

OPTN Pediatric Transplantation Committee

Meeting Summary

March 15, 2023

Conference Call

Emily Perito, MD, Chair

Rachel Engen, MD, Vice Chair

Introduction

The OPTN Pediatric Transplantation Committee (the Committee) met via Citrix GoToMeeting teleconference, on 03/15/23 to discuss the following agenda items:

1. Welcome and Announcements
2. Information Concerning OPTN Heart Transplantation Committee's ABO incompatible (ABOi) Public Comment Proposal
3. Pediatric Bylaw Change: 2-Year Monitoring Report
4. Recap and Update: Monitoring Report Reference Documents

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

1. Welcome and Announcements

Committee leadership welcomed the Committee and gave a few announcements.

2. Information Concerning OPTN Heart Transplantation Committee's ABOi Public Comment Proposal

Staff provided an overview of the plan to implement a portion of the Heart Committee's Pediatric ABOi proposal.

Presentation Summary:

Staff summarized the information that was provided in an email to the Pediatric Committee on March 14, 2023, and included the following:

- An email was received from a transplant program inquiring about whether the program could request an exception for a status 1A pediatric patient who is now greater than two years old and who would qualify for ABOi heart offers under the proposed policy. The transplant program asked if the OPTN could identify a remedy that would allow their critically ill patient to receive the benefit of the policy proposal immediately. Unfortunately, there is no exception pathway available for this type of request.
- OPTN leadership considered the options and determined that because the proposal has been receiving broad support during public comment, the OPTN Executive Committee has the authority to approve the proposal.
- The OPTN Executive Committee will review the Heart Committee's proposal and consider approval of the policy language that expands the current heart and heart/lung ABOi eligibility to include candidates registered before the age of 18 years if they meet titer requirements and report titers every 30 days, to be effective immediately. This would allow patients to immediately qualify under the new policy. The Executive Committee would also consider an additional resolution that would allow and advise the Heart Committee to continue with the

remaining parts of the proposal, including considering incorporating additional post-public comment changes, and bring the final portion of the proposal back to the Board during the June 2023 meeting as originally anticipated.

- If approved, the policy language will be effective immediately. IT programming will not be immediately available, so the OPTN Contractor has identified an alternate method to enable an immediate implementation.
 - Transplant program would work with the OPTN Contractor to create a second waiting list registration for the candidate with a date of birth indicating the patient is just older than one year old. This secondary listing would require the transplant program to enter the isohemagglutinin titer information required by policy and would determine ABOi offer eligibility according to the policy requirements.
 - The OPTN computer system would then appropriately include and rank the candidate in the correct sequence on subsequent deceased donor match runs, providing qualifying candidates with ABOi offers.

Summary of Discussion:

Staff noted that this change will not be applied to one individual, but to all patients that meet the criteria within the proposed policy language.

A member expressed support for this proposal but had concerns about the manual process for requesting an exception. She added that the process to create two different registrations seems confusing.

A member asked if this process has been used in the past. Staff responded that expedited consideration and implementation has been done previously. Staff added it was not the routine policy process but may be done in certain urgent circumstances as determined by the Executive Committee and Board of Directors.

A member asked how long implementation of the full proposal would take once the OPTN Board of Directors approves the proposal in June 2023. Staff responded that policy implementations usually occur within one year of Board approval unless a policy includes new data collection which requires approval by the Office of Management and Budget (OMB). A member added that this policy change should be a high priority so members and the OPTN do not have to utilize the interim manual process for an extended period of time.

A member expressed concern about setting a precedent for other policy proposals. She added that this proposal affects a smaller population of patients, unlike the liver and kidney population. Those patients could also make an argument that they would benefit from expedited implementation of policy changes. Staff responded that it was an excellent point and stated that OPTN leadership always weigh the risk and benefit when deciding on alternative pathways. Staff further added that the OPTN Heart Committee supports this approach and is trying to do the best thing for this patient population.

A member expressed support for this proposal since it will open opportunities for more patients and could also benefit transplant programs.

Another member supported this effort on behalf of recipient parents and asked if this was an opportunity to develop a standard process for the rapid deployment of policies.

The Committee had no further comments or questions.

3. Pediatric Bylaw Change: 2-Year Monitoring Report

Research staff presented the 2-year monitoring report for the Pediatric Bylaw changes that were implemented on December 15, 2020. This bylaw change established requirements for pediatric components of transplant programs, including minimum qualifications for primary pediatric transplant physicians at kidney, liver, pancreas, heart, and lung programs.

Data Summary:

The following components of the policy were monitored in the 6-month, 1-year and 2-year reports:

- Maps of approved programs and counts of pediatric transplants by organ and program.
- Counts of reasons for removal from the OPTN Waiting List by organ including removals for transplant, death, being too sick for transplant, and others.
- Counts of cases where programs without an approved pediatric component listed a pediatric candidate and how many of those were registered using the emergency exception pathway.

The 2-year report compared metrics for the 2 years before and after the following dates:

- Pre-implementation era spanning from December 15th, 2018 to December 14th, 2020.
- Post-implementation era going from December 15th, 2020 to December 15th, 2022.

Note: Both eras were set to include exactly 730 days.

Kidney

There are currently 102 kidney programs with an approved pediatric component.

- Region 11 currently hosts the most pediatric kidney programs with 14 total programs, and Region 6 has the least with 2 programs.
- This is a decrease of 3 programs since the 1-year monitoring report, with Region 2, Region 4, and Region 9 losing an active kidney pediatric program due to it becoming inactive or no longer meeting criteria of the bylaws.

Liver

There are currently 56 active liver programs with an approved pediatric component.

- Region 11 has the highest number of programs, with a total of 8, while Region 6 has the least amount with 1 program.
- This is an increase of 1 active program since the 1-year report, with Regions 4 and 5 each gaining an active program, and Region 1 losing an active pediatric program.

Pancreas

There are 28 currently active pancreas programs with an approved pediatric component.

- The distribution of pancreas programs across all OPTN regions ranges from 1 to 4, with Regions 5, 9, and 10 each having 4 programs, and Regions 1, 4, 6, and 8 each having a single program.
- This shows an increase from the 1-year report, with Region 7 having an additional program with an approved pediatric component.

Heart

There are 60 heart programs with an approved pediatric component.

- Region 5 hosts the most programs with a total of 10, and Region 6 hosts the least with 1 program.

- This is an increase of 2 programs in the past year, with Regions 5, 7 and 10 gaining an active program and Region 8 decreasing by 1 due to a program becoming inactive or no longer meeting the bylaws requirements.

Lung

There are currently 42 lung programs with an approved pediatric component.

- Region 10 hosts the highest number of programs at 6, and Regions 1 and 6 have the least with a single program with a pediatric component.
- This is an increase of 2 approved programs in the past year, with an additional 2 programs in Region 11 now having an approved peds component.

Pediatric Transplant Volume

There was a total of 3594 pediatric transplants in the post-policy implementation era across all organ programs, compared to 3605 that occurred in the pre-policy era.

- Pediatric kidney transplants were the only organ type which saw an increase from pre to post.
- Liver, heart, and pancreas pediatric transplants decreased slightly, but the biggest decrease was seen in pediatric lung transplants which were halved. Research staff noted this could be due to improved treatments for cystic fibrosis leading to children with CF being less likely to require a transplant. Additionally, less patients were being listed post implementation.

Program Count of Pediatric Transplants by Organ and Era

Pediatric transplant volume by program in the pre- and post-policy implementation eras.

- Over half of all programs for each organ type remained consistent or increased the number of pediatric transplants they performed from the pre- to post-policy eras with the exception of lung programs. Research staff noted this includes 26 programs with no current active pediatric component.

Program Count of Pediatric Waitlist Additions by Organ and Era

- A little over 50% of all programs remained consistent or increased their number of pediatric additions to the waitlist from the pre- to post-policy era with the exception of lung programs.

Removal Reasons for Pediatric Waitlist Removals

- In both the pre- and post-policy eras, removal due to transplant was the reason for majority of pediatric waitlist removals for all organ types.
- The most common reason for removal in the “other” category was that the candidate’s condition improved, and transplant was no longer needed.

Pediatric Candidates Listed from Programs Without an Approved Pediatric Component

There have been 7 total uses of the emergency exception pathway.

- 2 heart cases occurred where the emergency exception criteria were met and 5 cases where the criteria were not met which includes 2 heart, 2 liver, and 1 lung case.

Summary of Discussion:

A member commented that it is reassuring to see no significant reduction in pediatric programs or transplants. She noted her only concern was the decrease in pediatric lung transplants and that patients

who have not been listed could be an issue due to access to transplant. Another member noted she has observed an increase in lung transplants in teenagers with COVID-19, with some of those patients going to adult programs.

A member commented that access to transplant care is an issue for all organs and is difficult to identify in the data. For example, what patients are not added to the waitlist because they do not live near a pediatric lung transplant program. Another member noted that insurance companies sometimes restrict patients from seeking transplant services in other states. A member added that reviewing the distance between a patient's home and the nearest transplant center might provide more information about access challenges.

The Vice-Chair asked if the 2-year monitoring report was the last scheduled update. Research staff responded that the Committee could request an additional year. A member suggested following up on the lung data due to the changes.

A member suggested having a breakdown as to why we had 50% fewer lung transplants. For example, if a certain state no longer has a pediatric lung program, then access to care is challenging for some patients. She added that in that scenario an exception can be made for them to get transplanted at an adult program.

Several members supported the recommendation to request a three-year report focusing solely on lung. Another member noted that there might be similar situations for liver programs in some states.

A member noted that one charge of the Committee is to reduce pediatric mortality. She recommended the Committee review mortality as well as the number of transplants and try to identify the reason, whether it is allocation, transplant programs, geography, or some other reason.

The member commented to Scientific Registry of Transplant Recipients (SRTR) staff about previous discussions about waitlist mortality across all the organ systems. SRTR staff responded that when evaluating waitlist mortality, they look at age, disease, status or score, geography, and other factors. She added that this can be reviewed to ensure there is a comprehensive assessment of pre-transplant mortality for each of the organ types.

A member suggested that professional organizations could come together as a community and review the waitlist mortality. She added that this could be powerful information in addition to what is reported by the SRTR. SRTR staff noted previous discussions around how to best describe the patient population in the annual data report to provide transparency to ensure the best possible data is being reported. For example, geographic data could show the distance between home zip code to transplant centers and better inform access issues created by geography.

Next Steps:

The Vice-Chair suggested the Committee review a three-year report with some modifications the Committee could identify during future meetings.

4. Recap and Update: Monitoring Report Reference Documents

The Committee Chair provided an overview of this project to create an internal reference document to help identify specific pediatric metrics to include in policy monitoring reports.

Presentation Summary:

All implemented policy projects are monitored through OPTN monitoring reports, but the metrics reported are not standardized.

- Research staff develops outcome metrics in conjunction with sponsoring committees for each project proposal.
- There may be more helpful or additional metrics for evaluating the impact of policies on Pediatric candidates that are not currently included on monitoring reports.

The Chair noted that this effort is in conjunction with a larger effort by the POC/Research to make policy evaluation metrics more accessible.

The Pediatric Committee discussed this during its January 2023 in-person meeting. The feedback from that meeting included:

- Age stratification:
 - Liver/Intestine: 0-2, 3-6 and 7-10 (or 3-10), 11-17
 - Heart: under 1, include weight and size data along with age breakdown
 - Kidney/Pancreas: 0-5, 6-11, 12-17; keep 0-17 for overall allocation reporting
 - Lung: under 1, 1-5, 6-10, 11-17 (or at least 0-10, 11-17)
- Include confidence intervals of 95% and ignore P-values when small populations in monitoring reports.
- Include transplant rate (actual number) rather than percentage.
- Include pediatric data in MR reports even if cohort count is small and specifically call out outliers when appropriate.
- Heart and liver: report ABOi vs ABO compatible
- Monitor wait time accumulation (active wait time) as WL mortality is less accurate for pediatric patients.
- More data about offer rates desired.

Summary of Discussion:

Staff identified the following questions for the Committee to discuss:

ABOi/ABO Compatible

- Report just counts?
- Counts by age breakdown?

A member recommended counts by age breakdown since the various policies address different age groups. For example, the ABOi policy previously discussed will need to be evaluated to understand the potential impact on other age groups.

The Vice-Chair asked members if there was input for other organ types. A member responded that transplant counts are always important because the pediatric population is always small.

Transplant Rate

- Would it be helpful to report counts instead of or in addition to percentage?

A member noted that counts and percentages would be useful for liver. The Vice-Chair supported either approach but noted both percentage and numbers are important as illustrated in the data previously presented by Research staff.

Offer Rates

- Can you provide an example of a report this was missing from and what data you would like to see? For example, for liver we typically do offer rates by adult vs pediatric by MELD/PELD. Are there additional breakdowns for K/P or other organs that you would like to see for offer rates?

A member recommended collecting data on donation after brain death (DBD) versus donation after circulatory death (DCD). The Vice-Chair and staff noted that offer rates are also part of the organ-specific documents.

Dual Organ Combinations

- Report just counts or something else?
- Which dual organ combinations are particularly important?

The Vice-Chair asked if this was specific to pediatric multi-organ transplants. Staff responded that it is specific to pediatrics. Another member suggested collecting for both adults and pediatrics because it is important to monitor trends. A member noted the most common pediatric multi-organ combinations (liver-kidney, heart-lung) and added that multi-visceral transplants have decreased since 2012. She added it might be beneficial to know why those numbers have been decreasing.

Dual Listing

- Does this refer to one patient listing at multiple centers, or pediatric patients listed for example, a liver and another organ?
- Would you like counts reported or something else?

A member suggested having standard language so it is clear dual listing is referring to being listed at multiple centers while multi-organ is being listed for multiple organs. A member noted that it is interesting to look at one person listing at more than one center, especially given the Ethics Committee discussions. The Vice-Chair noted that “multiple listing” is the term used by the Ethics Committee. She added that it would be helpful to see both numbers and percentages.

A member commented that it is difficult to answer these questions in an abstract way. She added it would be helpful to review reports prior to publishing since not all reports may need to include the ABOi and ABO data.

Next Steps:

Staff will revise the reference documents and distribute them to the Committee members. The Committee will continue the discussions during upcoming conference calls.

Upcoming Meeting

- April 19, 2023 (Teleconference)

Attendance

- **Committee Members**
 - Rachel Engen
 - Evelyn Hsu
 - Abigail Martin
 - Caitlin Peterson
 - Caitlin Shearer
 - Neha Bansal
 - Gonzalo Wallis
 - Jennifer Lau
 - Johanna Mishra
 - Meelie Debroy
 - Kara Ventura
 - Reem Raafat
 - Shantavia Edmonds
- **HRSA Representatives**
 - Marilyn Levi
 - Jim Bowman
- **SRTR Staff**
 - Jodi Smith
 - Katherine Audette
- **UNOS Staff**
 - Kieran McMahan
 - Matt Cafarella
 - Betsy Gans
 - Dzhuliyana Handarova
 - Robert Hunter
 - Susan Tlusty
- **Other Attendees**
 - Daniel Ranch
 - JoAnn Morey
 - Melissa McQueen