

Briefing Paper

Revisions to Pediatric Emergency Membership Exception Pathway

OPTN/UNOS Pediatric Transplantation

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Revisions to Pediatric Emergency Membership Exception Pathway

Affected Policies: OPTN Bylaws Appendices F.7.E and H.4.E
Sponsoring Committee: Pediatric Transplantation
Public Comment Period: July 31, 2017 – October 2, 2017
Board of Directors Date: December 4-5, 2017

Executive Summary

In December 2015, the OPTN/UNOS Board of Directors (Board) approved minimum training and experience requirements for key personnel at pediatric heart, kidney, and liver transplant programs. An emergency membership exception pathway (pathway) was included in the proposal for adult heart and liver transplant programs that did not meet the pediatric key personnel requirements, but wanted to register a patient less than 18 years old on the waiting list. The intent of this pathway was to allow a one-time membership exception for the identified patient under certain exigent circumstances. Members of the Board recognized opportunities for improvement and requested the OPTN/UNOS Pediatric Transplantation Committee (Committee) work on amendments to the pathway.

The Committee collaborated with the OPTN/UNOS Membership and Professional Standards Committee (MPSC) to amend the pathway in 2016. The proposed changes include objective requirements for heart and liver transplant programs that want to register a candidate less than 18 years old. These changes will address concerns over:

- the OPTN's ability to monitor and enforce the requirements
- subjective and ill-defined language in the prior version of the pathway
- how the pathways will work operationally
- what objective criteria will be used to determine when it is acceptable to transplant a pediatric candidate using one of the pathways

The scope of this proposal only includes modifications to the emergency membership exception pathways for heart and liver transplant programs. Modifications to the minimum training and experience requirements approved by the Board in 2015 are not being made.

What problem will this proposal address?

The emergency membership exception pathways currently required an adult heart or liver transplant program to show the following in order to register a candidate less than 18 years old:

1. That the transplant was necessary to prevent a serious and imminent threat to the patient's health or safety
2. That it was medically inadvisable or commercially impractical for the transplant program to transport the candidate to a designated heart or liver transplant program with an approved pediatric component

As currently written, the language for the emergency membership exception pathways would require the MPSC to retrospectively review a transplant team's clinical decisions. A transplant team making the decision would have no frame of reference for how much risk would be considered acceptable while they make the decision because of the subjective nature of the requirements. For example, the listing transplant program must demonstrate that it "believes" the patient must be transplanted, and that transfer to a pediatric hospital is "medically inadvisable or commercially impractical." Less specific member obligations, such as these, can result in inconsistent interpretation and application of these rules.

Similar language regarding a transplant program's "belief" exists for review board and status exceptions.¹ Such language is necessary in status exceptions due to the complexity and non-standard nature of these medical exception cases. If a transplant is performed at a status exception and the status is later evaluated as inappropriate by a review board, the transplant program is subject to potential adverse action. However, the transplant in question would have occurred at a hospital that nonetheless met the minimum training and experience requirements approved to perform the transplant.

Using such subjective language for membership requirements is problematic due to the need to consistently program who use the exception over time, and to reduce risk to candidates and recipients. Subjective membership language would expose OPTN members to risk of adverse action by the OPTN for performing an unapproved organ transplant.

Why should you support this proposal?

Support for the inclusion of this pathway for adult heart and liver transplant programs was suggested in two rounds of public comment in January and August 2015 during the development of the 2015 pediatric membership bylaws proposal. Thus, the transplant community and the general public felt these pathways were necessary elements to the proposal. The amendments to the pathways contained in this proposal add clear, objective criteria that are consistent with other areas of OPTN policy, and address concerns in the earlier language.

The scope of this proposal only includes modifications to the emergency membership exception pathways for heart and liver transplant programs without an approved pediatric component.

How was this proposal developed?

In December 2015, the Board approved minimum training and experience requirements for key personnel at pediatric transplant programs. An emergency membership exception pathway (pathway) was included

¹ See Policy 6.3 *Status Exceptions* for heart ("Transplant physician believes, using acceptable medical criteria, that a heart candidate has an urgency and potential for benefit comparable to that of other candidates at the requested status"); Policy 9.3 *Status and Score Exceptions* ("If a candidate's transplant program believes that a candidate's MELD or PELD score does not appropriately reflect the candidate's medical urgency, the transplant physician may apply to the Regional Review Board (RRB) for a MELD or PELD score exception."); and Policy 10.2.B *Lung Candidates with Exceptional Cases* ("If a candidate's transplant program believes that a candidate's current priority or LAS does not appropriately reflect the candidate's medical urgency for transplant, the transplant program may request approval of a specific priority or LAS by the LRB.").

in the proposal for adult heart and liver transplant programs. This pathway allows an approved adult transplant program that did not meet the pediatric key personnel requirements to register a patient less than 18 years-old on the waiting list.² Use of these pathways would not indicate approval for a pediatric program; rather, the pathways would only apply to one candidate at a time. The intent of these pathways was to allow the listing and transplant of an identified pediatric patient in the circumstance that the patient presented at an approved adult transplant program in urgent need of a transplant, but unable to be safely moved to an approved pediatric hospital. Members of the Board recognized opportunities for improvement in the pathway and requested the Committee amend the pathways.

During the spring of 2016, Committee leadership discussed how to proceed with the Board’s request. A working group was formed with members from the Committee and MPSC. The central tenet of this working group was to amend the pathway in a way that preserved the ability for a medical team at an adult transplant program to execute medical judgment in life-threatening emergencies. The goals of the working group were to address:

- the OPTN’s ability to monitor and enforce the requirements
- subjective language in the Board approved version of the pathways
- how the pathways will work operationally
- what objective criteria will be used to determine when it is acceptable to transplant a pediatric candidate using one of the pathways

Over the course of the discussions, there was consensus that the pathways need to be constructed in a manner that did not undermine the minimum training and experience requirements for key personnel at a pediatric transplant program approved by the Board in 2015.

Patient level characteristics

The pathways from 2015 included language that a patient would qualify if they met requirements for pediatric Status 1A under OPTN Policies 6.1.D (Pediatric Heart Status 1 Requirements),³ or 9.1.B (Pediatric Status 1A Requirements).⁴ Table 1 below profiles the number of transplant candidates waiting on December 31st with the OPTN as heart or liver Status 1A by year and organ from 2012 to 2016.

Table 1: Status 1A Pediatric Heart and Liver Candidates Waiting on December 31, 2012-2016 by Year and Organ

Year	2012	2013	2014	2015	2016
<i>Heart</i>	91	104	103	99	76
<i>Liver</i>	0	1	1	1	3

Based on OPTN data as of May 26, 2017.

Data subject to change based on future data submission or correction.

The working group felt inclusion of all status 1A requirements for heart and liver candidates was too broad, and would allow inclusion of patients that were otherwise not “emergency” listings. The group held lengthy discussions about clinical criteria that may appropriate for the pathways.

Heart Pathway Criteria

The working group discussed a profile of heart failure patients that could be eligible for the pathway. This included life support therapies that would qualify a patient for a Status 1A listing. **Table 2** below lists the type of life support methods used for the 349 pediatric Status 1A heart transplant candidates added to the OPTN waiting list in 2016. Of these candidates, 277 were on at least one type of life support at listing.

² https://optn.transplant.hrsa.gov/media/2074/policynote_20151201_pediatric_training_experience.pdf

³ https://optn.transplant.hrsa.gov/media/1200/optn_policies.pdf#nameddest=Policy_06

⁴ https://optn.transplant.hrsa.gov/media/1200/optn_policies.pdf#nameddest=Policy_09

Table 2: Pediatric Status 1A Heart Candidates Listed in 2016 by Life Support Treatment at Listing

Note: Candidates may have been on more than one type of life support at listing.

Life Support Method	N	%
Extra-corporeal Membrane Oxygenation (ECMO)	34	12
Intra-aortic Balloon Pump (IABP)	3	1
Prostaglandin infusion	29	10
Intravenous inotropes	193	70
Inhaled Nitrous Oxide (NO)	4	1
Ventilator support	111	40
Ventricular Assist Device (VAD)	57	21
Other	14	5

Based on OPTN data as of June 2, 2017.

Data subject to change based on future data submission or correction.

Early discussions with the working group included whether patients on ECMO should be added in the pathway. This cohort of candidates represented approximately 12% of pediatric candidates on life support therapy for heart failure. Cardiology experts on the working group reported that patients on ECMO have increased inpatient mortality and poor transplant outcomes associated with longer treatment periods on ECMO.^{5, 6} Thus, it may be in the patient's best interest to receive an emergency exception and remain at the adult transplant program on ECMO where time on this support therapy may be less.

However, there were diverse opinions on the Committee regarding availability of critical care transport for patients on ECMO. When this proposal was first developed, working group members felt critical care transport for ECMO patients was available, though not widely so. Thus, it was reasonable to include ECMO patients in the pathway. Over the course of discussions, some Committee members verbalized that ECMO transport services became more widely available. As a result of the expansion of this transport capability, it may be in the patient's best interest to be transported to a pediatric hospital where there is clinical expertise and potential greater depth of therapeutic options to treat pediatric heart failure. With the split opinions on the inclusion of ECMO as a criteria in the heart pathway, the Committee opted to include this criteria and solicit public comment whether ECMO should be included. If public comment reflected inclusion of ECMO was not appropriate, the inclusion of ECMO in the pathway would be reconsidered. If public comment supported including ECMO as a criteria, ECMO would remain in the heart pathway.

The working group noted that approximately 20% of pediatric heart status 1A transplant candidates on life support therapy for heart failure are maintained on at least one VAD. The group felt that most patients on VADs can be safely transported between hospitals. Further, many of these patients can be discharged home after inpatient stabilization and are followed in an outpatient setting.⁷ Thus, a pediatric patient with a VAD generally does not justify an emergency situation under the pathway. The working group did discuss the use of non-dischargeable devices (based upon U.S. Food and Drug Administration (FDA) designation). The consensus of the working group was that the transport challenges posed by a pediatric patient maintained on non-dischargeable devices justified an emergency situation under the pathway.

The working group also considered the scenario where an adolescent may receive a durable VAD that is approved by the FDA for discharge in adults, but not approved for pediatrics. Cardiology experts on the working group verbalized this is a frequent clinical practice, and is based on the clinical decision-making

⁵ Fraser, CD, Jaquiss, RD, Rosenthal, DN, Humpl, T, Canter, CE, Blackstone, EH, et al, "Prospective trial of a pediatric ventricular assist device," *New England Journal of Medicine* 367, (2012): 532-541.

⁶ Rossano, JW, Kim, JJ, Decker, JA, Price, JF, Zafar, F, Graves, DE, et al, "Prevalence, morbidity, and mortality of heart failure-related hospitalizations in children in the United States: a population-based study". *Journal of Cardiac Failure* 18, (2012): 459-470.

⁷ M. Schweiger, et al, "Outpatient Management of Intra-Corporeal Left Ventricular Assist Device System in Children: A Multi-Center Experience", *American Journal of Transplantation* 15, (2015): 453-460. doi: 10.1111/ajt.13003

of the patient's care team.⁸ Further, many of these pediatric patients are discharged on the adult-approved VAD. As a result, the working group felt that placement of a durable VAD approved for adults but not pediatrics did not merit an emergency exception.

The working group discussed whether patients on inotropic support should be included in the pathway. This cohort of candidates represented approximately 70% of pediatric status 1A candidates on life support therapy for heart failure. The group felt that pediatric patients on inotropic support can nearly universally be transported to other hospitals, and some of these patients are stable enough to receive home infusion therapy. As a result, the working group did not feel that inotropic therapy would be an appropriate criteria.

Another criteria that was discussed by the working group was intra-aortic balloon pumps (IABPs). This cohort of candidates represented approximately 1% of pediatric candidates on life support therapy for heart failure. The consensus of the working group was IABP are rarely used in pediatric heart failure candidates and transport of a patient on an IABP is a routine practice for critical care transport services.⁹

The working group discussed the administrative elements of OPTN Policy 6.1.D, specifically recertification timelines for a heart transplant candidate.¹⁰ They felt that the recertification timeline of 14 days for pediatric heart Status 1A candidates in OPTN policy was appropriate and should not be changed for the pathway.

Liver Pathway Criteria

The working group discussed the profile of end-stage liver disease patients that could be eligible in the liver pathway. Committee members acknowledged that fulminant hepatic failure and acute decompensated Wilson's disease can manifest over a short period of time. These patients may seek out medical care at a nearby hospital. As a result, there was wide agreement that the liver Status 1A criteria for fulminant hepatic failure and acute decompensated Wilson's disease should be included in the pathway. The questions whether to include post-transplant complications that qualify a liver recipient as Status 1A were also discussed. Although an infrequent event, post-transplant surgical complications can occur. Should instances of primary non-function (PNF) or hepatic artery thrombosis (HAT) occur, an urgent re-transplant may be indicated. Thus, the working group felt it was appropriate to include PNF and HAT the pathway. The working group felt that the objective clinical requirements outlined in OPTN Policy 9.1.B were appropriate, and different clinical criteria would be not be appropriate for the pathway.

Members of the working group discussed the possibility of including liver Status 1A exceptions in the liver pathway. Hepatology experts on the working group did not support the inclusion of Status 1A exception patients in this pathway. The rationale for this position was that Status 1A exceptions by nature are often subjective. The subjective nature of liver Status 1A exceptions would create a potentially problematic situation where a patient was listed as a 1A exception, but then denied the exception when the review board reached its decision, potentially after transplant under the pathway. The consensus of the working group was that it was important to have objective criteria for emergency membership exceptions.

The working group discussed the administrative elements of OPTN Policy 9.2, specifically recertification timelines for a liver transplant candidate.¹¹ They felt that the recertification timeline of 7 days for pediatric liver Status 1A candidates in OPTN policy was appropriate and should not be changed for the pathway.

Pediatric Consultation

The original version of the pathways included subjective language that allowed an adult heart or liver transplant program to use the applicable pathway if, in their opinion, "...it was medically inadvisable or commercially impractical for the transplant program to transport the candidate to a designated liver transplant program with an approved pediatric component." The intent of this requirement was to limit the

⁸ Adachi, I, Burki, S, Zafar, F, Simone-Morales, DL, "Pediatric ventricular assist devices", *Journal of Thoracic Disease* 12, (2015), 2194-2202. doi: 10.3978/j.issn.2072-1439.2015.12.61

⁹ Maymi M, et. al, "Pediatric mechanical circulatory support", *Journal of Pediatric Critical Care* (3), 2016, 186-192. DOI:10.21304/2014.0103.00033

¹⁰ https://optn.transplant.hrsa.gov/media/1200/optn_policies.pdf#nameddest=Policy_06

¹¹ https://optn.transplant.hrsa.gov/media/1200/optn_policies.pdf#nameddest=Policy_09

use of this pathway to circumstances when it was not possible or not safe to transfer the patient to a pediatric transplant program.

The working group understood Board and MPSC concerns over this subjective requirement and the absence of a pre-registration “check” built in to the pathways. The absence of a pre-registration “check” would allow the adult transplant program sole discretion over registering pediatric heart or liver transplant candidates. Thus, the absence of a “check” would undermine the minimum training and experience requirements for key personnel at a pediatric transplant program approved by the Board in 2015. Working group members felt it was important that the adult transplant program intending to use the pathway consult with a transplant program with an approved pediatric component of the same organ type needed for the patient. The purpose of this consultation is not to obtain peer review of the *transplant* decision, but instead to verify the medical judgment of the adult transplant program that the patient in question is not suitable for *transport* to a pediatric transplant program. This consultation must be with the primary transplant physician or primary transplant surgeon of the pediatric transplant program. Doing so would ensure the consulting physician would have the highest level of qualification.

The working group also discussed what role, if any, geographic proximity to the consulting pediatric program should be from the adult transplant program. The group felt there were no justifications to place restrictions on the geographic proximity of the consulting pediatric transplant program.

Post-transplant Care

The working group discussed the idea of requiring the post-transplant care be performed at a transplant hospital with an approved pediatric component. Members of the working group felt there was no compelling reason to apply further program requirements for post-transplant care. Members felt that the adult transplant program accepting responsibility for transplanting a pediatric patient also accepted responsibility for post-transplant care. Further, transplant programs applying for this pathway already need to have indicated their readiness to provide for post-transplant care as outlined in OPTN Bylaws, Appendices D.4 and D.8.¹² Working group members thought it would be remiss to not hold that transplant center responsible for post-transplant recipient outcomes.

Collaboration during Development

Leadership from the Pediatric Committee shared an update with the OPTN/UNOS Thoracic Organ Transplantation Committee during development of this proposal. The Thoracic Committee felt the pathway was overall reasonable, and initiated discussions on the scenario where an adolescent patient may receive a VAD that is FDA-approved for adults only (mentioned above under Heart Pathway Criteria). The Thoracic Committee also brought up concerns over the distance a pediatric patient may need to travel to reach an approved pediatric transplant program, and the potential for conflicting beliefs on patient transfer between clinical staff and the family/caregiver of a patient (when the family/caregiver disagrees with the adult transplant program’s belief that transfer to a pediatric transplant program is warranted). The Chair of the Pediatric Committee acknowledged these concerns during the discussions, noting that these latter concerns were addressed in the Bylaws proposal approved by the Board in 2015. Data from the OPTN noted that, in general, programs that did not meet the case volume requirement within the proposal were located in proximity to transplant programs that do, ensuring equitable access geographically to pediatric transplantation.¹³ The scope of the current proposal is to improve only the language pertaining to the heart and liver emergency membership exception pathways at the request of Board members and the MPSC.

The Committee also sought the input of liver transplant specialists on the liver criteria in the pathway. This feedback was favorable and one suggestion was offered. One individual felt it would be very helpful to adult transplant programs if a programmed feature in WaitlistSM could include the contact information for an approved pediatric component for consultation. Members of the Pediatric Committee felt locating this contact information was not difficult, and programming such a feature would be onerous given the estimated low frequency the pathways are anticipated to be used.

¹² https://optn.transplant.hrsa.gov/media/1201/optn_bylaws.pdf#nameddest=Appendix_D

¹³ https://optn.transplant.hrsa.gov/media/1181/0815-01_pediatic_bylaws.pdf (page 8)

On April 21, 2017, the Committee voted to approve the proposal as amended, and recommend to solicit public comment to the OPTN/UNOS Policy Oversight and Executive Committees (15-Yes, 0-No, 0-Abstain).

How well does this proposal address the problem statement?

It is unknown precisely how often these pathways will be utilized by heart or liver transplant programs. Table 3 below from the 2015 proposal outlines the number of transplants performed at transplant hospitals that met the case volume requirements.

Table 3: Number of pediatric transplants at hospitals meeting the proposed pediatric volume requirements, 1/1/10-12/31/14

Organ Transplanted	Number of Pediatric Transplants	Number and Percent of Pediatric Transplants at Centers Meeting Volume Requirements *	Number and Percent of Pediatric Transplants at Centers <u>NOT</u> Meeting Volume Requirements *
	<i>N</i>	<i>N</i>	<i>N</i>
Heart	1,918	1,838 (96%)	80 (4%)
Liver	2,667	2,533 (95%)	134 (5%)

* Volume criteria:

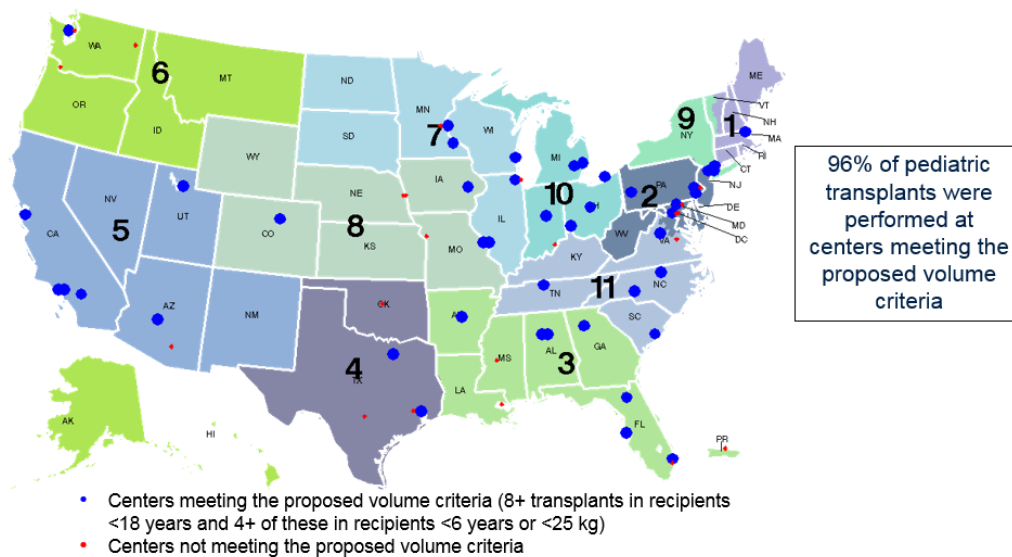
- o Liver: 15+ transplants in recipients <18 years, and 8+ of these in recipients <6 years or <25 kg
- o Heart: 8+ transplants in recipients <18 years, and 4+ of these in recipients <6 years or <25 kg

As Table 3 demonstrates, nearly all pediatric heart and liver transplants are performed at transplant hospitals that likely meet the case volume requirements of the 2015 proposal.

Heart Pathway

The Committee believes the pathway for heart transplant programs will be used infrequently. Figure 1 below depicts the geographic location of heart transplant programs that would likely meet the case volume requirements, and those adult transplant programs that have transplanted a pediatric recipient (but would likely not meet the case volume requirements for an approved pediatric component).

Figure 1: Geographic locations of centers performing pediatric heart transplants January 1, 2010 to December 31, 2014



Based on past transplant activity, it is reasonable that one or more of these adult transplant programs that would not meet the pediatric case volume requirements may elect to use the heart emergency membership exception pathway in the future.

Data outlined in Table 2 (page 3) above described the incidence and type of life support therapies used to treat pediatric heart Status 1A candidates in 2016. VAD use is reported to the OPTN on the Transplant Candidate Registration form for newly listed candidates. This amounts to 12% of new pediatric Status 1A registrations. Table 5 below denotes the specific types of VADs in-use at registration for pediatric candidates added to the waiting list in 2016. Four devices are FDA approved for use out of hospital (Heartware HVAD – 17 primary, 1 secondary, Thoratec PVAD – 1, Heartmate II – 1, and SynCardia CardioWest* - 1). The remaining devices are only FDA approved for in hospital use.

Table 5: Types of Ventricular Assist Device (VAD) for newly listed pediatric status 1A heart transplant candidates in 2016

Primary VAD Brand	
Brand	N
Berlin Heart EXCOR	26
Heartware HVAD *	17
Other, Specify	3
PediMag (Thoratec/Levitronix)	2
CentriMag (Thoratec/Levitronix)	2
Heartmate II *	1
Impella Recover 2.5	1
Maquet Jostra Rotaflow	1
Thoratec PVAD *	1
CentriMag (Thoratec/Levitronix)	1
SynCardia CardioWest*	1

Secondary VAD Brand	
Brand	N
Berlin Heart EXCOR	7
Other, Specify	1
Heartware HVAD *	1
CentriMag (Thoratec/Levitronix)	1

* FDA-approved for out-of-hospital use.

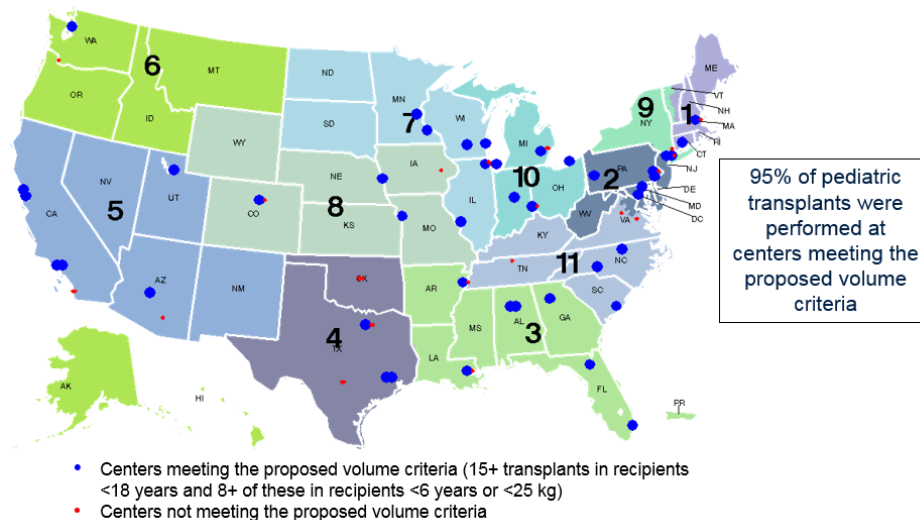
While the Berlin EXCOR had the highest use reported (39% of the candidates), the average age of patients in the U.S. who received a Berlin EXCOR was reported in literature was 18.2 months old.¹⁴ These very young children will almost universally be cared for at comprehensive pediatric transplant hospitals due to the complexity of their disease and comorbidities. The instances in which other inpatient devices are used in pediatric patients is very low, supporting the Committee's expectation that the use of the heart pathway would be infrequent.

Liver Pathway

Figure 2 below depicts the geographic location of liver transplant programs that would meet the case volume requirements, and those adult transplant programs that have transplanted a pediatric recipient (but would not meet the case volume requirements).

¹⁴ Zafar, F., et. al, "Biventricular Berlin EXCOR Pediatric Use Across the U.S.," *The Society of Thoracic Surgeons* 99, no. 4, (2015): 1334, <https://doi.org/10.1016/j.athoracsur.2014.09.078>

Figure 2: Geographic locations of centers performing pediatric liver transplants January 1, 2010 to December 31, 2014



Based on past transplant activity, it is reasonable that one or more of these adult transplant programs may elect to use the liver emergency membership exception pathway in the future.

The Committee achieved consensus that the pathways were amended in a manner that does not undermine the minimum training and experience requirements approved by the Board. Further, the amendments give the OPTN the ability to monitor the pathways, provide OPTN members objective guidance how to meet the elements of the pathways, and address concerns expressed by both the Board and MPSC. In doing so, the amendments continue to allow an adult heart or liver transplant program the option to register a patient less than 18 years old on the waiting list under emergency circumstances.

Was this proposal changed in response to public comment?

Public comment was sought on this proposal during a 60-day period from July 31, 2017 to October 2, 2017. This proposal was widely supported in public comment, both in response to a national webinar in August 2017, and presentations to three OPTN committees. Professional societies and individuals also submitted comments on the proposal online. Themes identified in public comment included:

- Geographic restriction on consulting pediatric transplant program
- Limitation on number of pediatric consults
- Heart criteria should allow all pediatric Status 1A candidates

The Committee carefully considered the feedback received on the proposal.

Geographic Restriction on Consulting Pediatric Transplant Program

Concerns were raised that a pediatric consult could be obtained anywhere in the U.S. The central concern was that distant pediatric programs would evaluate the transport decision differently if the program would not consider the transport to their own facility e.g.: due to excessive distance. By including a geographic restriction in the pathways, only those pediatric transplant programs that may have potential to consider the transport would be allowed to assess for lack of clinical suitability for transport.

The Committee revisited the topic that came up in working group discussions. Members of the working group were sensitive to the competitive environment that may exist between healthcare systems in certain geographic areas. Members felt issues of this type are challenging to sort through and recommended that more general requirements would be best. Further, they felt that extended distances between the adult and consulting pediatric transplant programs would not detract from the merits of the

clinical assessment offered by the consulting individuals, and that any geographic limitations would be arbitrary. Members understood that programming the pathways to include the nearest approved pediatric transplant program would be resource intensive and it was not recommended given the anticipated infrequency of use. The purpose of the consultation is to verify the medical judgment of the adult transplant program that the patient in question is not suitable for transport to *any* pediatric transplant program due to the patient's medical condition. It is not intended to be a question to the pediatric transplant program about whether that program wants to treat the patient themselves.

Limitation on Number of Pediatric Consults

Concerns arose in public comment from the perception that the pathways would allow an adult transplant program to request consults from pediatric transplant programs repeatedly until a consult was obtained that was favorable with adult transplant program's position of not transporting the patient in question. Adding a limit to the number of consults would reduce the likelihood of an adult transplant program seeking a consult until such time as a consult was obtained supporting the decision to not transport the patient. Members expressed the intent of this pediatric consult was a reasonable verification in the process of registering a pediatric heart or liver transplant candidate at an adult transplant program. The working group and Committee held several discussions on this question and felt it was important to allow for second opinions in the pathways. Members discussed the potential of the adult transplant program to maintain a log to document contacts to pediatric transplant programs. UNOS staff shared embedding this type of granular requirement in the OPTN Bylaws would be challenging and perhaps inappropriate. OPTN Bylaws are intended to describe *what* the OPTN is, and *who* can be members of the OPTN. The Committee's post-implementation monitoring will include an assessment for any behavior patterns in exception cases. The MPSC Chair verbalized his support of this review, commenting that if the MPSC was reviewing a case involving use of either heart or liver pathway, they would request documentation to show compliance with the bylaw requirements.

Heart Criteria Should Allow All Pediatric Status 1A Candidates

The Committee then considered the suggestion that the heart pathway should include all status 1A candidates. The OPTN/UNOS Thoracic Committee shared feedback that the VA ECMO and non-dischargeable VAD requirements of the heart pathway appeared to be arbitrarily chosen, and that the rationale for including all pediatric heart status 1A criteria was that a pediatric candidate might not be transportable for other reasons (otherwise medically urgent, unstable or geographically isolated, etc.) and therefore the pathway should not be limited to just those status 1A candidates on VA ECMO or a non-dischargeable VAD. The Committee discussed this feedback, noting there was good consensus during Working Group discussions, and agreement in public comment that the criteria should not be altered. Further, inclusion of only ECMO and non-dischargeable VADs was supported by the Extracorporeal Life Support Organization (ELSO), the American Society of Transplantation (AST), and the International Society for Heart and Lung Transplantation (ISHLT). The MPSC Chair participated in these discussions and commented that the MPSC would not support removing other requirements only to be left with the requirement that a patient would need to meet any of the Status 1A requirements. This would inject a large amount of subjectivity and increase the likelihood of the MPSC to retrospectively review a transplant team's clinical decision-making.

During post-public comment discussions, one Committee member asked if the requirement for a consult from the primary transplant surgeon or primary transplant physician at a pediatric transplant program regarding the *transport* decision exposed the consulting program to any legal liability. Specifically, would an untoward event during patient transport, or bad outcome for the patient following transplant expose the consulting pediatric transplant program, or primary transplant surgeon or physician, to risk of legal action by the family/representative of a patient? Committee members felt this scenario was analogous to a critically ill patient in need of lung transplant presenting at a hospital that was approved to perform heart transplants. Despite a surgeon's possible degree of comfort performing a lung transplant (perhaps experience gained during fellowship training), the transplant hospital is not approved to perform lung transplants. The patient in this scenario would need to be transferred to a transplant hospital that was approved to perform lung transplants. Thus, the Committee members felt such liability would be low.

UNOS General Counsel was asked to consult on the concern raised by the Committee member. Counsel shared that, in all cases, the legal risk to the OPTN was deemed to be low or non-existent. However, the

legal risk to the index adult transplant program or the consulting pediatric transplant program is unknown. Potential legal exposure is state-specific based on the laws and availability of those claims. Based on the wide approval in regional votes, support from AST, ASTS, ELSO, and the Studies of Pediatric Liver Transplantation (SPLIT), and absence of concern in written responses on the requirement for a pediatric consult, the Committee feels it is appropriate to maintain this requirement.

On October 12, 2017, the Committee voted to approve the proposal as written (15-Yes, 1-No, 0-Abstentions).

Which populations are impacted by this proposal?

This proposal will impact any designated heart or liver transplant program that will not have an approved pediatric component but may want to transplant a candidate who is 18 years or younger. It will also affect pediatric candidates who seek transplantation at heart or liver programs without an approved pediatric component. There is no anticipated impact on adult transplant candidates, living donors, or deceased donors.

How does this proposal impact the OPTN Strategic Plan?

1. *Increase the number of transplants:* There is no expected impact on this goal.
2. *Improve equity in access to transplants:* There is no expected impact on this goal.
3. *Improve waitlisted patient, living donor, and transplant recipient outcomes:* These changes specifically outline the criteria for transplant programs who are not otherwise approved to perform pediatric transplants to be able to perform an emergency transplant into a pediatric candidate. The criteria should help to ensure, at a minimum, that a conversation with a pediatric transplant program has occurred and transfer was considered, but not possible due to exigent circumstances surrounding the candidate or the medical care. Additionally, the underlying pediatric Bylaws proposal that was submitted to the Board in December 2015 was a Goal 3 project.
4. *Promote living donor and transplant recipient safety:* These changes will clarify when a transplant program that is not otherwise approved to perform pediatric transplants can perform an emergency transplant into a pediatric liver or heart candidate, thereby helping to ensure that pediatric patients get access to qualified care.
5. *Promote the efficient management of the OPTN:* Clarifying the process for evaluating pediatric program emergency exception requests will allow for more consistent evaluation of such requests.

How will the OPTN implement this proposal?

If approved by the OPTN/UNOS Board of Directors, these proposed Bylaws will be implemented pending programming and notice to members. During the time prior to implementation, the OPTN will provide updates on the pending implementation date and educational opportunities to help prepare for the implementation of these changes, as well as the larger pediatric requirements proposal.

It is expected that this will be implemented concurrently with the related minimum training and experience requirements for key personnel of pediatric transplant programs approved in 2015. The original estimates for the 2015 proposal included a “no sooner than” date of December 2018 to allow members time to prepare. Based on the need for programming of the 2015 proposal and a required review by the U.S. Office of Management and Budget (OMB), implementation for both proposals is not anticipated until sometime in 2019. The OPTN will notify members as the necessary changes near completion. Educational support on these changes will be included under the implementation of the 2015 proposal.

UNOS IT provides cost estimates for each proposal that will require programming to implement. The cost estimates can be small (108-419 hours), medium (420-749 hours), large (750-1,649 hours), very large

(1,650-3,999) or enterprise (4,000-8,000). The IT estimate for this proposal is medium. This estimate includes changes to the candidate registration page in WaitlistSM, and notifications when a pediatric candidate is registered by an adult transplant program.

This proposal also requires a small effort for the UNOS Member Quality Department to implement (161-400 hours). This includes changes to an internal database, and a mechanism to review materials from adult heart and liver transplant programs that choose to use these pathways.

The overall fiscal burden of this proposal is expected to be less as compared to the ongoing costs associated with the 2015 proposal. The pathways included in the 2015 proposal would have consumed greater resources to evaluate and enforce. Thus, the current proposal would represent a long-term savings.

How will members implement this proposal?

Transplant Hospitals

Heart and liver transplant programs without an approved pediatric component will need to become familiar with the justification forms in UNetSM. A heart or liver transplant program that elects to use one of the pathways will need to maintain documentation as outlined in the proposal. If a candidate is registered using one of these pathways, the clinical status must be continually met. Recertification is allowed according to OPTN Policy 6.1.D (heart) and OPTN Policy 9.2 (liver). Candidates not meeting the clinical requirements of the pathways need to be removed from the waiting list within 24 hours.

The OPTN/UNOS Fiscal Impact Advisory Group reviewed the proposal and assessed there would be minimal staff effort to adjust to the new pathways and provide training. Further, there would be no ongoing costs to transplant programs from this proposal.

This proposal will not impact organ procurement organizations (OPOs) or histocompatibility laboratories.

Will this proposal require members to submit additional data?

Heart and liver transplant programs that intend to register a patient less than 18 years old on the waiting list will be required to complete a corresponding Status 1A justification form. This form will be very similar to existing justification forms, and will only contain fields to record the information required to qualify for the pathway. Other status 1A criteria not permitted in the pathways will be removed from the justification form.

How will members be evaluated for compliance with this proposal?

OPTN members will only be allowed register a transplant candidate using the pathways after the exception criteria are met and entered in UNetSM. Thus, all data entered in UNetSM may be subject to review by the OPTN. Members using one of the pathways will be required to provide documentation to support the emergency exception criteria as requested. This may include medical record documentation to support the candidate's status, the consulting pediatric primary transplant surgeon's or primary transplant physician's assessment of the transport decision, and other documentation as requested by the MPSC. Transplant programs found to have inappropriately used the pathways may be subject to adverse actions outlined in OPTN Bylaws, Appendix L.

How will the sponsoring Committee evaluate whether this proposal was successful post implementation?

The Pediatric Committee will collaborate with the MPSC to review each instance in which an adult heart or liver transplant program uses these pathways. Additionally, the Committee will review aggregate data annually on the frequency these pathways are used. The Committee will review the frequency of use and types of life support utilized to determine if there is a need to update the bylaw language for currency. If

the pathways are rarely used, the Committee will consider whether to maintain the language in the OPTN Bylaws.

Policy or Bylaws Language

Proposed new language is underlined (example) and language that is proposed for removal is struck through (example).

1 **RESOLVED**, that changes to Bylaws, Appendices F.7.E (Emergency Membership Exceptions for
2 **Candidates Less than 18 Years Old**) and H.4.E (Emergency Membership Exceptions for
3 **Candidates Less than 18 Years Old**), as set forth below, are hereby approved, effective pending
4 implementation and notice to OPTN members.
5

6 **Appendix F:** 7 **Membership and Personnel Requirements for Liver** 8 **Transplant Programs**

9 **F.7.E. Emergency ~~Pediatric~~ Membership Exceptions for Candidates Less** 10 **than 18 Years Old**

11 A designated liver transplant program that does not have an approved pediatric component may
12 register a patient less than 18 years old on the waiting list if ~~all~~ both of the following conditions are
13 met:

- 14 ~~1. The transplant program believes it must transplant the pediatric patient to prevent a serious~~
15 ~~and imminent threat to the patient's health or safety~~
- 16 ~~2. The patient is pediatric Status 1A according to Policy 9: Allocation of Livers and Liver-~~
17 ~~Intestines.~~
- 18 1. The patient meets the requirements for pediatric status 1A according to OPTN *Policy 9.1.B:*
19 *Pediatric Status 1A Requirements.* This does not include a patient who meets the status 1A
20 requirements by exception according to OPTN *Policy 9.3: Status Exceptions.*
- 21 2. The primary pediatric physician or primary pediatric surgeon at an approved pediatric liver
22 component confirms that it is not medically advisable to transport this patient to a liver
23 transplant program with an approved pediatric component. The transplant program that
24 registers the candidate must document this confirmation.

25 If at any time the candidate no longer meets these criteria, the transplant program must remove
26 the candidate from their waiting list within 24 hours, and may not transplant the candidate. The
27 transplant program must assist candidates in transferring to other designated transplant
28 programs.

29
30 Registration of a candidate less than 18 years old through an emergency exception does not
31 grant the transplant program pediatric component approval.

32
33 ~~The transplant program must submit a pediatric membership exception request to the OPTN~~
34 ~~Contractor within 72 hours of the candidate's registration on the waiting list.~~

35
36 ~~The MPSC will retrospectively review pediatric membership exception requests. As part of this~~
37 ~~review, the MPSC will consult with the Pediatric Transplantation Committee. In submitting the~~
38 ~~pediatric membership exception request, the transplant program must demonstrate *all* the~~
39 ~~following:~~

- 40 3. ~~That the transplant was necessary to prevent a serious and imminent threat to the patient's~~
41 ~~health or safety~~
- 42 4. ~~That it was medically inadvisable or commercially impractical for the transplant program to~~
43 ~~transport the candidate to a designated liver transplant program with an approved pediatric~~
44 ~~component~~

45 5. The candidate was registered as pediatric Status 1A and remained pediatric Status 1A until
46 the time of transplant

47 If the member fails to demonstrate the criteria for this emergency exception, any listing made
48 thereunder will be a violation of OPTN obligations and will be referred to the MPSC.

49
50 Approval of an emergency pediatric membership exception request does not grant the transplant
51 program approval of the pediatric component.
52

53 **Appendix H:** 54 **Membership and Personnel Requirements for** 55 **Heart Transplant Programs** 56

57 **H.4.E. Emergency Pediatric Membership Exceptions for Candidates Less** 58 **than 18 Years Old**

59 A designated heart transplant program that does not have an approved pediatric component may
60 register a patient less than 18 years old on the waiting list if *all* of the following conditions are met:

- 61 1. The transplant program believes it must transplant the pediatric patient to prevent a serious
62 and imminent threat to the patient's health or safety
- 63 2. 1. The patient has one of the following conditions: The patient is pediatric Status 1A
64 according to *Policy 6: Allocation of Heart and Heart-Lungs*
 - 65 a. Is admitted to the transplant hospital and is supported by a surgically implanted, non-
66 endovascular ventricular assist device (VAD) that is not FDA-approved for out of
67 hospital use for any age group.
 - 68 b. Is admitted to the transplant hospital and is supported by veno-arterial extracorporeal
69 membrane oxygenator (VA ECMO).
- 70 2. The patient meets the requirements for pediatric status 1A according to *OPTN Policy*
71 *6.2.A: Pediatric Heart Status 1A Requirements.*
- 72 3. The primary pediatric physician or primary pediatric surgeon at an approved pediatric heart
73 component confirms that it is not medically advisable to transport this patient to a heart
74 transplant program with an approved pediatric component. The transplant program that
75 registers the candidate must document this confirmation.

76
77 If at any time the candidate no longer meets these criteria, the transplant program must remove
78 the candidate from their waiting list within 24 hours, and may not transplant the candidate. The
79 transplant program must assist candidates in transferring to other designated transplant
80 programs.

81
82 Registration of a candidate less than 18 years old through an emergency exception does not
83 grant the transplant program pediatric component approval.

84
85 The transplant program must submit a pediatric membership exception request to the OPTN
86 Contractor within 72 hours of the candidate's registration on the waiting list.

87
88 The MPSC will retrospectively review pediatric membership exception requests. As part of this
89 review, the MPSC will consult with the Pediatric Transplantation Committee. In submitting the
90 pediatric membership exception request, the transplant program must demonstrate *all* the
91 following:

- 92 1. That the transplant was necessary to prevent a serious and imminent threat to the patient's
93 health or safety

- 94 2. ~~That it was medically inadvisable or commercially impractical for the transplant program to~~
95 ~~transport the candidate to a designated heart transplant program with an approved pediatric~~
96 ~~component~~
97 3. ~~The candidate was registered as pediatric Status 1A and remained pediatric Status 1A until~~
98 ~~the time of transplant~~

99 ~~If the member fails to demonstrate the criteria for this emergency exception, any listing made~~
100 ~~thereunder will be a violation of OPTN obligations and will be referred to the MPSC.~~

101 ~~Approval of an emergency pediatric membership exception request does not grant the transplant~~
102 ~~program approval of the pediatric component.~~
103