

OPTN/UNOS Pediatric Transplantation Committee
Meeting Summary
April 14, 2015
Chicago, Illinois

Eileen Brewer, MD, Chair
William Mahle, MD, Vice Chair

Discussions of the full committee on April 14, 2015 are summarized below and will be reflected in the committee's next report to the OPTN/UNOS Board of Directors. Meeting summaries and reports to the Board are available at <http://optn.transplant.hrsa.gov/>.

Committee Projects

1. Establish Pediatric Requirements in the Bylaws

The public comment period recently closed for the "Proposal to Establish Pediatric Training and Experience Requirements in the Bylaws." The Committee received support for this proposal from pediatric specialists, including organizations such as the American Society of Nephrology (ASN), the American Society of Pediatric Nephrology (ASPN), the North American Pediatric Renal Trial and Collaborative Studies, the Studies of Pediatric Liver Transplantation (SPLIT), as well as parents and family members of pediatric transplant patients. Transplant professionals supportive of the proposal voiced appreciation for defining the widely-accepted subspecialty of pediatrics in the Bylaws, as well as for establishing a standard of quality and safety for all pediatric patients. Parents expressed an expectation that these quality and safety standards exist, as well as a desire for all children to receive care from highly-qualified individuals who understand their unique needs.

However, despite the Committee's efforts to build consensus for proposed requirements, many recurrent themes emerged from public comment. These include that the proposal:

- Lacks evidence of a patient safety concern
- Cannot define a pediatric patient as less than 18 years old
- Lacks evidence to support the proposed caseload requirements
- Limits access to transplantation for pediatric patients
- Needs to stratify caseload requirements by age, weight, and other clinical factors.

During its in-person meeting, the Committee considered all public comment feedback and responded to each of themes as follows.

The proposal lacks evidence of a patient safety concern.

The National Organ Transplant Act (NOTA) requires that the OPTN "recognize the differences in health and in organ transplantation issues between children and adults throughout the system and adopt criteria, policies, and procedures that address the unique health care needs of children."¹ Pediatric membership requirements are the most fundamental of criteria the OPTN could adopt to recognize the unique needs of children in transplantation. As early as 1993, the MPSC has sought guidance from the Pediatric

¹ 42 USC Sec. 274 (b)(2)(O).

Committee in establishing pediatric requirements so it could better assess key personnel applications.

While centers not meeting the proposed criteria do not experience poor outcomes immediately post-transplant, long-term patient and graft survival is significantly better at centers that meet criteria. Some have suggested that this justifies excluding the surgeon from any pediatric requirements. However, the primary surgeon is integral to the leadership of a program and shares responsibility with the primary physician and medical director for its long-term outcomes.

The proposal cannot define a pediatric patient as less than 18 years old.

For the purposes of addressing the unique health care needs of children throughout the transplantation system, NOTA states that “the term ‘children’ refers to individuals who are under the age of 18.” Defining a pediatric patient as less than 18 years old is also consistent with CMS and the American Academy of Pediatrics. Any alternative to the definition of a pediatric patient as less than 18 years old in the Bylaws could have implications for allocation policy, where currently most candidates registered prior to 18 years old receive pediatric priority.

The proposal lacks evidence to support the proposed caseload requirements.

Many have asked the Committee to produce evidence to support the proposed case volume requirements for the primary pediatric surgeon. As with all OPTN membership requirements involving case volume, the proposed case volume requirements were developed through clinical consensus. None of the OPTN membership requirements, alone, are predictive of good program outcomes. Many factors contribute to the success of a program. However, qualified key personnel are important contributors to a program’s success, and case volume is the most basic way a surgeon demonstrates requisite experience.

The purpose of these requirements is to establish criteria for membership; therefore, the Committee does not have to demonstrate improved outcomes associated with these requirements. However, in an effort to build consensus, the Committee investigated outcomes data.² A descriptive analysis of OPTN data showed significantly better unadjusted Kaplan-Meier graft and patient survival for pediatric transplants performed at high versus low volume kidney, liver, and heart programs from 1995-2010. High volume programs were determined using the proposed case volume requirements for each organ, i.e., at least 12 kidney transplants, 18 liver transplants, 8 heart transplants, and 4 lung transplants. While high-volume lung transplant programs also experienced better patient survival outcomes, the difference was not statistically significant. Additionally, adjusted analyses that were performed independently by UNOS showed that as a group, centers performing <18 pediatric liver transplants during 2000-2010 had an increased risk of graft loss and death within 5 years (i.e., worse outcomes) as compared to centers performing 18+ pediatric liver transplants during that period; and centers that performed <12 pediatric kidney transplants during 2000-2010 had an increased risk of graft loss and death within 5 years (i.e., worse outcomes) as compared to centers that performed 12+ pediatric kidney transplants during that period.

² These analyses are included in the public comment proposal and briefing paper and are also available upon request.

The proposal limits access to transplantation for pediatric patients.

In response to feedback from the Regions, the Committee made major comprises in the development of these proposed Bylaws in the interest of access to transplantation for pediatric patients. The resulting proposal better balances the competing interests of quality of care, including patient safety, and access to transplantation for pediatric candidates. In fact, from January 1, 2005 through July 31, 2014, 97.7% of pediatric transplants were performed at centers that would have met the proposed pediatric volume criteria. Again, because of the limitations of OPTN data, center volume is being used as a proxy for primary surgeon volume. A low volume center could still be approved for a pediatric component so long as a surgeon that has performed the required number of pediatric surgeries over the history of his or her career can serve as key personnel. Programs may also take advantage of a 24-month conditional pathway to establish a new pediatric component or accommodate a change in key personnel.

The Committee continues to receive requests for an exception that would allow programs without a pediatric component to perform a pediatric transplant in an emergency, such as acute fulminant liver failure. The Committee has thoroughly considered and decided against proposing such an exception, which would represent a departure from the current standard that OPTN members must fully meet program and program component requirements in order to perform transplants. In these exceedingly rare instances, patients can be safely transported to a qualified pediatric component program.

The proposal needs to stratify caseload requirements by age, weight, and other clinical factors.

At the Regional Meetings in the fall of 2013, the Committee presented initial requirements that were stratified by age, weight, and other relevant clinical factors in an effort to build consensus prior to public comment. Among the initial requirements, the primary pediatric kidney surgeon must have performed 6 transplants in patients weighing 20 kilograms or less at time of transplant, and the primary pediatric liver surgeon must have performed 9 transplants in patients less than 12 years old and 5 technical variants, including split, reduced, or living donor liver transplants. This experience had to be achieved over a recent five year period. As mentioned above, the Committee received overwhelming feedback to modify the requirements to preserve access to transplantation for pediatric patients. In response, the Committee eliminated stratifications from the pediatric caseload requirements and proposed that the requisite surgeries could be performed over an entire career, so long as the surgeon demonstrates currency of experience as currently defined in the Bylaws. Informed by the development process, the Committee knows it cannot achieve consensus for stratified caseload requirements and recognizes its responsibility to balance quality of care with access to transplantation for pediatric patients.

After carefully considering feedback received during public comment, the Committee voted to approve the proposed Bylaws without modification (16-Support, 0-Oppose, 0-Abstain). The Committee believes this proposal fulfills the long-standing need to establish pediatric requirements in the OPTN/UNOS Bylaws, while appropriately balancing the competing interests of quality of care, including patient safety, and access to transplantation for pediatric patients.

2. Evaluation of Current Lung Allocation Policy for 0-11 Year Old Candidates

At its recent in-person meeting, the Committee also reviewed monitoring data on pediatric lung allocation policy. Implemented on September 12, 2010, this policy established broader sharing of 0-11 year old deceased donor lungs, as well as a simple priority system for 0-11 year old candidates. The Committee learned that:

- Following policy implementation, waiting list death and transplant rates increased significantly for pediatric candidates ages 6-11 and 12-17.
- Most recipients received lung transplants from donors in their same age group.
- Following policy implementation, patient survival within two years of transplant among pediatric recipients was not adversely affected.

Several Committee members expressed concern at the increased waiting list death rate post-implementation. The Statistician explained that the actual number of deaths on the waiting list decreased post-policy, but so did the time candidates spent on the waiting list, which contributes to an increased rate. The Chair suggested that increased access to transplantation, the intended goal of the policy, also contributes to shorter waiting times and increased transplant rates. One Committee member, a pediatric pulmonologist, shared that the demographic of the waiting list at his program has changed since this policy was implemented. He estimated that over half of the pediatric patients on the waiting list at his center are on extracorporeal membrane oxygenation (ECMO) or mechanical ventilation, compared to a quarter of patients six years ago. These patients are at higher risk of poor waiting list outcomes.

The Committee continues to collaborate with the Thoracic Organ Transplantation Committee on its proposed pediatric lung allocation policy, which will be released for public comment in August, 2015. The Committee hopes the new broader sharing sequence for child and adolescent donor lungs included in this proposal will further contribute to improved outcomes for pediatric lung candidates.

3. Evaluation of ABO-Incompatible Pediatric Heart Policy

The Committee continues to monitor ABO-incompatible pediatric heart policy, most recently at its in-person meeting on April 14, 2015. According to the currently implemented policy, Status 1A and 1B candidates less than two years old at listing who meet the eligibility requirements set forth in Policy 5.3.C, including in utero candidates for whom blood type is unknown, may accept a heart from a donor of any blood type. The Committee found that:

- The majority of candidates willing to accept an ABO-incompatible heart were Status 1A infants less than one year old at listing.
- Among candidates willing to receive an ABO-incompatible donor heart, the majority actually received an ABO-identical heart.
- The vast majority of ABO-incompatible transplants were performed in Status 1A recipients less than one year old at both listing and transplant.
- Results of ABO-incompatible heart transplants, performed mostly in pediatric patients less than one year old, suggest comparable patient survival with ABO-identical or compatible transplants.
- Of recipients of ABO-incompatible hearts who died within one year of transplant, titer values prior to time of death were low (less than 1:4).

Of the 891 registrations less than two years old at listing that met the eligibility requirements, 524 (58.8%) were not willing to accept an incompatible blood type at time of listing. The Vice Chair confirmed that the OPTN does not collect data on the listing titers for these candidates to understand if more are candidates are clinically-eligible than are willing.

The Committee will continue to monitor the new ABO-incompatible heart policy passed by the Board in June, 2014. One Committee member, a pediatric cardiologist, said he is anxious for implementation and anticipates better organ offers and post-transplant outcomes. The implementation of this policy is pending programming, which is scheduled to begin in October, 2015.

4. Evaluation of Open Variance for Segmental Liver Transplantation

At the recommendation of the Board of Directors, the Committee tabled discussion on their proposed split liver policy in favor of monitoring data from OPOs and regions participating in the Board-approved segmental liver variance. Since 2012, the Committee has routinely reviewed match run data to identify the number of pediatric candidates prioritized above the second recipients of split livers but who did not receive the livers on the original match run within the OPO or region. The Committee most recently reviewed this data at its in-person meeting on April 14, 2015.

From the beginning of the variance through December 31, 2014, 57 deceased donors were transplanted as splits at four OPOs and one Region. After limiting the analysis to split liver transplants where one segment was transplanted into an adult recipient and the other into a pediatric recipient at the same or an affiliated center, there were 24 donors. An examination of the match run data for these 24 donors found the following:

- For 20 donors, the pediatric candidate was the index patient and allocation of the remaining segment appeared to follow *Policy 9.6.A: Segmental Transplant and Allocation of Liver Segments*.
- For the remaining 4 donors, where the adult candidate was the index patient, only one remaining segment appeared to follow *Policy 9.8.A: Open Variance for Segmental Liver Transplantation*. In this instance, 7 pediatric candidates were bypassed above the pediatric acceptor. Of these, six were not waiting at the same or an affiliated center, and one required a multi-organ transplant at the same center.

Only one of the split liver transplants performed between the implementation dates of the variance and December 31, 2014 has been allocated using Policy 9.8.A. The Committee finds that a voluntary variance is not having the intended outcome and will consider this summer whether to continue work on this project under the new Strategic Plan.

Other Significant Items

5. Strategic Planning

The UNOS Policy Director presented the proposed 2015-2018 OPTN Strategic Plan. One Committee member expressed concern that the percent of resources allocated to patient safety may not be adequate due to factors beyond the OPTN's control, for example changes in federal regulation or insurer expectations. Another wondered if other organizations could have more influence than the OPTN on increasing the number of transplants, including those that promote organ donation. Therefore, it is essential to partner with such organizations on initiatives that are beyond the scope of the OPTN, or

that require coordination among federal agencies or individual payers. The Chair asked Committee members to brainstorm new projects that meet the goals of increasing the number of pediatric transplants or improve access for pediatric patients. The Committee will consider ideas for new projects this summer.

6. Kidney Allocation System (KAS) Update

The Kidney Transplantation Committee Chair presented monitoring data from the first three months of KAS implementation. The Committee expressed relief that the pediatric transplant rates are recovering from an anticipated initial decrease. In response to a question from a Committee member, the Kidney Committee Chair shared that there has not been a significant change in the recovery or discard rates for high KDPI kidneys post-implementation, but that his Committee continues to monitor this.

7. Examination of Inactive Pediatric Registrations

At its meeting on August 26, 2014, the Committee reviewed an analysis of inactive pediatric kidney registrations in regard to length of inactivity, as part the on-going evaluation of Kidney Share 35 policy. The Committee expressed concerns about the high percentage of pediatric kidney registrations who were inactive and requested that the analysis be expanded to pediatric registrations waiting for heart, lung, and liver transplants. After reviewing this analysis, the Committee discussed the reasons for inactivity, the impact of inactivity on waiting time and allocation, and waiting list management. At this time, the Committee has not purposed action regarding patient inactivity on the waiting list.

8. Length of Committee Terms

At the request of the Policy Oversight Committee, the Vice Chair asked the Pediatric Transplantation Committee to provide its recommendation on extending the length of terms for all OPTN Committees to three years. Members identified several benefits to three-year terms, including retaining historical knowledge on the Committee and increased productivity. However, they emphasized the importance of communicating that commitment early in the nomination process. Members expressed concern for adequately representing all the constituencies the Committee is responsible for, including surgeons and physicians within each organ specialty, with only eighteen positions. They also want to ensure opportunity for new individuals to participate in OPTN Committees. Members voted to support extending service terms to three years but added that the Pediatric Transplantation Committee requests additional members to accommodate longer terms while adequately representing all essential constituencies (16-Support, 0-Oppose, 0-Abstain).

Upcoming Meeting

- June 17, 2015