

New consensus care guideline will significantly improve quality of life for adults with Duchenne muscular dystrophy

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Many more adults with Duchenne muscular dystrophy (DMD) are living longer thanks to improvements in treatment, however international

standards of care have not yet fully addressed the complex needs of these patients. The Adult North Star Network (ANSI) has developed a set of consensus-based recommendations designed to significantly improve quality of life for patients living longer with DMD. These recommendations are published in the *Journal of Neuromuscular Diseases*.

DMD is a severe, progressive, muscle-wasting disease that leads to difficulties with movement and, eventually, to the need for assisted ventilation and premature death. It is caused by mutations in the gene encoding dystrophin that abolish the production of this protein in muscle. Corticosteroid treatment and international published standards of care for DMD have significantly improved survival, and many more adults are now living with the condition well into their 40s. These patients have complex medical needs that to date have not been comprehensively addressed in international standards of care.

"Most adults with DMD are medically frail and thus highly vulnerable. As their condition progresses, in addition to severe muscle wasting and weakness, they may experience increasingly complex health issues including [cardiac failure](#), weight loss, metabolic decompensation, pain, bladder symptoms, renal dysfunction, and bowel dysmotility that do not generally occur in childhood," explained lead author Ros Quinlivan, MD, MRC Centre for Neuromuscular Disease, UCL Institute of Neurology, National Hospital for Neurology and Neurosurgery, London, UK. "For [adult patients](#) the emphasis of care shifts from a preventive approach to a treatment approach, which is not fully addressed in previously published guidelines, and healthcare providers are less experienced in managing these patients."

ANSN worked to develop a consensus-based standard of care through a series of multidisciplinary workshops held over a three-year period. These included clinicians and allied health professionals from a wide

range of clinical areas (neurology, cardiology, respiratory medicine, gastroenterology, endocrinology, palliative care, rehabilitation, renal medicine, anesthetics, and clinical psychology) as well as service users and representatives from patient advocacy groups.

The resulting set of guidelines, approved by all members of ANSN including service users, adds to, but does not replace, previous guidance in the international standard of care for DMD. In line with concerns raised about adult care, it serves as a framework to support clinicians and improve clinical services including multidisciplinary care for adults living with DMD.

Among the key recommendations are:

- Adults with DMD have complex health needs and should be seen in centers with experience and expertise in the management of DMD.
- Adults with DMD on corticosteroids require proactive management to mitigate against the ongoing complications of steroids, and the need to initiate non-invasive ventilation (NIV) is likely to be delayed in this group.
- There is no evidence that starting steroids in adults is beneficial, however, stopping steroids should only be considered when side effects outweigh benefit or if the patient chooses to discontinue.
- All patients require regular monitoring of respiratory function and screening for nocturnal hypoventilation. Patients on steroids are also at risk of obstructive sleep apnea.
- Cardiac function should be monitored at least once a year and all patients should be receiving an ACE inhibitor and/or beta blocker with consideration of adding eplerenone.
- Cardiac drug therapy must be carefully titrated and monitored, and additional agents may be required.
- There should be regular monitoring of full blood count and renal

function (cystatin C) as markers of renal insufficiency.

- Assessment of swallowing and [nutritional status](#) should be included in the regular assessment of patients.
- There should be good lines of communication and information sharing among all specialists caring for DMD adults, particularly when general anesthetic will be administered.
- Psychological support, including medical treatment when necessary, to improve participation and reduce symptoms of anxiety and depression should be made available to all patients.
- Adult patients approaching end of life should have access to a palliative care consultant to manage their symptoms and discuss an advance care plan.

"We hope that these recommendations will support physicians caring for adults with DMD, and thus provide better equity of care for patients and improve outcomes," said Professor Quinlivan. "This Guideline also highlights gaps in the literature where further research is required, for example, in managing gastroenterology and renal complications of the disease."

More information: R. Quinlivan et al, Adult North Star Network (ANSN): Consensus Guideline For The Standard Of Care Of Adults With Duchenne Muscular Dystrophy, *Journal of Neuromuscular Diseases* (2021). [DOI: 10.3233/JND-200609](https://doi.org/10.3233/JND-200609)

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