OPTN/UNOS Pediatric Transplantation Committee
Meeting Minutes
April 19, 2019
Conference Call

William Mahle, M.D., Chair
George Mazariegos, M.D., Vice-Chair

Introduction

The Pediatric Transplantation Committee met in Chicago, IL on April 1, 2018 to discuss the following agenda items:

1. Discussion – Concept Document on Improving OPTN/UNOS Committee Structure
2. Policy Oversight Committee Update
3. Research updates
4. Project Discussion – Transition Guidance
5. Project Discussions
6. Update on Ad-hoc Geography Committee
7. Joint Post-implementation Review – Pediatric Heart Allocation
8. Our Journey App
9. Modifications to SRTR Annual Report
10. Committee Member Recognition

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

1. Discussion – Concept Document on Improving OPTN/UNOS Committee Structure

The Committee regrouped on the *Improving OPTN/UNOS Committee Structure* concept document and discussed the themes that emerged from the pediatric transplant community.

Summary of discussion:

UNOS staff initiated the discussion by describing what OPTN concepts documents are/are not, and the goals of sharing concept documents with the community. This approach was utilized to share an idea with the goal of:

- Increase participation by the community and SMEs
- Improve minority participation and diversity in perspective
- Improve connections between the Board and committees

Public comment was sought on the concept from January 23, 2018 to March 23, 2018. The Committee previously submitted a formal response to the concept following their March 2018 conference call (see minutes previous minutes). In total, 157 comments were received from nearly all OPTN Committees, professional societies and numerous individuals.

While there was wide support for the overall goals of the concept document, the feedback from the Committee and wider pediatric community was one of concern:

- Diminishing the voice of some committees by transitioning to expert councils
- Lack of a clear path for policy development
- Proposed subject committees are ill-equipped to consider depth and complexity of pediatric considerations in transplant policy
- Inadequately addresses NOTA mandate for policy to "…address the unique healthcare needs of children."

The Chair thanked UNOS staff for the summary and invited UNOS Chief Executive Officer (UNOS CEO) to share his comments. Members candidly shared key concerns on the proposal. Leading among these were 1) the potential loss of regional representatives as a result of transitioning to an expert council, and 2) the loss of ability to sponsor proposals. One member verbalized that a similar effort was undertaken with a national transplant society and the goals of the transition were not realized. This experience may be shaping the perspective of the pediatric transplant community toward the OPTN concept paper. Members did verbalize the concept document served to energize the pediatric transplant community to be further engaged with the OPTN.

UNOS CEO shared that a proposal to amend the OPTN Bylaws that would transition existing committees to expert councils is not planned for the August 2018 public comment period. UNOS is diligently considering a format for expert councils and tools to use in a pilot, and the results of the pilot program would be carefully considered before any pursuit of a formal proposal. The CEO asked the Committee to carefully consider their charge; this along with the goals of the proposal will be instrumental to moving forward.

The Chair thanked the CEO and the members for their thoughtful comments and segued to the next topic on the agenda.

Next steps:

The Committee will remain engaged on this topic and asked to hear an update on the experience trialing expert councils.

2. Policy Oversight Committee Update

The Vice Chair shared an update on recent POC discussions.

Summary of discussion:

The Vice Chair continues to serve with the OPTN/UNOS Policy Oversight Committee (POC). The purpose of this role is to examine proposals from other committees, as well as represent the Committee during project discussions.

The POC has reviewed a total of seven new committee projects since October 2017. Projects that were in alignment with Goal Two of the OPTN Strategic Plan and a multi-organ allocation project were reviewed and prioritized. The POC also reviewed and approved 12 committee proposals for public comment. This public comment period will run from January 23, 2018 to March 23, 2018. The Vice Chare noted that the Committee had no proposals in this batch.

The Vice Chair noted that the portfolio of active committee projects will reviewed during the POC meeting in May 2018. The purpose of this review is to assess if appropriate progress is being made and if the projects continue to be the most appropriate use of resources. The Committee’s project to *Reduce Pediatric Liver Waiting List Mortality* will be discussed when the POC reviews all active projects.

UNOS staff profiled the current proposal alignment with the OPTN Strategic Goals. The three projects involving the Committee (either as the sponsoring or collaborating committee) were highlighted. One member commented that the number of active projects involving the Pediatric Committee is the highest in memory.

Next steps:

The Vice Chair will continue to represent the Committee on the POC through June 2018. The leadership transition in July 2018 will result in a new vice chair/representative to POC. This individual will be announced at the conclusion of the meeting.

3. Research updates

UNOS staff provided updates on two earlier data requests from the Committee.

Summary of discussion:

UNOS staff shared updates on two earlier data requests:

- Transition of Pediatric Recipients to Adult Providers Survey Results
- Transition of Pediatric Recipients to Adult Providers Comparison of Post-Transplant Events for Recipients Followed and Lost to Follow-up

Transition of Pediatric Recipients to Adult Providers Survey Results

The Transition Working Group sent out a survey to all transplant administrators at active liver and kidney programs that performed at least one pediatric transplant between 2012 and 2016 regarding challenges to and effective practices for transitioning pediatric recipients to adult practitioners after they reach adulthood.

There were 148 complete responses to the survey, resulting in a response rate of 72%. UNOS staff reported on several metrics from the survey. These included:

- Survey Responses by Organ
- Survey Responses by Program Size
- Survey Responses by Program Type
- Survey Responses by Program Follow-up Rate

**See Policy Evaluation Report for details.*

At the conclusion of the presentation, UNOS staff summarized that there was an association between program size and program type, with large volume programs tending to be stand-alone pediatric programs, and comparisons across categories for these groups yielded similar results. A similar association was seen between program type and lost to follow-up (LTFU) rate, with programs with below average LTFU rates tending to be stand-alone pediatric programs, though this relationship was not as consistent.

Programs serving only pediatric patients were more likely to have a formalized transition procedure in place than those programs partnered with adult transplant providers. These processes included requiring transition or transfer at a specific age and employing transition specialist or social workers to aid in transition. Combined adult and pediatric programs were less likely to have such procedures, likely due to the connections they have within their center. These programs estimated they transfer the majority of their patients to an adult provider within their center.

Programs with below average LTFU rates seem to serve a population of patients that are more challenging to follow. These programs were more likely to face challenges finding adult providers to accept their patients due to geographic or payer related restrictions. Additionally, these programs were more likely to transfer patients to adult providers not affiliated with a transplant program, and to cite difficulty in obtaining follow-up information from these providers.

Transition of Pediatric Recipients to Adult Providers Comparison of Post-Transplant Events for Recipients Followed and Lost to Follow-up

On April 21, 2017, the Pediatric Committee was presented data on linkage results for heart, kidney, and liver recipients who were lost to follow-up. The Committee was concerned with the high proportion of these lost recipients who had died, been relisted at another center, or, for kidney recipients, returned to dialysis. The committee wanted to compare these rates to those seen for kidney and liver recipients who were not lost to follow-up.

UNOS staff reported on several metrics from the request. These included:

- Lost to follow-up rates for kidney transplant recipients 2000-2010 by age at transplant
- Lost to follow-up rates for liver transplant recipients 2000-2010 by age at transplant
- Linkage results recipients lost to follow-up within 10 years of transplant 2000-2010 by organ and age at transplant
- Kidney recipient deaths by status (lost or followed)
- Kidney recipient re-listings by status
- Kidney recipient return to dialysis by status
- Liver recipient deaths by status
- Liver recipient re-listings by status

**See Report for details*

At the conclusion of the presentation, UNOS staff reported that followed recipients were as or more likely to die, be relisted, or return to dialysis than lost recipients, regardless of organ or age at transplant.

The Chair thanked UNOS staff for the presentation and opened the floor for discussion. Members shared several comments:

- The data helps illustrate the existence of a LTFU problem *after* transition.
- Close to 80% of the pediatric kidney transplant performed in the U.S. were performed at programs that fell into the small volume classification.
- There appeared to be issues with the recipient hand-off and communication between the index transplant program and receiving program/provider.
- Payers indeed do influence where a recipient is transitioned.
- Perhaps the inability to complete all of the required fields on transplant recipient follow-up forms (TRFs) and the desire to not be “dinged” for data submission noncompliance motivates some programs to designate recipients as lost to follow-up.
- Some interest in developing a profile the programs with the best LTFU.
- One innovative solution would be to have recipients report their own data.
- Interest to see if there is a way to assess for successful transition between programs, but recipient factors later that contributed to LTFU.
- It is reasonable to conclude that if a recipient is not deceased, relisted, or on dialysis (kidney) then the recipient is in good health and under the care of a provider somewhere (as evidenced by the need for continued immunosuppression prescriptions).
- Was there some potential for incentivizing form completion?
- Was there potential to consider follow-up data submission every two years?

Members then asked UNOS staff for future analysis including:

- Socioeconomic data for recipients who are lost to follow-up.

Members were reassured by the presentation that the majority of the recipients designated as LTFU are not 1) re-listed for transplant, 2) reported as deceased, or 3) dialysis is re-initiated.

This would appear to “de-bunk” the assumption that recipients who are not followed by providers have worse post-transplant outcomes than those recipients who are followed by providers. Members were also in agreement that the survey data indirectly supports that the risk LFTU designation is higher for recipients originating in programs that have certain transition practices. The Committee acknowledged that correlation would require assessments of clinical data across a study population and a control group; this would be very challenging. At the conclusion of the discussion, members felt it may be time to examine the data collection forms for recipient follow-up to assess if the clinical data collected serves a bona-fide purpose.

The members thanked UNOS staff for the comprehensive work on the survey and related data reports.

4. Project Discussion – Transition Guidance

The Committee received an update on the project status.

Summary of discussion:

The Subcommittee Chair shared an update on the progress to-date. She thanked members for their contributions and recognized the involvement of one member of the OPTN/UNOS Minority Affairs Committee. Two recent conference calls in January and March 2018 were productive to finalize the survey and solicit contributions to the document. To-date, the document is approximately 60% complete. There are remaining areas of the guidance to develop and the survey content will be very helpful in these areas. Outreach on this project is planned to OPTN and non-OPTN stakeholders in the coming months. The goal of this outreach is to share the concepts of the guidance and encourage feedback during the public comment period.

One of the questions posed to the Committee was, should the scope of the document be expanded to discuss the principles of transition? Members verbalized this was an important consideration. Though the guidance documents usually intended for use by OPTN members, they are often read by a much wider audience. As a result, it may be prudent to explain the concept of transition.

The Chair thanked the Subcommittee Chair for the update and opened the floor for questions. One member commented, the transplant outcomes shared with patients/caregivers may actually be a bit better that what is reflected in the OPTN data. Other members verbalized agreement with this sentiment and felt this emphasized the need for guidance on transition that will help improve the accuracy of recipient and graft survival data. Another member suggested the document include content to help adult providers “perfect the catch” when receiving recipients from pediatric transplant programs. Members also inquired about the role of supporting literature in OPTN guidance documents. UNOS staff responded that supporting literature is always useful and the guidance document under construction uses several papers for this purpose.

Members asked if there was potential to publish the data identified by the Committee. UNOS staff responded this was certainly within the purview of the Committee. UNOS staff then shared that future data analyses may include other data sources e.g.: health insurance companies (e.g.: are recipients getting immunosuppression prescriptions filled). These discussions are in the early stages.

UNOS staff thanked the Committee for their insightful comments and profiled the timeline to complete the guidance. The Subcommittee will need to meet by conference call the week of June 4, 2018 to discuss and decide whether to hand-off to the Committee. The Committee would need to vote during their call on June 20, 2018 whether to solicit public comment. The Subcommittee Chair acknowledged the timeline was tight, but expressed confidence the goals would be met.

Next steps:

The Transition Subcommittee will consider the results of these data analyses and comments from Committee members when wrapping on the guidance document.

5. Project Discussions

Committee members provided updates on two projects where the Committee is either the sponsoring or key collaborating committee.

Summary of discussion:

Kidney and liver transplant subject matter experts (SMEs) are actively engaged in two project with impact on pediatric transplant patients.

Kidney-Pediatric Working Group

UNOS staff profiled a short summary of the progress to-date. Two year Kidney Allocation System (KAS) analysis reflected that:

- Pediatric kidney candidates have decreased access to deceased donor kidneys as compared to adult kidney candidates.
- Increase in wait list mortality in 1-5 year old candidates post-KAS.
- The median Kidney Donor Profile Index (KDPI) and median donor age of kidneys allocated to pediatric candidates has decreased. However, there is concern about the decrease in the number of kidneys from deceased donors < 18 years old allocated to pediatric candidates.
- Median waiting time for kidneys from deceased donors < 18 years old is less than one year.
- Improvement in graft survival one year post-KAS.

Also reported was:

- Percentage of pediatric kidney transplants from living donors has decreased to ~40%. Prior years were over 50%.

Additional data to consider:

- Preemptive transplant rates for pediatric kidney candidates pre-KAS and post-KAS.
- More granularity on graft survival to include donor type, ABO, age, ethnicity, etc…
- More exploration of delayed graft function for recipients < 10 years old.

Members verbalized some concern that the pediatric perspective appeared to be an “add-on” to the project, rather than an equal partner. Some of this sentiment comes from the manner in which the initial call was coordinated and the appearance the adult transplant colleagues on the Working Group are ambivalent that issues in pediatric kidney transplant access exist. Members verbalized the desire for a greater sense of buy-in to the project with all members of the Working Group. Members shared ideas of identify co-chairs (one from Kidney and one from Pediatric), mutual input on agenda planning, and early sharing of meeting materials.

The Chair thanked the members for their input and asked to be kept informed of future work on the project. UNOS staff verbalized their commitment to help with engagement concerns and to foster an environment that allows mutual input from all stakeholders on this project. Members ask

Next steps:

Working Group participants were asked to keep the Committee informed on the project.

Pediatric-Liver Working Group

UNOS staff shared progress to-date regarding the Pediatric sponsored project to *Reduce Pediatric Liver Waiting List Mortality*. The OPTN/UNOS Policy Oversight Committee (POC) discussed the roles of “co-sponsoring” and “collaborating” with regard to projects with more than one key stakeholder committee. The POC supported the model of close collaboration between the Pediatric and Liver/Intestine Transplantation Committees for this project. As a result, the Vice Chair engaged the Liver Committee in March 2018. The Liver Committee expressed keen interest in the project, as well as a potential project to revise the Pediatric End-stage Liver Disease (PELD) score. Five individuals from the Liver Committee volunteered to participate on the Working Group.

At the conclusion of the discussion, additional Committee members volunteered to help with this project. The Vice Chair commented that additional input would be critical to the success of this effort. This would include collaboration with other OPTN committees, as well as transplant societies/groups.

Next steps:

UNOS staff will coordinate scheduling of conference calls through the summer of 2018.

6. Update on Ad-hoc Geography Committee

UNOS staff shared an update on a Board directive to examine geography as a component of organ distribution.

Summary of discussion:

In December 2017, the Board of Directors formed an Ad-Hoc Geography Committee to examine geography as a component of organ distribution. The charge of the Geography Committee is not to change any existing policy, but to:

- Establish defined guiding principles for the use of geographic constraints in organ allocation
- Review and recommend frameworks/models for incorporating geographic principles into allocation policies
- Identify uniform concepts for organ specific allocation policies in light of the requirements of the OPTN Final Rule

UNOS staff then profiled the composition of the Geography Committee. This committee will:

- Discuss and establish defined organ distribution principles for the use in geographic constraints for all organs
- Review and recommend organ distribution frameworks/models using defined principles
- Identify concepts that meet the requirements of the Final Rule
- Report recommended organ distribution frameworks to Executive Committee and Board of Directors

It is outside the scope of this committee to:

- Tell other committees what to do
- Change allocation policies
- Send proposals for public comment
- Work in isolation

The Geography Committee will be provided a report to the Board in June 2018 that will describe:

- Development of Ad Hoc Committee

- Journey of Committee discussions
- Principles of Organ Distribution
- Thematic Models that Align with Principles

The Chair thanked UNOS staff for the update and opened the floor for discussions. Members acknowledged the importance of the discussion, but noted that no representatives on the Geography Committee specifically from the pediatric transplant community. The geography question is very impactful in pediatric transplantation as pediatric transplant teams often travel much farther to recover organs (or organs are transported farther) than their adult colleagues. UNOS staff shared that any future proposals would be subject to public comment and this was planned for the fall of 2018.

Next steps:

The Committee will remain engaged on this topic and will be watchful for a future public comment proposal.

7. Joint Post-implementation Review – Pediatric Heart Allocation

The Pediatric and Thoracic Committees jointly reviewed data following the March 2016 implementation of the Pediatric Heart Allocation policy.

Summary of discussion:

A modification to heart policy redefining pediatric Status 1A and Status 1B criteria went into effect on March 22, 2016. The goal of this change was to improve waiting list mortality for pediatric heart candidates by creating an allocation system more dependent upon candidates' medical urgency than their waiting time.

The Pediatric and Thoracic Committees reviewed data from the first twelve months of the new policy in October 2017. Pediatric death rates on the heart waiting list did not change after policy implementation. Committee members noted a marked increase in the use of exceptions to justify placing candidates in Status 1A, particularly among those diagnosed with cardiomyopathy. These same candidates saw increased access to transplants for certain ages, while no such change was seen for candidates with other diagnoses. Furthermore, there was substantial regional variation in the percentage of transplants going to candidates in Status 1A by exception. The Committees hypothesized the increase in Status 1A exceptions could be attributable to physicians seeking exceptions for their candidates with cardiomyopathy who had formerly been waiting in Status 1A, and these requests being granted by regional review boards composed almost entirely of adult practitioners. They also acknowledged the increased use of exceptions could mean the new Status 1A criteria are too restrictive and exclude a group of medically urgent patients.

UNOS staff reported on several metrics to assess the effectiveness of the proposal. These included:

- Waiting list additions by age group (<1, 1-5, 6-11, 12-17) and status criteria
- Waiting list death rates by:
    - Age group (<1, 1-5, 6-11, 12-17) and status criteria
    - Age group (<1, 1-5, 6-11, 12-17) and diagnosis category
- Waiting list transplant rates by:
    - Age group (<1, 1-5, 6-11, 12-17) and status criteria
    - Age group (<1, 1-5, 6-11, 12-17) and diagnosis category
- Transplants by:
    - Recipient age group (<1, 1-5, 6-11, 12-17) and status criteria
    - Recipient age group (<1, 1-5, 6-11, 12-17) and diagnosis category

**See Report for details.*

At the conclusion of the presentation UNOS staff summarized that:

- There has been a sustained decrease in the proportion of waiting list additions and transplant recipients in Status 1A in the year and a half since the changes to pediatric heart criteria were implemented. However, there has been very little change in waiting list mortality for the sickest candidates.
- Use of Status 1A exceptions remained higher than pre-policy levels. A higher proportion of Status 1A transplants went to recipients with exceptions after implementation. Use Status 1A exceptions varied across regions post-policy. Candidates waiting in Status 1A by exception had lower waiting list mortality than other candidates with the same priority after policy implementation. Additionally, exception candidates were the only group in Status 1A to have a significant increase in transplant rate under the new criteria.
- A higher proportion of transplant recipients diagnosed with cardiomyopathy were in Status 1A by exception after implementation. This change was not observed for recipients diagnosed with CHD. Waiting list mortality for candidates with cardiomyopathy in Status 1A was not different from that of candidates in 1B both before and after policy implementation, and candidates waiting in Status 1A had significantly higher transplant rates than those in 1B.

The Chair thanked UNOS staff for the informative presentation and opened the floor for questions. Members of both committees expressed gratitude for the presentation and the opportunity to reflect jointly on the information. Members discussed whether training for review board members, formal guidance for review boards or exception submissions, or a National Heart Review Board may be viable solutions to the problem. Members also discussed the need for additional data to understand the problem(s). This may include:

- waitlist mortality across age, diagnosis, and treatments
- waiting times by region, age, diagnosis, and medical urgency
- waiting list removals by removal codes
- profile of exception applications submitted

One member asked the committees carefully consider the diverse audiences that would consume the data. Parents/caregivers of transplant patients often consider these data to become better informed on the journey that is pediatric heart transplantation. One area that would be helpful is clear understanding of how congenital heart disease is defined in the system. This, along with the other criteria, would help families/caregivers understand the rationale behind the priority.

<u>Next steps:</u>

The Pediatric and Thoracic Committees expressed interested in a future project to address any gaps in the proposal. Additional discussions will follow in the months ahead with the respective committees.

8. Our Journey App

A member was invited to profile a mobile app that she generated in collaboration with her local pediatric transplant hospital.

<u>Summary of discussion:</u>

A Committee member shared that she has been working for the last several months with The Emily Center at Phoenix Children’s Hospital to develop free apps for Apple and Android mobile devices. The goal is for these apps to help serve as a “virtual advocate” that can be used before, during, and after encounters with the transplant team. Specifically, these apps are designed to help families/caregivers of children needing organ transplants with the lengthy and complex journey of appointments, diagnostic tests, clinical language spoken, etc… At present, modules are available for heart and liver transplantation.

The committee member concluded the presentation, noting the app is available in English and Spanish, and future organ-specific modules will be released soon. The Chair thanked the committee member for her innovative work that will certainly help other transplant families. Members similarly applauded the work and were eager to learn more. Some members commented that resource constraints at some institutions may limit the ability to derive their own apps. Further, they were interested in future discussions that may allow these apps to be access by families at other transplant hospitals.

9. Modifications to SRTR Annual Report

Representatives from the SRTR profiled changes to their Annual Report for pediatric transplants.

Summary of discussion:

In April 2017, the Committee expressed interest in providing feedback on the SRTR's Annual Data Report. Feedback from the Committee was that some elements reported by the SRTR are less relevant to pediatric transplantation. SRTR representatives recently invited members of the Committee to review elements of the Annual Data Report and share recommendations for changes. Based on the feedback, several changes were made to more appropriately reflect pediatric specific data. SRTR representatives were very appreciative for the collaboration with the Committee. They requested members continue to be engaged to enhance the future versions of the report. Future discussions may include "re-fitting" the Pediatric End-stage Liver Disease (PELD) score.

10. Committee Member Recognition

The Vice Chair recognized two outgoing members of the Committee.

- The Chair has shown great leadership and dedication in the position. He thanked members for their extremely hard work and dedication to advancing the field of pediatric transplantation. He also thanked UNOS staff members who integral to the Committee's success. The Chair was presented with a plaque as a token of appreciation. The Chair will be remaining with the Committee as Immediate Past-chair for one year.
- An additional At Large committee member was acknowledged as a great contributor to the Committee in her term. She has taken a position outside of her institution in another area of clinical practice.

UNOS staff announced that the Vice Chair. would be transitioning from Vice Chair to Chair. Another committee member who has served on the committee since 2015 was selected by the OPTN Vice President to serve as Vice Chair. Both individuals will serve two-year terms beginning in July 2018.

With no further business to discuss, the meeting was adjourned.

Upcoming Meeting

- June 20, 2018 4-5 PM Eastern (conference call)