NICE National Institute for Health and Care Excellence

NICE advice

# DMD Care UK's guideline on cardiac care of children with dystrophinopathy and females carrying DMD-gene variations: NICE review

NICE review

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www.nice.org.uk/advice/nr1

# Contents

This advice replaces NG251.

# Overview

NICE has reviewed DMD Care UK's guideline on cardiac care of children with dystrophinopathy and females carrying DMD-gene variations.

NICE has carried out this review to support improved care and outcomes for people with Duchenne muscular dystrophy (DMD), and to assist health and care practitioners in their decision making. However, NICE has not developed its own recommendations for this topic.

NICE has chosen to present this guideline on the basis that:

- it covers a condition that NICE does not have an existing guideline on
- it was developed using an appropriate process and methods.

**Disclaimer**

NICE does not assume responsibility for the content of the guideline, including its clinical recommendations, accuracy, or relevance to specific patient circumstances. Healthcare professionals should exercise their own clinical judgment and consider local policies, individual patient needs, and other relevant guidance before applying any information from the linked material in practice.

# NICE commentary

NICE has reviewed DMD Care UK's guideline on Cardiac care of children with dystrophinopathy and females carrying DMD-gene variations.

We think the guideline from DMD Care UK is a useful resource that will help clinicians improve care in this area.

## Summary of the review

Overall, the recommendations in the guideline are specific, clearly described and editorially independent, and relevant stakeholders were involved throughout the process. Although the evidence used was not identified through a systematic review, the recommendations were created and curated by a large number of clinical experts and patient representatives.

The applicability limitations could be addressed in future updates by analysing data from an audit of Duchenne muscular dystrophy care centres, to identify remaining barriers to implementing the recommendations.

## Review process

NICE has reviewed the guideline using AGREE II assessments. This is a structured approach to evaluating the quality of clinical guidelines.

## Strengths of the guideline

- The guideline was developed by an expert working group made up of specialist clinicians and patient representatives from across the UK. It was reviewed and endorsed by the British Cardiovascular Society. Additionally, the recommendations were reviewed by over 100 clinical experts. This gives confidence in the acceptability of the recommendations to services and users.
- The guideline clearly describes the objectives, health questions and the population.
- The management pathway is well presented, and the key recommendations are easy

to identify.

- The expert working group was editorially independent, as the guideline clearly stated the funding body and the group had no competing interests.

# Limitations of the guideline

- There are inherent challenges in developing guidance for rare diseases, such as limited published evidence.
- The guideline used a literature review rather than a systematic review to identify any relevant evidence.
- No information on the strengths and weaknesses of the included evidence is reported. However, because of the lack of data on Duchenne muscular dystrophy, many of the recommendations are based on the consensus of the expert working group rather than evidence identified from the literature review.
- There is a lack of information on the applicability of the recommendations. No information is provided on implementation advice, facilitators and barriers, or resource implications. No cost and resource analysis was carried out.

**Users should be aware that this is not NICE guidance and that the development does not follow NICE's published methods and processes.**

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