Providing hope

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Amodeo and colleagues¹ share their pioneering series providing chronic left-ventricular assist support to young men with Duchenne muscular dystrophy (DMD) in severe medically resistant heart failure. DMD is an X-linked progressive disorder of the dystrophin gene that leads to progressive muscle weakness resulting in loss of lower extremity use (with patients becoming wheelchair bound in their second decade) and eventually loss of upper extremity use and respiratory muscle function requiring positive-pressure ventilation. Despite their physical infirmities, these young men are neurologically intact, have interactive and engaging lives, and are often the epicenter of their family unit. Unfortunately, they almost all develop heart failure and approximately 50% to 60% die as a result of medically resistant heart failure in the late second or third decade of life.^{2,3} In most cases, these young men are not considered heart transplantation candidates because of the progressive nature of their disease, resulting in respiratory failure and the inability to undergo physical rehabilitation.⁴ The knowledge that one will have progressive heart failure, which will be treated with multiple admissions and an increasing escalation in medical therapy, usually resulting in a chronic infusion of milrinone and eventual hospice care, is a fact that many of these young men face. However, the advent of continuous-flow left-ventricular assist devices, which do have their issues, has provided reliable support for many patients for more than 5 years, and for a few patients it has provided support for almost a decade. These statistics have led members of the medical community to contemplate the potential use of ventricular assist device (VAD) therapy for patients with DMD.

The current series demonstrates, as has been shown at our institute as well as others, that one can provide an effective therapy for certain patients with DMD and heart failure.⁵⁻⁸ As the authors highlight, there are challenges with implantation, postoperative care, and discharge but the results are impressive, albeit from a small series of 7 patients. When VAD therapy is

Dr Morales at the OPBG, Italy, visiting the first patient with DMD supported with a VAD.

Central Message

Ventricular assist device therapy is being effectively used for medically resistant heart failure in patients with Duchenne muscular dystrophy, extending their lives and perhaps providing hope.

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applied to a new cohort, maximizing outcomes is almost certainly linked to understanding where in the disease process VAD therapy should be applied, and that is particularly true in patients with DMD who have multiple medical and physical limitations. Experience with this patient population is increasing and many feel that initiating VAD therapy in young men, once they are on chronic milrinone and still have upper extremity function and are not fully ventilated may be an appropriate time to intervene. However, there is much to still be learned.

There are many new therapies being developed for patients with DMD to halt and possibly even reverse the progression of their disease.⁹ However, the population of young men with DMD and heart failure resistant to medical therapy continues to grow. Amodeo and colleagues¹ have demonstrated that some of these patients can be treated effectively with VAD therapy. These young men may, therefore, have the option to extend their lives and possibly have the opportunity to benefit from the impressive medical advances being made. Perhaps they and their families have been provided hope.

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