Letter to the editor

Left ventricular assist device in Duchenne Cardiomyopathy: Can we change the natural history of cardiac disease?

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End stage dilated cardiomyopathy (DCM) is currently one of the most challenging elements in the management of patients affected by Duchenne muscular dystrophy [1]. DCM is a complication of Duchenne muscular dystrophy, and leads to advanced heart failure and premature death [2,3]. Until the last decade, cardiomyopathy in Duchenne muscular dystrophy accounted for only 20% of deaths because respiratory failure occurred earlier than cardiac failure. Due to recent technological advances, respiratory care has greatly improved and life expectancy has increased to 30–40 years. The efficacy of standard heart failure treatment for improving the clinical outcome of these patients has been proven, but nevertheless more than 40% will die of heart failure [2].

Duchenne syndrome has generally been considered a contraindication for cardiac transplantation due to the associated progressive skeletal myopathy leading to limited functional capacity [4]. This concern has resulted in a reluctance to offer cardiac transplantation to these patients in an era of donor shortage. The recent advances in left ventricular assist devices, used as destination therapy, have made feasible the use of such devices for the treatment of DCM in Duchenne patients.

We present two adolescents with Duchenne muscular dystrophy admitted to our department because of acute heart failure, for which a ventricular assistant device (VAD) was decided as destination therapy. Case 1. A 15 year-old boy who, during the last year, experienced three episodes of acute cardiac failure despite maximal medical treatment, was admitted to our ICU with intractable cardiac failure despite maximal medical therapy, due to rapid and intractable cardiac failure unresponsive to high dosage inotropes. We elected to implant, as destination therapy, a left VAD (Jarvik 2000) with the pedestal inserted into the skull. The patient needed daily treatment with a cough machine and non invasive ventilation (NIV) for two months. He was discharged three months after surgery and resumed normal activities, including attending secondary school. One year post surgery, he developed osteolysis at the pedestal site which required surgical revision with displacement of the pedestal position. Case 2. A 14 year-old boy with Duchenne DCM went into acute cardiac failure and was admitted to our hospital. A few hours after admission, he suffered a cardiac arrest requiring institution of ECMO. A Jarvik 2000 device was implanted on the 12th day of ECMO as destination therapy. A spleen lesion occurred during chest drain insertion, necessitating four laparotomies and discontinuation of heparin infusion for 35 days due to persistent bleeding. He was discharged in good condition 6 months from surgery.

Our two case reports describe for the first time the use of VAD as destination therapy in young patients emphasizing the use of VAD as a new therapeutic option in Duchenne DCM. This subgroup of patients currently represents a newly-emerging therapeutic challenge. Cardiac involvement is present in about 90% of these patients, the majority of whom develop DCM in their mid-teens [2] and options for treatment of the end-stage disease are scarce. In a multi-institutional study involving 29 transplant centers in North America, over a period of 15 years only 3 cardiac transplants were performed for Duchenne syndrome out of a total of 7810 transplantations [4]. In the 2005 the American Academy of Pediatric has underlined that the cardiac involvement was not yet identified and made a call to action to maximize the understanding and the treatment of the cardiovascular involvement [5]. This is the first report of successful VAD implantation in Duchenne patients with end-stage cardiac failure and their subsequent discharge home. Given the increasing pediatric and adult population of Duchenne with end-stage cardiac failure, this experience represent a significant stepforward for patients with otherwise no therapeutic option.

References


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