VAD and Heart Transplantation in Duchenne Muscular Dystrophy: Guidance for Providers

ADVANCED CARDIAC THERAPIES IMPROVING OUTCOMES NETWORK

HARMONIZED PROTOCOL

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INTRODUCTION

Evolving Outcomes in DMD

The care of patients with Duchenne Muscular Dystrophy (DMD) is evolving rapidly, with advances in survival due to improved respiratory care measures, use of steroids to reduce inflammatory injury to skeletal muscle, and rapid introduction of gene targeting therapies to help preserve muscle function in a wide variety of means. Given this revolution in care and outcomes, it is appropriate to review the role of advanced cardiac therapies for this population, to ensure that use of VAD and access to heart transplantation is consistent with current medical realities.

This document is intended to provide shared knowledge and guidance for internal use by programs that wish to launch programs offering advanced cardiac care options to patients with DMD, or to enhance existing programs.

Heart Failure Phases in DMD

Prophylactic Care
Prophylactic therapy for early intervention, aims to preserve cardiac muscle function and delay the onset of cardiomyopathy. Guidance for this phase of care is provided elsewhere and addresses time to initiate heart failure medications as well as the need for systematic monitoring.

Asymptomatic LV Dysfunction
As DMD cardiomyopathy progresses and signs of cardiac dysfunction begin to appear, the pharmacologic approach intensifies. Combination heart failure therapy should be provided as tolerated, including Angiotensin blockade (e.g. ACE inhibitor, ARB or Sacubitril/Valsartan), beta blockade, aldosterone antagonist and perhaps SGLT2 inhibitor.

In this phase, the goal is to maintain stability in cardiac imaging and biomarkers, and to identify when heart failure symptoms develop.

Symptomatic Heart Failure
In the symptomatic phase of HF, the treatment focus shifts to the active management of HF symptoms. Symptoms may manifest as fatigue, diminished exercise capacity, impaired respiratory status not thought to be due to skeletal muscle dysfunction, or fluid overload. Diuretics play an increasing role in care, and the clinical course may evolve rapidly due to complications such as arrhythmias.

Current Experience with HF and VAD

A small number of case reports demonstrate feasibility of VAD support for DMD patients with outcomes limited primarily by the progression of DMD, and adverse events occurring at rates comparable to other populations treated with VAD. Literature includes a multi-center series of 18 muscular dystrophy patients treated with VAD, with 8 having DMD. Notably, 95% of the total cohort survived to hospital discharge. This series highlights the feasibility of VAD therapy for certain DMD patients while leaving most questions of selection and outcome unresolved.

With respect to heart transplantation, there are scattered case reports showing successful use of heart transplantation to treat advanced heart failure in patients with DMD. Additionally, a multi-center publication from the Cardiac Transplant Research Database identified 3 individuals with the diagnosis of DMD in that database, which spanned 1990-2005 and included 7,820 adult heart transplant recipients. Muscular dystrophy patients in this cohort had outcomes broadly comparable to the overall cohort. Similar to the situation with VAD, the use of heart transplantation in the DMD population has been uncommon, but appears feasible in selected candidates.

PRIOR TO INITIAL REFERRAL

Barriers to Referral

Awareness of the options of VAD and heart transplantation for DMD remains limited among patients, caregivers, and medical teams. Many individuals with DMD receive care at multidisciplinary clinics that have considerable DMD expertise but lack the capability to place and manage VADs or perform heart transplants. Partnerships between DMD care teams and advanced cardiac care centers can optimize not only access but also health outcomes for DMD patients.

Neuromuscular and cardiology providers should begin conversations about the potential of advanced therapies with patients and caregivers early in the care of DMD, well before any acute needs arise. These discussions could be integrated into system-based checklists used to manage care by many neuromuscular centers or "bright future" style anticipatory guidance during routine visits. Key topics for this approach include:

Table 1: Anticipatory Cardiac Guidance for DMD Care

- *Framing VAD placement and/or heart transplant as potentially viable, (though optional) treatments for some DMD patients with heart failure*
- *Building a general understanding of pre-, peri-, and post-operative management for these interventions*
- *Contextualizing advanced cardiac therapy within broader DMD care (e.g., nocturnal ventilation, heart failure management)*
- *Making patients and caregivers aware of additional resources available through partnering centers, advocacy organizations, and other reputable sources.*

Advanced cardiac centers may also consider offering adjunctive group or telehealth visits in the pre-referral period or after a referral. These visits would provide patients and caregivers an opportunity to meet care team members, ask nuanced questions specific to each treatment site, and help patients make informed care decisions.

For patients with DMD, routine cardiology care is recommended with a cardiologist experienced in managing DMD. It is important for DMD patients to establish a connection to a program that offers advanced cardiac therapies before significant heart failure symptoms develop as the clinical course subsequent to onset of symptoms is difficult to predict and may progress rapidly. Additionally, prolonged debilitation from heart failure may preclude eligibility for advanced cardiac therapies.

Optimizing Longitudinal Care

The consideration of advanced cardiac therapies may directly impact the management choices in certain DMD patients. Optimizing aspects of DMD care may increase a patient's eligibility for VAD placement or heart transplant later, particularly when specific factors for a poor surgical outcome are identified and proactively managed. By mitigating these potential vulnerabilities, patients may face fewer risks and have improved outcomes when they become candidates for VAD or transplant. Areas that should be targeted include:

Table 2: Optimizing Longitudinal Care

Area of Risk	Potential Interventions
Scoliosis	*Comfortable seating, reduced risk of pressure ulceration, improved pulmonary biomechanics*
Nutritional Health	*Gastrostomy tube to preserve nutritional status, leading to reduced risk of pressure ulceration, improved wound healing and more robust tissue integrity*
Osteopenia	*Support of bone health with directed therapeutics and appropriate steroid regimen can minimize risk of stress fracture and compression fractures, and improve post-operative mobilization*
Sarcopenia	*Pre-treatment activity regimens with stretching and strength building can improve pulmonary function and skeletal muscle strength, leading to earlier extubation and mobilization*
Weight management	*Avoidance of obesity and cachexia can reduce surgical risk, facilitate earlier extubation and mobilization, and reduce risk of pressure ulceration*
Pacemaker/ICD utilization	*Prevention of arrhythmia complications including cardiac arrest*
Respiratory Insufficiency	*Airway clearance and non-invasive respiratory support including positive pressure*

Indications and Timing of Referral

Traditional tools such as cardio-pulmonary exercise testing and NYHA heart failure classifications often prove inadequate to assess HF severity in DMD due to the neuromuscular limitations of DMD patients, especially for those who are non-ambulatory.

Referral for advanced cardiac therapy evaluation should be strongly considered if any of the following are present:

Table 3: Indications for Referral for Advanced Cardiac Therapy Evaluation

- *LVEF < 40%*
- *End-organ dysfunction (renal or liver dysfunction) due at least in part to heart failure*
- *Persistently elevated NT-proBNP*
- *Persistent fluid overload or increasing diuretic requirement*
- *Recurrent defibrillator shock*
- *Heart failure hospitalization*
- *Need to reduce/discontinue standard HF medications due to side effects*

These criteria should not be considered an absolute indication for advanced therapy, but rather an opportunity to engage relevant providers and families in the discussions regarding goals of therapy, indications for advanced therapy, and potential barriers to consideration for advanced therapies. Referral for advanced cardiac therapy evaluation should not necessarily entail a transfer of care to centers offering advanced cardiac therapies. Shared cared between the longitudinal care center and heart failure specialist is strongly encouraged.

PRE-SURGICAL EVALUATION

VAD vs Heart Transplant

In broad terms, VAD or heart transplant may be considered when medical therapy alone no longer proves effective, either by unacceptable heart failure symptoms, or the expectation of limited survival due to cardiac disease. VADs may serve as either a bridge to transplant (BTT) or destination therapy (DT).

Ambulatory status is not a primary determinant in this decision; however, if a patient does not have access to a 24/7 caregiver, hand function and proximal arm strength become essential considerations for managing VAD equipment independently.

Given the limited experience with both VAD and heart transplantation in this population, a comparison between these options should be informed by discussion with the patient and family regarding goals of care and priorities.

Assessment for Candidacy

For both VAD and heart transplantation candidacy, a comprehensive evaluation is indicated and should typically address the following considerations:

Table 4: Components of Pre-VAD and Transplant Evaluation

- *Pulmonary Function Testing (PFT): Collect baseline and trend data to inform perioperative planning and long-term management strategies.*
- *Neuromuscular Assessment: Comprehensive evaluation of strength and flexibility. Assessment of upper extremity function is particularly important for patients without full-time caregiving support, to determine capacity to manage VAD equipment.*
- *Nutritional Assessment: Ensure the patient's nutritional status is optimized prior to surgery to enhance recovery and support long-term health.*
- *Swallow Evaluation: Assess patient's ability to swallow prior to implant given the potential for sternotomy to affect vocal cord mobility and swallowing dynamics independent of neuromuscular status.*
- *Pediatric Surgery Planning: Coordinate with pediatric surgery for G-tube placement when indicated and discussion regarding VAD driveline placement.*
- *Psychosocial and Palliative Care Support: Involve palliative care early to address quality-of-life issues and establish realistic expectations for post-operative outcomes.*
- *Assessment of Caregiver Capacity: For patients reliant on caregivers, assess caregiver support to confirm adequate resources and abilities for post-VAD care.*
- *Assessment of Patient Capacity: Assess the patient's cognitive function and tailor education, assent/consent based on their capacity.*
- *Physical Therapy and Occupational Therapy (PT/OT) Assessment: Tailor PT/OT support to address specific rehabilitation needs, potential challenges following sternotomy (e.g. lifting and transfers), and maximize the patient's functional independence post-surgery.*
- *Orthopedic and Bone Health Evaluation: Evaluate scoliosis, chest wall stability, contractures, and bone health due to the long-term impact of steroid therapy, which can affect surgical and post-operative care.*
- *Venous Access Mapping: Plan venous access carefully, as DMD patients on long-term steroids may have calcified veins, increasing the risk of access challenges*

The patient with DMD who is referred for heart transplantation evaluation should undergo a comprehensive evaluation following the applicable institutional practice. This should be performed within the existing transplant selection process.

For patients accepted for transplant and placed on a waitlist, the dynamic nature of the underlying skeletal muscle and pulmonary disease should be kept in mind during a potentially long waiting interval. At the time of donor acceptance, the candidate should have recent pulmonary function testing (PFTs) and assessments of respiratory muscle strength (MIP/MEP, cough peak flow), ideally within the last 6 months.

SURGICAL AND PERIOPERATIVE CONSIDERATIONS

Preparation for Surgery

Ideally, assessments and interventions in key areas for longitudinal care discussed above should already be performed, but this should be reviewed and confirmed. Additionally, skin integrity should be regularly assessed and any ulcerations promptly treated. Need for post-operative gastrostomy tube should be anticipated and locations of both gastrostomy tube and VAD drivelines should be physically mapped out with respect to wheelchair restraining belts. A scoliosis evaluation should be performed. In addition, respiratory function should be fully characterized. Loss of 25-30% of FVC should be anticipated following thoracotomy.

Table 5: Respiratory Evaluation and Preparation

- *Pulmonary function testing with FVC, and FEF 25-75 to identify lung capacity and any restrictive or obstructive concerns*
- *Assessment of respiratory muscle strength with Max Inspiratory Pressure, Max Expiratory Pressure and Cough Peak Flow*
- *Assessment of end-tidal capnometry to serve as baseline value*
- *Polysomnography to understand need for positive airway pressure pre-operatively*
- *Tracheostomy teaching should be performed to prepare the family and patient*
- *Training with respiratory equipment such as CPAP, IPPB*

Surgical Technique

Use of TRAP door implantation technique may be considered in LVAD implantation to expand the pericardial space and minimize diaphragmatic disruption. Wheelchair bound patients are at high risk for driveline infections so careful attention should be paid to driveline position, enhanced by additional padding to reduce pressure.

Anesthetic Considerations

Table 6: Key Anesthetic Considerations

- *Airway management may be complicated by contractures, scoliosis or macroglossia*
- *Secretion management may require nebulizers, airway clearance and humidification during surgery*
- *Small tidal volume ventilation during CPB may mitigate small airway collapse*
- *Due to risk of rhabdomyolysis and malignant hyperthermia-like reactions, volatile anesthetics and succinylcholine should be avoided.*
- *Anesthesia machine should be flushed to remove traces of inhalational agents*
- *Monitoring of neuromuscular blockade should be performed to prevent residual blockade and facilitate early extubation*
- *ACE inhibitors/ARNi and SGLT2 inhibitors should be managed per institutional practice*
- *Perioperative steroids may be required to prevent emergence of adrenal insufficiency, depending upon prior therapy*
- *Vasoplegia management protocol should be in place per institutional practice*

Hemostasis and Thrombosis

There is increased risk of bleeding in DMD patients due to enhanced fibrinolysis and platelet dysfunction. Viscoelastic testing can support appropriate use of goal-directed intra-operative hemostasis, including reversal of anticoagulation in

VAD implantation. Meticulous hemostasis should be a priority for the surgical team. In the post-operative period, thrombotic complications may occur due to impaired venous drainage, and thromboprophylaxis should be employed including use of compression stockings.

Airway Management

Early extubation, ideally within 6 hours if hemodynamics are stable, is a key goal of post-operative management, with multiple ramifications. This requires an integrated approach to mechanical ventilation and pain management that maintains lung recruitment, supports gas exchange and minimizes splinting.

Table 7: Key Tenets of Airway and Ventilator Care

- *Focus on early extubation, preferably within 6 hours*
- *Full respiratory support during intubation*
- *Careful titration of opiate medications to avoid blunting respiratory drive*
- *Extubate from full support to full non-invasive support (usually to bilevel support)*
- *Avoidance of spontaneous breathing trials*
- *Airway clearance initially q2-3h*
- *Post extubation monitoring for hypercapnia*

Tracheostomy should be strongly considered if extubation has not been achieved in 5-7 days, to facilitate physical rehabilitation and clinical progression. Patients should be counselled about risk of tracheostomy in advance of surgery.

Preservation of Functional Status

Careful attention to wound care, liberal use of wound vacs and meticulous attention to early signs of pressure injuries are important to minimize the risk of such injury, particularly for patients on steroid therapy. Early mobilization can also be helpful for this, as well as for preservation of muscle strength and flexibility. Incorporation of rehabilitation teams into post-operative care planning is essential.

LONGITUDINAL CARE

VAD

In general terms, VAD management in a patient with DMD will follow the same treatment goals and principles as VAD care for other adolescents and young adults. Anticoagulation protocols may need to be tailored to individual circumstances given increased risks of both thrombosis and bleeding.

Heart Transplantation

A structured document ("flight plan") may be helpful in organizing peri-operative and post-operative care planning. This can be reviewed in a pre-transplant huddle of care providers. Post-transplant induction and maintenance immunosuppression should follow institutional practice. Vamorolone should not be regarded as an equivalent substitute for prednisone in anti-rejection properties given the lack of efficacy data, and consideration should be given to conversion to prednisone or deflazacort in the first 6 months in preference to vamorolone. For rejection surveillance, endomyocardial biopsies should be minimized given the increased procedural risk related to pulmonary and skeletal muscle function. Consider non-invasive surveillance including cell-free DNA and gene expression profiling in patients over age 15 years, combined with ECG, echocardiography, NT-proBNP and troponin as screening tools. Monitoring of renal function should be performed using Cystatin C rather than creatinine, given the reduced muscle mass in DMD patients. DMD patients are not believed to be at unusual risk of opportunistic infections when compared to typical heart transplant recipients. Ongoing management of bone health should follow institutional practice, and may include radiographic assessment of spine fractures, use of Dexa scans and consideration of bisphosphonate therapy. Rehabilitation should be directed by a team familiar with DMD patients and their care needs.

Repatriation for ongoing DMD Care

After discharge, the patient's local cardiologist may oversee routine VAD management or post-transplant care as per institutional protocols, coordinating closely with the advanced cardiac center to co-manage any complications. The handoff between the advanced cardiac center and local providers is critical. To support local providers and minimize the risk of delays in addressing complications, advanced centers may consider creating detailed care protocols or formal case management systems. These should include clear instructions on when to escalate care and ensure that local providers have ongoing telehealth support and 24/7 consultation access to advanced cardiac centers as needed.

Families should receive written care plans with escalation pathways and delineation of responsibilities between the local team and the referral center. Details will vary depending upon local center and family resources, but should be jointly established with both centers.

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