OPTN Thoracic Committee
Pediatric Heart Workgroup
Meeting Summary
February 25, 2020
Conference Call

Ryan Davies, MD, Chair

Introduction

The Pediatric Heart Workgroup of the Thoracic Committee met via Citrix GoTo teleconference on 02/25/2020 to discuss the following agenda items:

1. Public Comment Update
2. Discuss Coronary Allograft Vasculopathy (CAV) and Re-transplant Patients
3. Review Pediatric Guidance Document

The following is a summary of the Workgroup's discussions.

1. Public Comment Update

The Thoracic Committee has a proposal out for public comment for a National Heart Review Board for Pediatrics. UNOS staff provided an update from the first half of the public comment period, noting that regions have been generally supportive of the proposal and that public comment ends on March 24th.

Next steps:

After public comment closes, UNOS staff will send more detailed feedback from public comment to the Workgroup for discussion. The Workgroup will consider changes to the proposal based on the feedback.

2. Discuss CAV and Re-transplant Patients

The Workgroup previously discussed populations of pediatric heart candidates that warrant specific guidance for exceptions. On this call, the Workgroup discussed CAV and re-transplant patients.

Summary of discussion:

The Chair led the discussion with the Workgroup, noting that CAV and re-transplant patients do not have any particular prioritization under the current allocation system. These candidates generally are assigned to status 2 but transplant centers ask for exceptions when they believe they are merited. The Chair said that there might be value in standardizing the process by identifying criteria that could be included in a guidance document to distinguish these candidates as status 1A or 1B.

The Workgroup discussed the lack of data for pediatric CAV and re-transplant patients given the small size of the population, noting that it would be easier to evaluate which candidates should be elevated to status 1A or 1B if there was more data available on waitlist mortality following the pediatric heart allocation changes implemented in 2016. However, the Workgroup agreed that this is a high-risk population that tends to have higher waitlist mortality, and that it is best to keep these patients off of mechanical circulatory support, as those who receive mechanical circulatory support tend to have worse outcomes. The Workgroup agreed that candidates who have been revascularized should get 1A status.

The Chair asked the Workgroup if there are other criteria besides revascularization that could be put into guidance, like angiographic findings, degree of dysfunction, end-diastolic pressure, or conduction abnormalities. The Chair noted that it would be against policy to grant a 1A exception to a candidate who was not hospitalized. Members discussed a 2015 paper in the *Journal of the American College of Cardiology* by Steven J. Kindel and colleagues entitled, "Improved Detection of Cardiac Allograft Vasculopathy." The paper defined graft dysfunction using the following criteria: ejection fraction (EF) less than 45%, right atrial pressure less than 12 mm Hg, or pulmonary capillary wedge pressure (PCWP) greater than 15 mm Hg. Members agreed that this is a good starting point for the guidance document.

The Chair asked how many re-transplants were conducted in recent years to gauge whether there would be enough data to estimate waitlist mortality. UNOS staff looked at OPTN data, finding that 22 out of about 350 total pediatric heart transplants conducted in 2019 were re-transplants. The Chair noted that there may not be adequate OPTN data and that the Workgroup may need to rely on various studies like the Kindel paper to estimate waitlist mortality.

Next steps:

The Chair asked UNOS staff if it would be possible to compare OPTN data to the mortality data reported in the studies in order to compare this patient population to other candidates. UNOS staff requested that the Workgroup select some survival measures so that UNOS staff can align the OPTN data for comparison. The Chair asked the Workgroup members to review the studies and identify the appropriate measures for UNOS by the following week.

3. Review Pediatric Guidance Document

The Chair reviewed the draft guidance document with the Workgroup, including recommended criteria for exceptions for candidates in three diagnostic categories: dilated cardiomyopathy (DCM), restrictive or hypertrophic cardiomyopathy (RCM/HCM), and single ventricle heart disease.

Summary of discussion:

Category 1: DCM

Candidates under 5 kg and under 10 kg

The proposed guidance states that candidates under 5 kg should be considered for a status 1A exception if they are on at least one high-dose inotrope. Candidates under 10 kg are eligible for a status 1A exception if they are supported by inotropes and demonstrate some evidence of poor systemic perfusion, including non-invasive positive pressure ventilation or feeding intolerance. The Chair noted that the intent of this criteria is to avoid situations in which a candidate is given a ventricular assist device (VAD) just to achieve a higher status for transplant. A member suggested adding in one high-dose inotrope for the under 10 kg group, and the Chair and other members agreed, noting that the guidance is intended to capture candidates for whom inotropes are not working well.

Another member mentioned that candidates with progressive pulmonary hypertension often need non-invasive positive pressure ventilation, not because of poor systemic perfusion but because the candidates have significant collapse due to cardiomegaly. The member noted that pulmonary vascular resistance (PVR) is often mentioned in exceptions requests but said that it may be too vague to include in guidance. The Chair agreed with excluding PVR from the criteria, reiterating that the goal of the guidance document is to limit exceptions in order to grant them to the candidates who are declining rapidly and who would ideally get a transplant instead of a VAD.

With regard to feeding intolerance, the Workgroup discussed adding more details to explain that the criterion refers to candidates who are unable to tolerate full calories (e.g. using a feeding tube but

vomiting). One member suggested adding lack of weight gain as a clarifying criterion. The Chair agreed to work on making this language more specific.

The Chair asked whether any other criteria should be included in this section, like renal insufficiency or other indications of poor systemic perfusion, though the Chair noted that the guidance should not encourage letting patients get sicker while they are on the waitlist. Members did not have any additions.

Candidates 10 kg and over

For this population, Chair noted that the primary reason to provide a 1A exception is the presence of contraindications to mechanical circulatory support. The proposed guidance document lists criteria that would demonstrate to a review board that a candidate has either contraindications to a VAD or indications that inserting a VAD would be very high-risk. Members expressed concern that the guidance might disadvantage candidates at centers without much experience with VADs or that are not willing to insert a VAD in certain circumstances. The Chair requested that the Workgroup members review this section again and offer suggestions for improvement, noting that the Workgroup will receive further feedback from the community when the guidance goes out for public comment.

Category 2: RCM/HCM

This category combines guidance for RCM and HCM patients and recommends a Status 1A exception for RCM/HCM candidates on inotropes, experiencing episodes of recurrent sudden death, experiencing syncopal episodes, or showing evidence of increased pulmonary vascular resistance.

The Chair reiterated that the goal is to serve the population of RCM and HCM patients that did not get 1A exceptions under the pediatric heart allocation changes implemented in 2016 and had very high mortality risks, as shown in a 2019 *American Journal of Transplantation* paper by Brian Feingold and colleagues entitled, "Impact of the 2016 revision of US pediatric heart allocation policy on waitlist characteristics and outcomes." A member noted that the guidance document will help in two ways: first by recommending an exception for these patients, and second, by better defining and limiting the population of DCM patients who qualify for a 1A exception. The Chair explained that he listed multiple criteria in addition to inotrope use so that the guidance document does not encourage clinicians to give inotropes to patients unnecessarily. The Workgroup agreed with the guidance listed in this section.

Category 3: Single Ventricle Heart Disease

The Chair noted that the adult heart allocation system changed to list adults with congenital heart disease (CHD) at adult status 4, which is equivalent to pediatric status 1B, but pediatric candidates with CHD remain at pediatric status 2. The Chair explained that the goal of this section is to make the policies more congruent so that CHD candidates are not more advantaged when they turn 18 than when they are under age 18. The guidance details when these patients should qualify for status 1A and when they should qualify for status 1B, based on current guidance for adults with CHD.

Workgroup members considered guidance for candidates who have undergone a Fontan procedure. Based on the adult CHD guidance, the Chair recommended that Fontan candidates qualify for a 1B exception because they are often listed at status 2 and then get sicker before transplant, which is not ideal. The Chair suggested that these candidates should qualify for a 1A exception if admitted to the transplant hospital and experiencing complications related to CHD.

Members expressed concern that this guidance may not appropriately align priority for transplant. One member was not certain that adult Fontan candidates have an advantage over pediatric Fontan candidates. The Chair explained that for adult donors, pediatric 1B candidates are in the same group as adult status 4, but for pediatric donors, pediatric 1B candidates are offered hearts ahead of adult status

4 candidates. The Chair noted that this guidance would not affect a lot of patients, but that a few transplant centers have expressed concerns about the apparent disparity.

The Workgroup agreed that it would be helpful to have more data to understand whether this population should be covered in the guidance document. The Chair said it would be helpful to know if CHD patients over the age of 12 have different outcomes on the waitlist before and after age 18 under the current allocation system. The Chair suggested that the Workgroup try to gather some data from the OPTN and/or the Pediatric Heart Transplant Society (PHTS) on this population. The Chair asked Workgroup members to consider whether there are single ventricle patients who should be listed at status 2 or whether they should be listed at status 1B as a baseline, noting that he does not think that CHD candidates are disadvantaged overall.

Next steps:

The Chair asked the Workgroup to continue considering the questions raised on the call to revisit in a future meeting. The Chair confirmed with UNOS staff that a draft product needs to be ready by late April or early May for the guidance document to be included in the next public comment period in fall 2020.

Upcoming Meetings

- March 24, 2020
- April 28, 2020