Title: Heart Transplantation in Males with Dystrophinopathy: An Advanced Cardiac Therapies Improving Outcomes Network (ACTION) Dystrophinopathy Registry Analysis

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Purpose: End-stage cardiomyopathy is the leading cause of mortality in the dystrophinopathy population. While heart transplantation (HT) remains the gold standard for end-stage heart failure, this has infrequently been offered to patients with dystrophinopathy. Given rarity, this study sought to collect and describe the outcomes associated with HT in males with dystrophinopathy.

Methods: The ACTION dystrophinopathy registry was created in 2021 with >20 centers and is meant to include all patients with cardiomyopathy. Data points include demographics, neuromuscular, cardiac, and pulmonary function endpoints, medication use, advanced cardiac therapies, and outcomes. Post-transplant complications include rejection, infection requiring hospitalization, post-transplant lymphoproliferative disease, and need for feeding tube or tracheostomy. The registry was analyzed for all enrolled male patients who underwent HT.

Results: A total of 25 males underwent HT at 16 different centers: 19 (76%) with non-Duchenne (classified as Becker or intermediate muscular dystrophy) and 6 (24%) with Duchenne muscular dystrophy (Table 1). The mean age at time of transplant was 16.6 (IQR 15.0-17.5) years. The majority were white (64%), ambulatory (88%), and did not require pre-transplant respiratory support (72%). Thirteen (52%) had a prior HF admission and six (24%) were bridged to HT with a ventricular assist device. The median time to extubation was 1 (IQR 1-2) day, with a median intensive care length of stay of 7.5 (IQR 5.3-17.0) days and hospital length of stay of 18.0 (13.8-29.4) days. Five (20%) experienced post-transplant complications: 1 with both rejection and infection, 2 with rejection, and 2 with infection. Survival was 100% at 1 year and 89.3% at 5 years post-transplant.

Conclusions: This study demonstrates that in a highly select population of males with dystrophinopathy, HT can be a viable option. Further research is needed to refine HT candidate criteria and enhance survival and quality of life.