

The importance of nutrition in Duchenne muscular dystrophy

Ingrid E.C. Verhaart^a, Lenie van den Engel-Hoek^b, Marta L. Fiorotto^c, Mirjam Franken-Verbeek^a, Elizabeth Vroom^{a,d}

^aDuchenne Parent Project NL, the Netherlands

^b Department of Rehabilitation, Donders Institute for Brain, Cognition and Behaviour, Radboud University Medical Center, Nijmegen, the Netherlands

^c Department of Pediatrics, USDA/ARS Children's Nutrition Research Center, Baylor College of Medicine, Houston, Texas, US

^d World Duchenne Organisation (UPPMD), the Netherlands



Duchenne muscular dystrophy

Duchenne muscular dystrophy (DMD) is caused by the absence of a functional dystrophin protein. During disease progression muscle tissue is gradually replaced by fibrotic and fat tissue.

Currently there is no cure available that is applicable to all patients; only two therapies for selected groups of patients are on the market. Patients are mainly treated symptomatically, most patients use corticosteroids.

Metabolism in DMD

- The absence of dystrophin has many secondary effects
- The metabolic system is affected due to
 - Disturbed intracellular signalling pathways
 - Changes associated with breakdown of muscle tissue
 - Corticosteroid use
- Humans/animal models show perturbation of
 - Insulin signalling
 - Mitochondrial function

Body composition in DMD

- Altered in DMD patients, which impacts
 - Energy expenditure
 - Nutrient requirements
- Risk overweight in early disease stages
 - Possible causes
 - + Decreased physical activity
 - + Corticosteroids
 - + Compensatory behaviour family members
 - Increased load on muscles
- Risk underweight in later disease stages
 - Possible causes
 - + Decreased appetite
 - + Difficulties chewing, swallowing and digestion
 - Lower immune response
 - Osteoporosis
 - Exacerbation muscle wasting
- Major impact on quality of life

Workshop on nutrition in DMD

- 16-18 March 2018
- Representatives academia, clinics, patient organisations, industry

Aims

- 1) Discussion existing knowledge regarding body composition and metabolic aspects DMD
- 2) Determine priorities for future research
- 3) Improvement nutritional guidelines and information for patients/caretakers

1. Existing literature

There is a lack of knowledge concerning

- Natural history data on nutrition-related outcomes in DMD
- Weight management during corticosteroid use
- Gastrointestinal problems in DMD
- Role glucose intolerance.

2. Research priorities

- Reference data (natural history) tailored to DMD
 - Body composition
 - Glucose metabolism
 - Dental involvement
- Current dietary habits/supplement use
- Methods to measure weight/body composition of non-ambulant patients
- Standard Operating Procedures (SOPs) for preclinical research needed to improve comparability and reproducibility of results between labs
- Psychological aspects of nutrition
 - Obstacles preventing dietary adjustment
 - Reluctance against gastrointestinal tube feeding

3. Nutritional guidelines

- The current guidelines are very general
- There is a need for better guidelines
 - Disease stage/age specific
 - + Childhood
 - + Late childhood/adolescents
 - + Adults
 - For patients, caregivers and clinicians/dietitians
 - Understandable
 - Easily implementable in daily life
 - Risks/benefits supplement use

Action points

- Dietitians should become part of the standard care team
- Task forces will be formed:
 - SOPs for preclinical research
 - + Including effects of diet
 - + Improve outcomes
 - Improvement guidelines
 - + Videos and one-page information sheets
 - Weight
 - Nutritional supplements
 - Gastrointestinal tube feeding

Recommendations for patients/caretakers

- Regularly consult a dietician
 - Especially during key periods (e.g. loss of ambulation)
- Maintain a healthy weight
- Discuss supplement use with the clinical team and pharmacist
- Dental hygiene is very important
- Adapt food if chewing/swallowing becomes difficult (smaller pieces, more fluid food)
- Make sure fluid intake is sufficient
- Drink after every meal

Duchenne Parent Project NL

Mission: Finding a cure or viable treatments for Duchenne Muscular Dystrophy and accelerating the development and availability of new drugs.

Website: www.duchenne.nl

Contact: info@duchenne.nl
+31206275062