Introduction

Advances in respiratory care have prolonged the lives of children with DMD such that death often results from cardiac sequelae. There have been several reports of successful heart transplantation for patients with Becker’s Muscular Dystrophy and carriers but not for patients with DMD, the most severe form of the disease. Organs are a scarce resource. In 2010, 532 heart transplants in children were reported worldwide. In contrast, a left ventricular assist device (LVAD) is not a scarce resource.

LVADs prolong survival, enhance quality of life, and improve functional status as compared with medical therapy alone. The 1- and 2-year survival rates with LVAD are similar to those of heart transplantation. Patients are now being offered LVAD therapy as destination therapy, LVADs prolong survival, enhance quality of life, and improve functional status with LVAD insertion as destination therapy. Patients are now being offered LVAD therapy as destination therapy, not just as a bridge to transplantation.

Case Report

A 17 year-old boy with DMD and DCM, with good functional status and quality of life, presented for emergency LVAD insertion as destination therapy. He was admitted to the PICU with sudden, rapidly deteriorating cardiac failure, requiring multiple inotropes, pressors and antiarrhythmic therapy. He suffered refractory episodes of ventricular tachycardia despite a previously placed AICD. LVAD insertion was uneventful and immediately life-saving; the patient recovered without complication, and was discharged home neurologically intact, in good spirits, on his usual pulmonary regimen.

References

1. Diagnosed with Dilated Cardiomyopathy (DCM) and Duchenne Neuromuscular Disease, with good functional status, presented for emergency LVAD insertion as destination therapy. The patient recovered without complication, and was discharged home neurologically intact, in good spirits, on his usual pulmonary regimen. 2. LVADs prolong survival, enhance quality of life, and improve functional status as compared with medical therapy alone. The 1- and 2-year survival rates with LVAD are similar to those of heart transplantation. 3. Patients are now being offered LVAD therapy as destination therapy, not just as a bridge to transplantation.

Discussion and Conclusions

A lethal diagnosis is a function of what medical interventions are available or offered to the patient. Futility in this case cannot be invoked as a reason for denial: the goal of care was to extend life, therefore this intervention could not be deemed futile. Both the family and patient fully understood the significant risks and benefits as well as short-term and long-term consequences of proceeding and provided informed consent.

This case provides an opportunity for the medical community to rethink whether a VAD (as destination therapy or even as a bridge to transplant) should be offered in DMD patients. Ethically unacceptable principles of utility and justice in the allocation of organs for transplant include ability to pay, social worth and past use of medical resources, principles that must be considered in this case as well.

UNOS has articulated a policy for equitable organ allocation: organs should be allocated based on medical criteria, striving to give equal consideration to medical utility (net medical benefit to all transplant patients as a group) and justice (equity in distribution of the benefits and burdens among transplant patients). Historically, patients with disabilities were not deemed appropriate transplant candidates but case law and advocacy are overturning even this notion.

There is a strong and growing advocacy for persons with serious or life-threatening disabilities that many believe pose serious moral and ethical dilemmas for society as a whole. Since LVADs are an acceptable option in adult patients with end-stage heart failure who are not candidates for transplantation and have been shown to prolong survival, enhance quality of life, and improve functional status when compared to standard medical therapy alone, it becomes increasingly difficult to deny such therapy to carefully selected children with neuromuscular disease.

As experience accumulates, technology will invariably improve and complication rate decrease making the argument of excessive cost and resource utilization less persuasive. 