

**OPTN Pediatric Heart Workgroup
Meeting Minutes
September 24, 2019
Conference Call**

Ryan Davies, MD, Chair

Introduction

The OPTN Pediatric Heart Workgroup met via Citrix GoTo teleconference on 09/24/2019 to discuss the following agenda items:

1. Review Pediatric Transplantation Data
2. Review Proposed Solutions and Common Diagnoses
3. Review Board Operations

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

1. Review Pediatric Transplantation Data

UNOS staff provided an overview of findings from the "2016 Changes to Pediatric Heart Allocation Policy Evaluation" monitoring report.

Data summary:

The percentage of pediatric heart transplants in candidates with Status 1A exceptions increased from pre to post policy change. This varied across regions post policy, with region 3 being the highest at almost 25%. Approximately 17% (N=38) of transplant recipients diagnosed with cardiomyopathy were in Status 1A by exception in the post policy period. The majority of pediatric heart transplants for status 1A exception candidates had a diagnosis of cardiomyopathy. Candidates waiting in Status 1A by exception had lower waiting list mortality than other candidates with the same priority after policy implementation. These were not statistically significant, probably due to small sample size. Additionally, exception candidates were the only group in Status 1A to have a noticeable increase in transplant rate under the new policy.

Summary of discussion:

One Workgroup member commented that the most concerning data for them was "deaths per 100 patient years" and "transplant per 100 patient years". For the deaths per 100 patient years, it showed that exception 1A candidates had the lowest death rate out of all the status 1A candidates pre and post implementation. Similarly, the status 1A exception candidates had higher values post-implementation for "transplant per 100 patient years". Because of this data, Workgroup members continued to be concerned that status 1A exception candidates may be unfairly advantaged under the current allocation system.

2. Review Proposed Solutions and Common Diagnoses

Staff began the discussion into identifying common diagnoses and solutions in relation to the increase in status 1A exceptions.

Summary of discussion:

Overall, Workgroup members supported two solutions: the development of a guidance document for cardiomyopathy candidates, and the creation of a national pediatric heart review board. During the discussion, most members agreed that “cardiomyopathy” was too broad of a category, and could encompass candidates ranging from a teenager ambulating around on a unit, to a neonate intubated in the ICU. One Workgroup member stated that the data suggested a fair number of older pediatric candidates were being approved for status 1A exceptions, despite there possibly being other options available to them. For example, VADs are options in older kids, whereby teenagers may receive a VAD and then be discharged to home rehab. There was group consensus that some cardiomyopathy candidates may not have the same mortality risk as others. In response to this, a Workgroup member was concerned in unintentionally punishing candidates that actually do need an exception. For example, a small child may not be a candidate for a VAD due to their small size. In this way, members agreed that there were situations that may warrant an exception.

In terms of a pediatric heart review board, members generally felt that this was warranted because it could address two main groups: candidates at risk of sudden death and high risk VAD patients. This pediatric national review board (NRB) might also solve the issue of reviewers who are not as familiar with the treatment of pediatric candidates granting exceptions for pediatric candidates by default because their expertise is focused on adult patients. As one member stated, reviewers who mostly work with adult patients do not want to deny a child a heart (even congenital diagnoses that are common can be difficult for reviewers). Members asked for data on the number of exceptions that are approved, however staff did not know the answer at the time. Another benefit of the pediatric NRB would be that this may improve consistency and help standardize the review process.

The advantages of developing additional guidance is that this solution could ensure exceptions are granted or denied based on common criteria, and that this in turn may improve consistency between reviewers. Most members agreed that guidance should first be developed for hypertrophic cardiomyopathy. Guidance on hypertrophic cardiomyopathy would be helpful as there is a high degree of variability in approval for cardiomyopathy under status 1A exceptions.

Next steps:

The Chair will work on developing the initial draft of guidance criteria to circulate among Workgroup members.

3. Review Board Operations

The Workgroup began discussing the operations and technicalities surrounding the operation of a national pediatric heart review board.

Summary of discussion:

Overall, Workgroup members agreed that a national pediatric heart review board was needed in order to address the increase in status 1A exceptions, and that the community would support this solution. The Workgroup considered the following elements needed in order to develop the review board:

- National heart review board composition
- Selection of the Chair
- Term
- Whether alternates are needed
- Number of reviewers
- How to determine conflicts

There was broad support for the inclusion of both a primary and alternate reviewer, because this may enhance and support a quicker response to an exception request. Several Workgroup members were concerned about the term of reviewers, stating that shorter terms will lead to high turn-over rates and therefore less consistency in the reviewing of exceptions. Another Workgroup member stated that if the review process was to remain retrospective, then the group should consider allowing members to contact or call each other if their exception case is denied. To note, this suggestion stems from a breakout session held at this past Region 2 meeting. The last point of discussion revolved around the burden of review. Several members were concerned about a high exception case volume per month, which may add burden on reviewers. In order to determine then how many reviewers to include, and the terms for reviewers, members asked for clarification regarding how many status 1A exceptions are submitted on a monthly basis. Based on the available data at the time, staff responded that there should be a low volume of status 1A exceptions per month, but will look into the data further to confirm.

Next steps:

Staff will send around the presentation from the meetings for those Workgroup members who were unable to attend; Ryan will circulate draft guidance document and summary of today's discussion (within approximately 1 week)

Upcoming Meeting

- October 22, 2019