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Destination therapy with VAD for patients with dystrophinopathies: A new way of life

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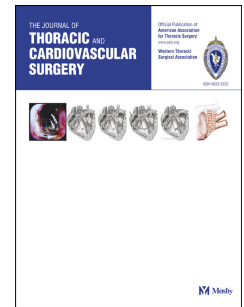
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**Destination therapy with VAD for patients with dystrophinopathies:
A new way of life**

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Dystrophinopathies are X-linked disorders associated with progressive skeletal muscle weakness and dilated cardiomyopathy. In the current era, end-stage heart failure is the predominant cause of mortality in this population. The coexistence of progressive skeletal and cardiac muscle compromise creates a real challenge to identify the real culprit for the progressive and unrelenting functional decline affecting these patients. Although selected individuals with preserved skeletal muscle function and clear evidence end-stage heart failure have been transplanted with good outcomes, this constitutes the exception as most patients are not considered suitable candidates due to advanced skeletal muscle compromise, lack of clear distinction between cardiac and skeletal muscle compromise or a combination of both.

The successful and widespread use of ventricular assist devices as destination therapy has opened the door to consider this novel management option for individuals with a dystrophinopathy affected by significant cardiac muscle compromise.

The report by Perri et al (1) provides further evidence to consider the use of ventricular assist devices, potentially changing the current management paradigm for these patients. In light of potential candidacy for VAD support, differentiation between worsening functional capacity secondary to heart failure as opposed to progression of the skeletal muscle pathology is a difficult but essential process to identify those individuals who would benefit the most from transplantation or VAD support. The use of VAD therapy not only offers the opportunity to evaluate patients for transplant candidacy once heart failure has been resolved, but as suggested by the authors, it can also provide effective and durable support as destination therapy for those not eligible for transplantation.

The small size of the new generation of pumps offers important advantages in smaller patients and those with skeletal muscle disease, expanding the applicability of this type of support. Moreover, the intra-pericardial placement can mitigate the impairment of diaphragmatic function, associated with large para-corporeal cannulas, leading to preservation of respiratory mechanics, which is essential in patients with muscular dystrophy. Lastly, the intra-corporeal nature of the VAD system utilized is

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