already been created for [children living with Duchenne](#)¹.

To create the care recommendations, expert working groups have come together to design best practice recommendations on the delivery of care for adults with Duchenne.

The resulting guidelines, planned to be launched and published in 2018, will outline the key interventions and care that should be available to any young adult with Duchenne, and will be the first formal document created that will outline best-practice care for adults with Duchenne. We hope the document will be adopted by all neuromuscular services.

The care recommendations will include:

- Neurology
- Physiotherapy
- Transition
- Respiratory care
- Cardiac care
- Palliative care
- Psychosocial needs
- Care Advisor support
- Speech and Language
- Renal and bladder
- Anaesthetics
- Neuro-Gastroenterology

We want to hear your views and experiences in each area. Please fill in the below document and return to Lloyd Tingley at l.tingley@muscular dystrophyuk.org by the 5th of January 2018.

¹ http://www.treat-nmd.eu/downloads/file/standardsofcare/dmd/lancet/the_diagnosis_and_management_of_dmd_lancet_comp_lete_with_erratum.pdf

Guideline sub-sections:

1. Physiotherapy

Recent guidelines for physiotherapists working with children living with neuromuscular conditions were released by the Association of Paediatric Chartered Physiotherapists (APCP)². A specialist group of physiotherapists working with adults with Duchenne have decided to follow the same format; breaking down different areas of physiotherapy care and writing guidance on them. These areas include:

- Respiratory physiotherapy
- Exercise and fitness
- Upper limb support
- Wheelchair seating
- Night positioning
- Contracture management
- Orthotics
- Leisure and sport
- Pain

Example questions to consider:

- What are the most important areas of physiotherapy for adults with Duchenne, how can these areas be improved?
- How can a physiotherapist best support you to live a better quality of life?

² <http://apcp.csp.org.uk/news/2017/03/27/new-publication-apcp-neuromuscular-committee-guidance-paediatric-physiotherapi>

2. Neurology

A consultant neurologist is often the head of care for an adult with Duchenne muscular dystrophy and is key in monitoring any changes in a person's condition, as well as plugging into appropriate other health services. Guidance for consultant neurologist's involves ensuring that adults with Duchenne are seen a minimum of once a year by a neuromuscular centre to track important changes and talk through any concerns a person and their family may have.

Questions to consider:

- What are the key things that adults with Duchenne want to find out at their appointment with a consultant neurologist?
- What are the best and worst aspects of current care?

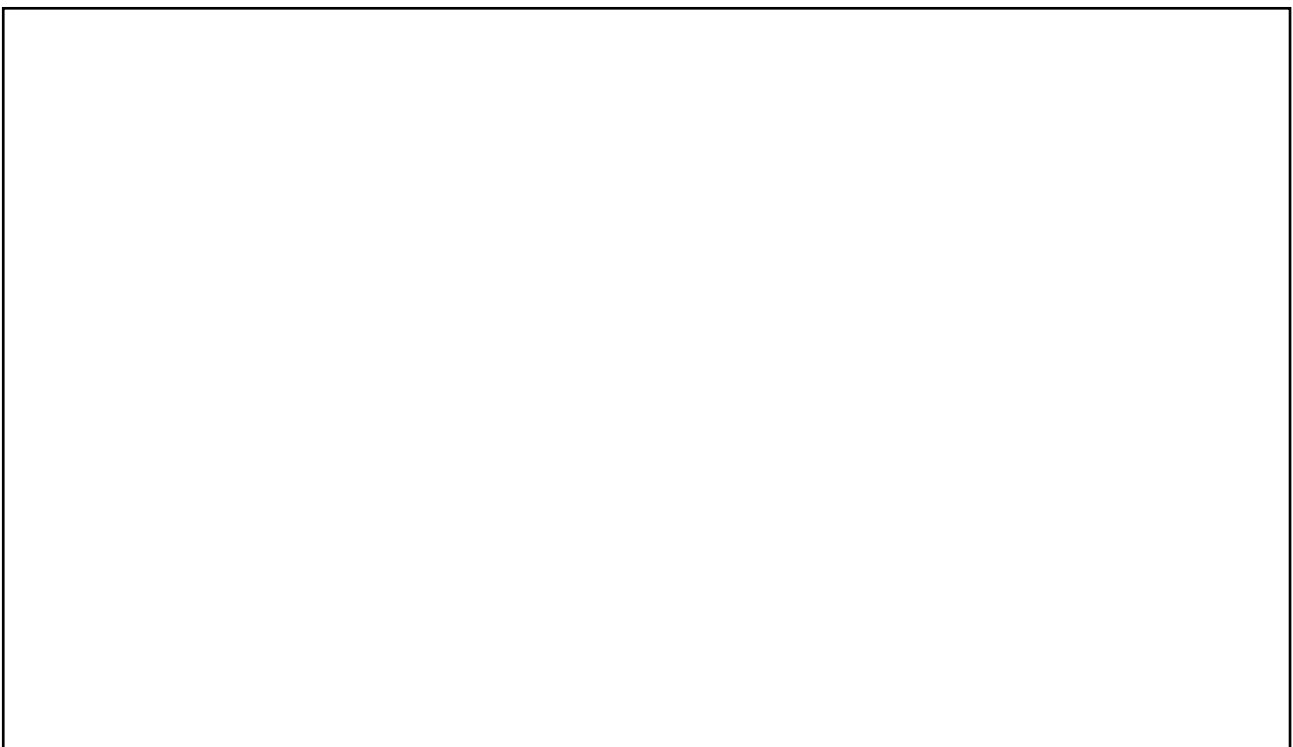
3. Transition

Transitioning from children's to adult neuromuscular services is an important time in the healthcare for people living with Duchenne. NHS England has already produced wide-ranging guidance which is non-specific to neuromuscular conditions on best-practice transition. This includes advice on special transition clinics and the person with the condition taking more control of their appointments.

Neuromuscular specific guidance focuses on these topics and ensuring people with Duchenne are prepared for changes in their care from paediatric to adult services with less physiotherapy input in particular.

Questions to consider:

- At what age should people with Duchenne be taking more control of their healthcare and appointments, and to what level?
- At what age is it correct to allow a person with Duchenne to have an appointment with a health professional without a parent/carer in the room?
- What advice would you give to someone with Duchenne throughout transition?



4. Respiratory care

Adults with Duchenne muscular dystrophy are living longer with the benefit of improved respiratory management; but are developing previously unseen complications. More patients are considering tracheostomy; saliva and secretion management is becoming more important.

There are a range of different Non Invasive Ventilation (NIV) options that respiratory specialists are able to support adults with. Guidance focuses on ensuring people with Duchenne have access to respiratory specialists whose role it is to prevent crises such as unplanned emergency admissions.

Questions to consider:

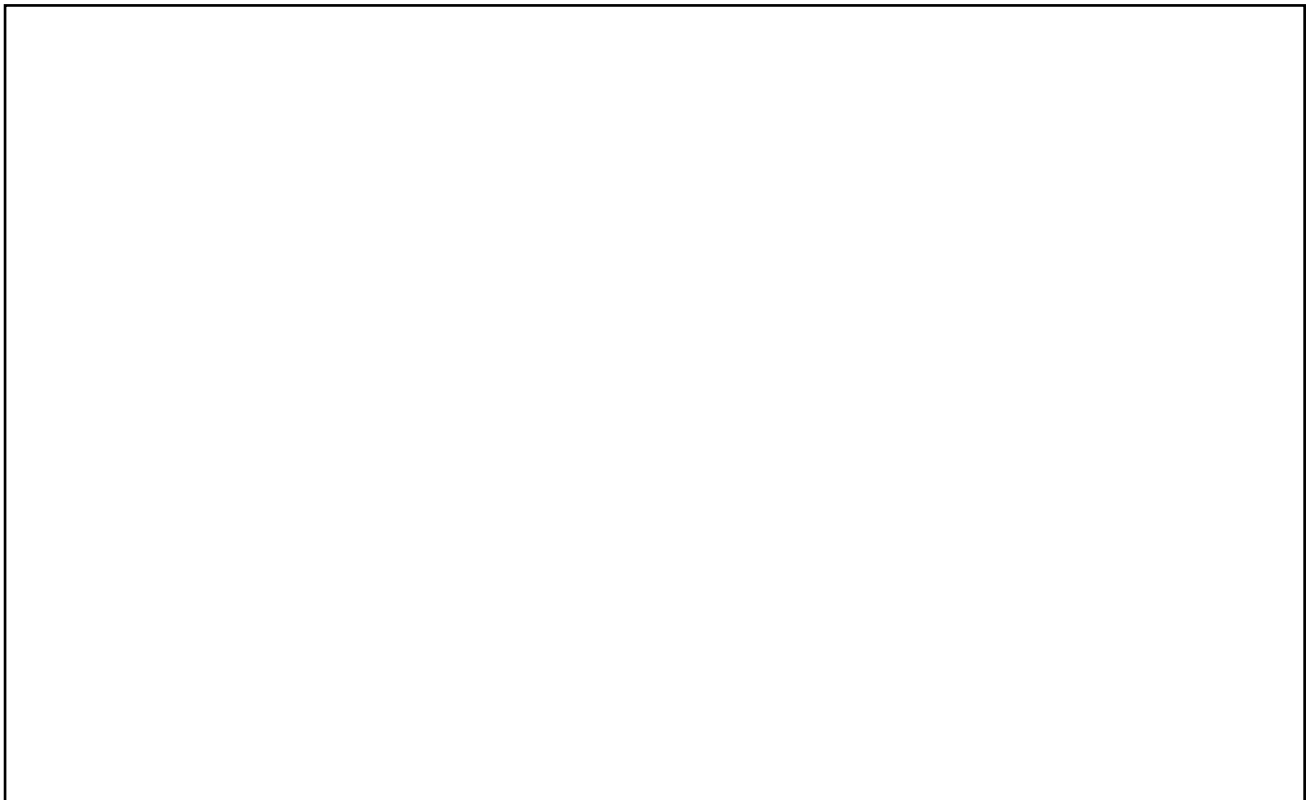
- What are the key areas in respiratory management that you feel would support adults with Duchenne to comfortably use equipment that will benefit them?
- What support is necessary to avoid unplanned emergency admissions because of respiratory implications?

5. Cardiac care

Heart imaging is recommended in children at diagnosis of Duchenne muscular dystrophy, every two years up to age 10 years and annually thereafter. Little research has been carried out on the most effective cardiac support to offer for adults living with Duchenne. However treatments such as Implantable Cardioverter-Defibrillators (ICDs) are being used more readily, but not routinely, in adults with Duchenne to prevent sudden arrhythmia deaths.

Questions to consider:

- How can we ensure that all adults with Duchenne have access to the correct cardiac input?
- What advice can health professionals be given on the thoughts of adults with Duchenne on devices such as pacemakers and ICDs



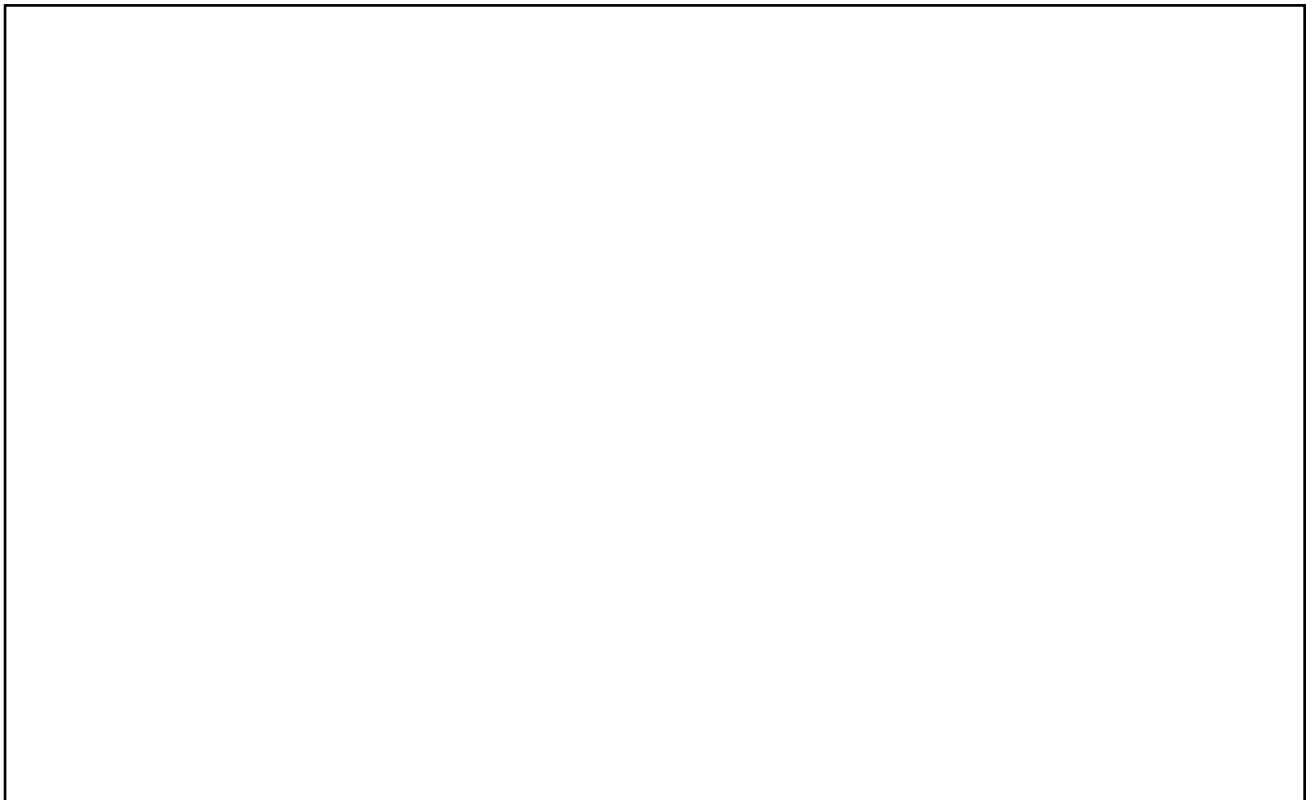
6. Anaesthetics

In general people with Duchenne have been safely anaesthetised but there are significant potential complications which are related to pre-existing cardiorespiratory disease and the choice of anaesthetic agent.

Although guidance for professionals is important in this challenging area of anaesthesia, in general the safest anaesthetic is usually that with which the operative anaesthetist is most familiar and rigid guidance about technique or drugs is not appropriate.

Questions to consider:

- What are the key things that any professional administering anaesthetics needs to know concerning an adult with Duchenne?
- What are the key discussions that should happen before any anaesthetic is administered to someone with Duchenne?



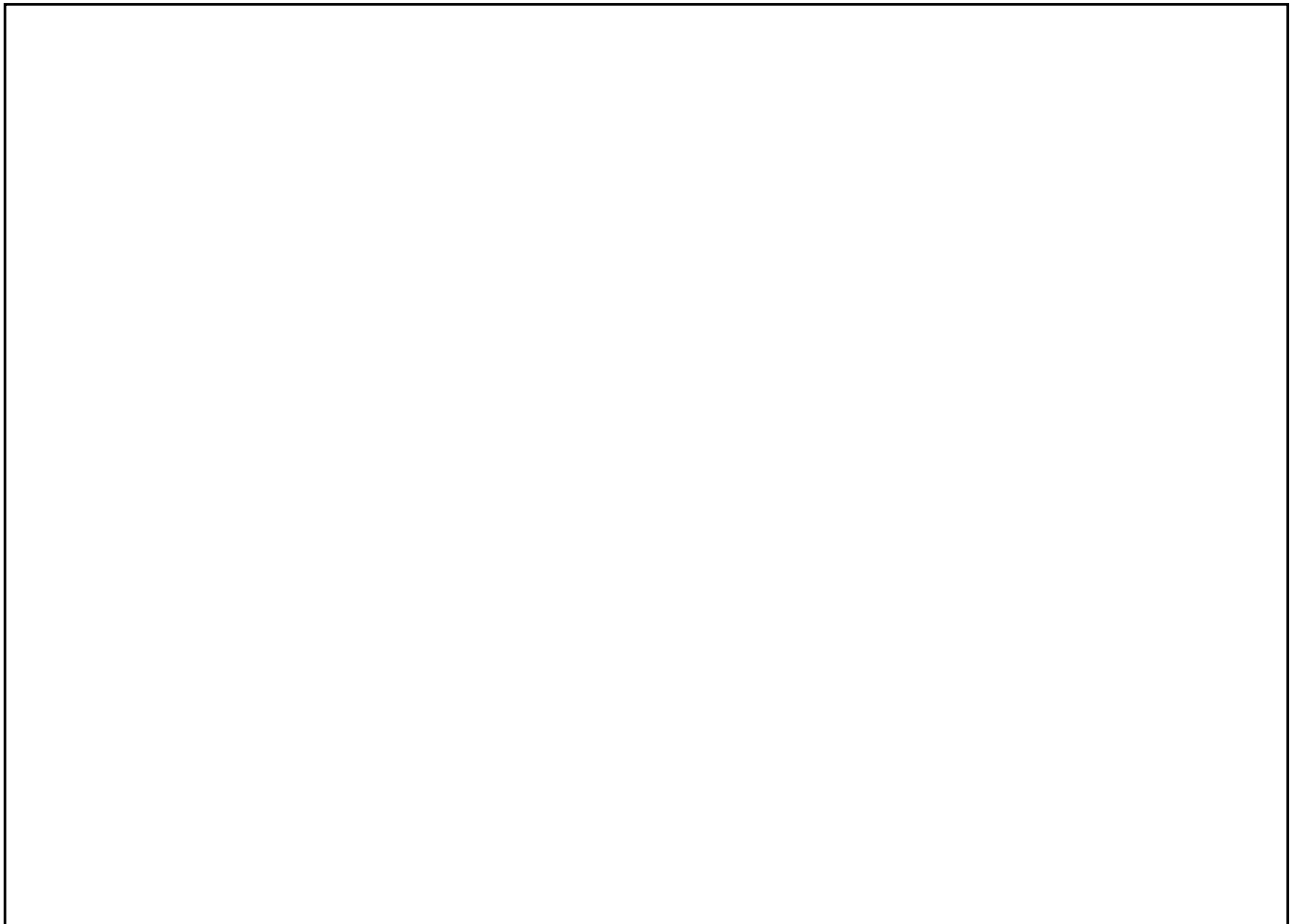
7. Psychosocial needs

There is a lack of psychological support available for adults living with Duchenne and people with muscle-wasting conditions within the NHS. This lack of support often means that adults with Duchenne are not able to access emotional support outside of their family and friends.

It is vital to ensure that all adults with Duchenne are able to access someone who has trained psychological expertise who can support them in managing their emotional wellbeing.

Questions to consider:

- What are the key areas of their emotional wellbeing that adults with Duchenne would like to be able to discuss with a trained mental health worker
- How often should adults with Duchenne be able to access a trained mental health worker



8. Palliative care

Recent research conducted by Professor David Abbott from Bristol University found that adults living with Duchenne would like to be given the opportunity to have palliative care discussions with their neuromuscular team and parents, but feel they are unable to broach these topics.

The research focused on the ideas that adults had on palliative care and living with a life-limiting condition, as well as advice for others who are diagnosed with Duchenne.

Guidance focuses on ensuring that adults with Duchenne are aware of their palliative care options and have a named palliative medicine specialist who they are able to raise any queries with.

Questions to consider:

- What support should be available within the NHS to allow adults with Duchenne to hold palliative care conversations with the correct health professional(s)
- What are the most important areas of palliative care that an adult with Duchenne should be able to discuss



9. Care Advisor support

A care advisor plays a vital role in ensuring that an adult with Duchenne is plugged into the correct local services such as physiotherapy, occupational therapy, hospices and support groups. A care advisor also supports on disability benefits and ensuring adults with Duchenne are accessing all of the support necessary.

There are now over 60 care advisors in the UK, with numbers growing massively in the last few years, allowing this support to be available more widely.

Questions to consider:

- What are the most important areas that a care advisor can support an adult with Duchenne with?
- What are the best methods for a care advisor to provide this support to adults with Duchenne?



10. Speech and language

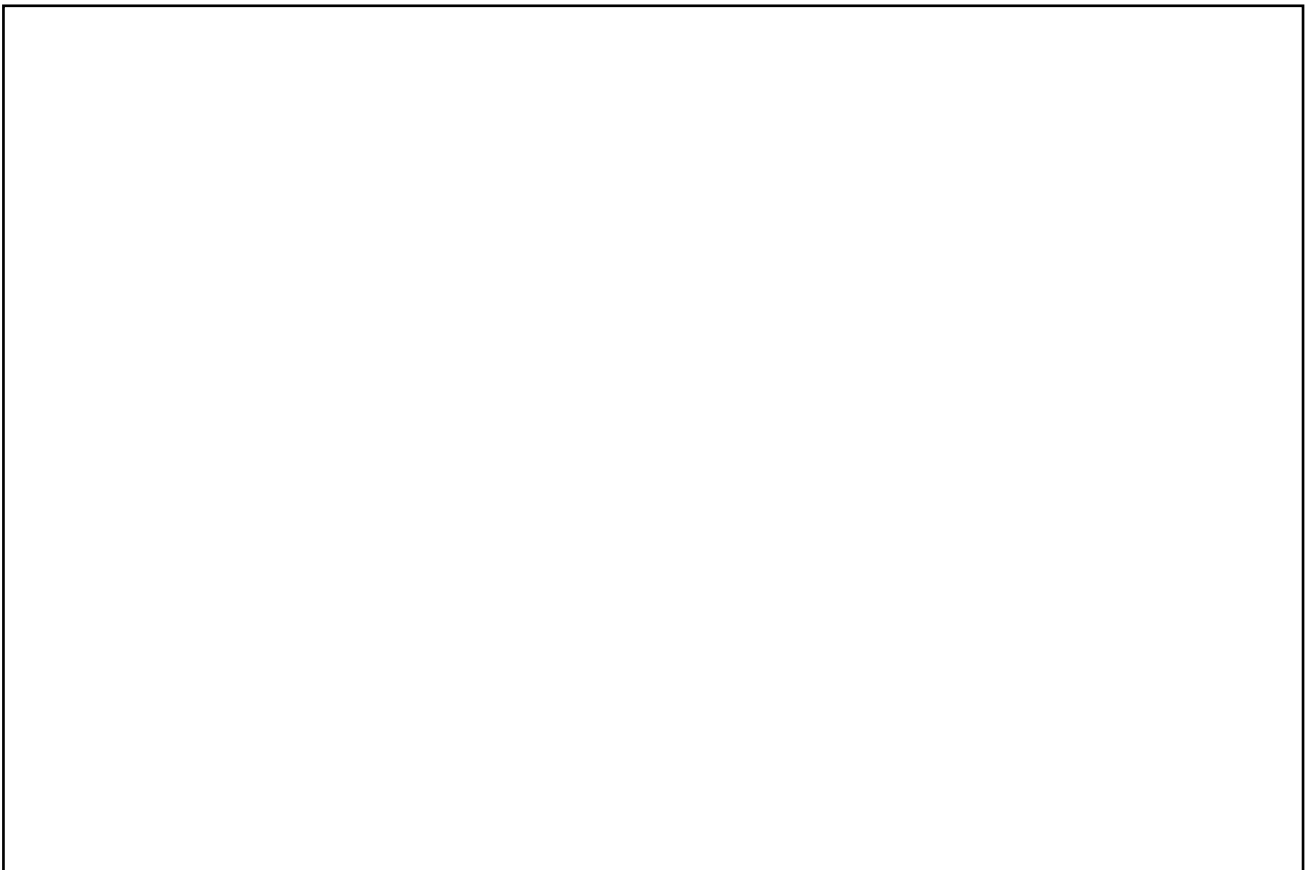
Increased lifespan of males born with Duchenne today has led to previously unidentified complications associated with swallowing, such as aspiration pneumonia, weight loss, malnutrition and dehydration.

Whilst repeatable assessment tools for orthopaedic, respiratory and cardiology monitoring have been identified, the most appropriate tool for swallowing has not yet been considered.

There are also few speech and language therapists who specialise in neuromuscular conditions and can provide the appropriate level of advice and support across the UK.

Questions to consider:

- What are the key areas surrounding speech and language that you would like to discuss with a speech and language therapist given the opportunity?
- What are the main issues that adults with Duchenne face with speech and language?

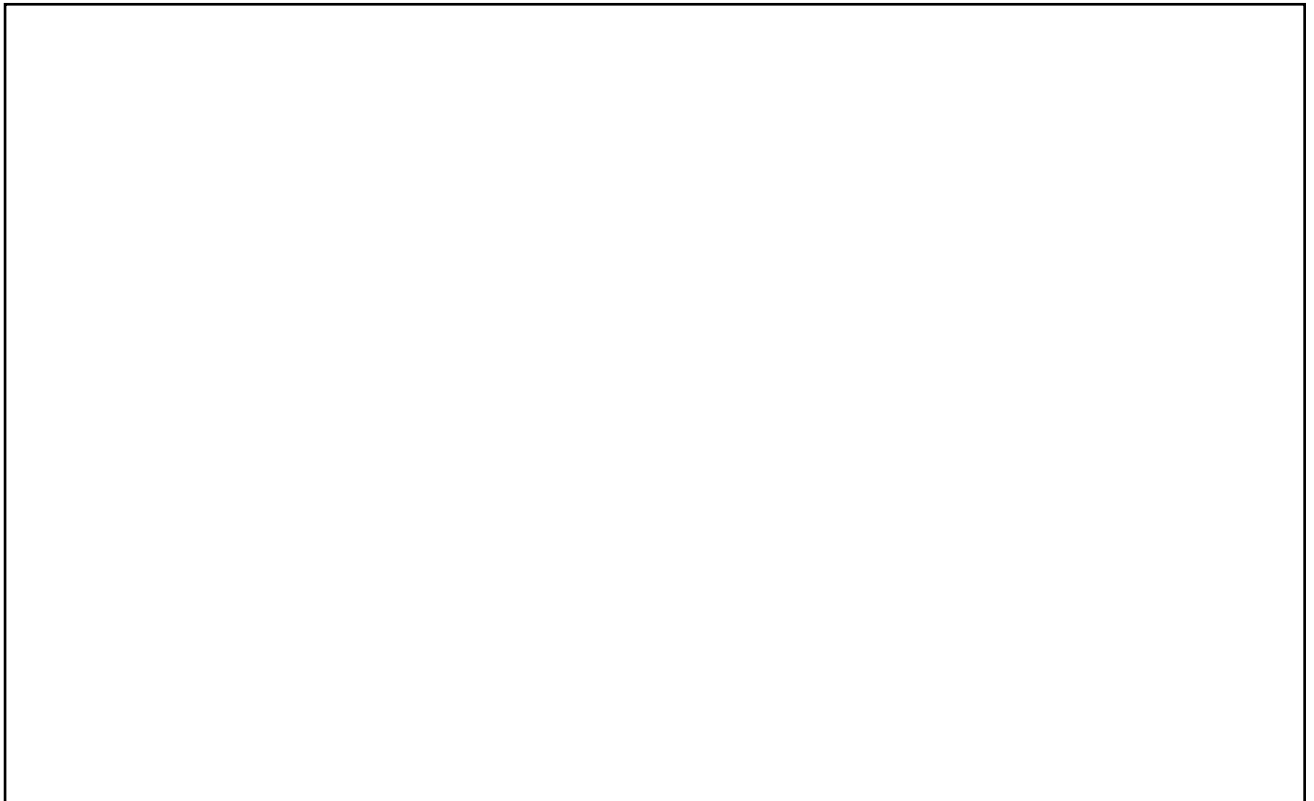


11. Renal and bladder

Renal management is vital for adults with Duchenne as potential complications can lead to progressive cardiac failure and poor outcomes if not monitored for adults with Duchenne. Accurate monitoring of renal function is difficult within adults living with Duchenne however guidance shows that it is vital to ensure unwanted complications are prevented via early screening.

Questions to consider:

- What are the most prevalent renal and bladder issues that affect adults with Duchenne?
- How can we ensure that adults with Duchenne are more aware of potential renal and bladder issues and how they may be prevented?



12. Gastroenterology

Management of the gut is an important area as people with Duchenne grow older. A Gastroenterologist is an important health professional in ensuring that adults with Duchenne muscular dystrophy are given advice on nutrition and monitor potential bowel problems.

A particular issue for adults living with Duchenne that research has been conducted in is life-threatening constipation. However as gastrointestinal function in adults with Duchenne has not been rigorously studied there is little evidence and knowledge available on the key bowel issues and how they are treated across the world.

Questions to consider:

- What are the key bowel issues that affect adults living with Duchenne muscular dystrophy?
- What methods are most useful to help overcome bowel issues in adults with Duchenne?
- What dietary advice is most useful to adults living with Duchenne?

13. Clinical Trials

Historically clinical trials and the focus on treatments for people with Duchenne muscular dystrophy have only been for children living with the condition. This has meant a large population of adults with the condition who have had no hope of emerging treatments that may be able to support them.

However, with people living longer with Duchenne and the first trials and treatments that can support adults now on the horizon greater input and thought over trials for adults is necessary.

Questions to consider:

- What are the key areas that treatments emerging from trials could treat within adults with Duchenne?
- How can clinical trials be best designed for adults living with Duchenne?

