Appendix A

Recent History of Policy Development Process

Leading to Fall 2011 Public Comment Proposals

OPTN/UNOS Liver and Intestinal Organ Transplantation Committee

Background and Framework

Liver **allocation** has been based on the model for end-stage liver disease (MELD) and pediatric end-stage liver disease (PELD) scores since 2002. This system prioritizes candidates based on mortality risk while awaiting liver transplantation and has been recognized as a major improvement in the way that candidates are prioritized for a liver transplant. **Distribution** refers to how donor livers are offered to the prioritized list of candidates, and has been based historically on the location of the transplant center relative to where the organ was procured.

The OPTN Final Rule[1], effective March 2000, is a set of federal regulations that amplify the legal authority for the OPTN contained in the National Organ Transplant Act of 1984 (NOTA)[2]. The Final Rule governs organ allocation and OPTN policy development. The Final Rule specifies the bases upon which organ allocation policies may and may not be predicated, as well as performance goals for equitable organ allocation policies. One of the performance goals in the Final Rule is to distribute "organs over as broad a geographic area as feasible under paragraphs (a)(1)-(5) of this section, and in order of decreasing medical urgency."

The Board has approved several changes to the liver distribution policy since the MELD/PELD allocation system was implemented in 2002 that are in compliance with this aspect of the Final Rule. The Committee recognized that this concept of "feasibility" is an important one and should not be ignored. What is feasible to one center, or region, may not be for another. Factors pertaining to feasibility include, but are not limited to, cold ischemic time (CIT), use of air versus ground transportation, possible increased costs, and system inefficiencies. The concept of "feasibility" also leads to considerations about what is practical from an operational standpoint.

The Current Distribution System and Recent Changes

The current system uses a local, regional, national algorithm. The local distribution unit is defined as the donation service area (DSA) of an organ procurement organization (OPO). After being offered to the sickest candidates (Status 1A/1B) regionally, deceased donor livers are offered locally, then regionally, to candidates with a MELD/PELD score of 15 or higher before being offered to candidates with lower MELD/PELD scores.

This "Share 15 Regional" policy, implemented in 2005, was intended to reduce waiting list deaths by directing livers to the patients who would most benefit. Analysis of national data showed that the vast majority of patients with a MELD score below 15 did not benefit from a liver transplant, and that the advantage of a transplant increases as the MELD score increases[3]. This research was the basis for the "Share 15 Regional" policy, which allocates livers first locally, then regionally, to candidates with MELD/PELD scores greater than 15 prior to local candidates with lower scores. The goal of this policy was to redirect deceased donor livers to sicker patients and away from less ill patients (MELD/PELD < 15)

[1] 42 CFR Part 121, see http://optn.transplant.hrsa.gov/policiesAndBylaws/final_rule.asp, hereafter referred to as the "Final Rule"

[2] National Organ Transplant Act (NOTA), 1984 Public Law 98-507, amended in 1988, 1990, and 2008 http://www.unos.org/SharedContentDocuments/NOTA_as_amended_-_Jan_2008.pdf

[3] Merion RM, Schaubel DE, Dykstra DM, Freeman RB, Port FK, Wolfe RA. The survival benefit of liver transplantation. Am J Transplant 2005;2:307-313

who, in general, will live longer without a transplant. This goal has been met with no adverse impact on post-transplant outcomes. At the same time the Share 15 Regional policy was implemented, another policy was implemented, which gave priority for pediatric donors to pediatric candidates. This was extended in December 2010, such that very young donors (age 0-10) are offered regionally, then nationally, to urgent pediatric candidates.

A policy that allocates livers locally, then regionally to critically ill (Status 1A/B) patients, first implemented in 1999, has been shown to reduce waiting list mortality.[4,5] Patients listed as a Status 1A/B must meet very strict criteria. If these criteria are not met, a patient can still be listed as a Status 1A/B, but the case is reviewed by the Liver Committee for potential referral to the Membership and Professional Standards Committee (MPSC). Because status 1A/1B patients continued to have a high wait list mortality relative to other wait listed patients, the distribution policy was expanded so that livers are offered first to local and regional Status 1A candidates, combined into one 'regional' list , followed by combined local and regional Status 1B candidates, before being offered to local patients with MELD/PELD scores of 15 or higher. This change was implemented in December 2010.

A proposal for regional distribution of livers to all patients ranked by their MELD/PELD score was distributed for public comment in the spring of 2009. A great deal of public comment was received in response to this proposal. Support for this policy was mixed and the Committee withdrew the proposal from Board consideration. However, a number of requests and ideas were put forward for the Committee to evaluate and assess in regards to improving the system. Subsequently, the OPTN/UNOS Board authorized a public forum to address issues related to liver allocation and distribution.

<u>Recent Policy Development and Collaboration</u>

Using the Final Rule's performance goals for guidance, the Committee set out to determine what changes to the distribution of livers would be "feasible," in terms of logistics, costs and risks related to transportation, patient outcomes, and community acceptance. This was done by a series of outreach efforts and committee discussions, including:

- Request for Information (RFI) Document and Survey, distributed in December 2009
- Public Forum held in Atlanta in April 2010
- Concept Paper and Survey, distributed December 2010
- Presentations at various transplant meetings (AASLD, ASTS Winter Symposium, ATC), in 2010 and 2011
- Updates to the OPTN/UNOS Board of Directors, 2010-2011
- Review of survey results at March 2011 Liver/Intestine committee meeting
- Review of SRTR modeling of National MELD 15 and Tiered Sharing at July 2011 Liver Committee meeting

[4] Washburn K, Harper A, Klintmalm G, Goss J, Halff G. Regional sharing for adult status 1 candidates: Reduction in waitlist mortality. Liver Transpl 12: 470–474, 2006.

[5] Humar A, Kwaja K, Glessing B, Larson E, Asolati M, Durand B, Lake J, Payne W. Regionwide sharing for status 1 liver patients – beneficial impact on waiting time and pre- and posttransplant survival. Liver Transpl 2004;10:661–665.

RFI and Forum

A request for information (RFI) was submitted to the public on December 18, 2009[6]. This document briefly summarized the history of and mechanisms for liver allocation and distribution, as well as some of the concepts that emerged from the spring 2009 public comment response. The RFI included a survey, which received 87 individual responses and many ideas for evaluation and possible change. For example, the committee recognized that there may be other methods for improving geographic access, such as concentric circles, as currently used in thoracic organ allocation. These helped shape the agenda for the forum held in April 2010. The agenda included 12 topical presentations from members of the community and Committee members. The audience was polled throughout the day, repeating many of the questions included in the RFI survey, and there was extensive time for audience participation and questions. More than 160 individuals attended the forum, with at least 70 more joining through an internet broadcast. Feedback from the RFI and forum highlighted several areas of common ground and the potential for consensus-building moving forward. During the Forum, several options for changes to distribution were discussed, through presentations and polling questions:

- Full Regional Sharing (Regions 1 and 9)
- Concentric Circles
- Extension of Share 15 Regional
- Tiered Regional Sharing
- Net Transplant Benefit (with and w/o broader distribution)

RFI respondents and forum participants felt that the MELD/PELD allocation system was not broken, but that further refinement of MELD (such as incorporation of serum sodium) might be warranted. While transplant outcomes were felt to be important, transplant benefit (which incorporates pre-transplant mortality and post-transplant outcomes into one score) was thought to be premature for serious consideration.

As most forum participants indicated that the current *allocation* system did not need significant modification, the Committee has placed more emphasis evaluating potential modifications to the current *distribution* system. A number of distribution concepts were explored and modeled, to assess the potential impacts on waiting list deaths and distances organs would travel. The predominant theme of the feedback was advocacy for small, incremental and practical changes that would produce the greatest reduction in waiting list deaths while limiting the distances organs travel.

Expansion of the current regional "Share 15 Regional" policy to a "Share 15 national" received substantial support. One other type of distribution system studied in detail was tiered MELD/PELD sharing, whereby livers are first offered regionally to candidates with scores over a certain MELD/PELD threshold. Distribution via concentric circles, akin to the current thoracic organ distribution system, was evaluated. Circles defined by population density were also considered. Although the use of concentric circles has many positive aspects, such as eliminating arbitrary geographic boundaries, distribution based on donor location rather than the transplant center, and current use and acceptance by the thoracic organ community, this system would substantially change liver distribution and may not be "feasible" given current sentiments and restrictions, as concentric circles could not be classified as a small, incremental step, nor has it been piloted in any way.

[6] http://optn.transplant.hrsa.gov/SharedContentDocuments/LiverRFI_121809.pdf

There was broad agreement that an expedited liver placement policy would help optimize utilization and would lead to more organs transplanted, reducing waiting list deaths.

One frequently-cited concern with broader sharing is the possibility that donor livers could be shared across a moderate sized geographic area when the difference between a local patient and the non-local patient is 1 or 2 MELD/PELD points. Having livers "criss-cross" for patients with comparable mortality risk is not optimal, but there was little data to demonstrate how often this would occur. The Committee included a concept termed a Sharing Threshold (ST) (formerly 'risk-equivalent threshold') in the RFI. This would set some MELD/PELD differential between a local and non-local patient that would preclude a regional share.

In June 2010, the Committee reported the results of the RFI survey and Forum to the OPTN/UNOS Board of Directors. At that time, the Board approved the following motion:

****** RESOLVED, that the Liver and Intestinal Organ Transplantation Committee shall be charged with making recommendations to reduce geographic disparities in waitlist mortality.***

Concept Document and Survey

Feedback from the RFI and forum highlighted several areas of common ground and the potential for consensus-building moving forward. These ideas were included in a Concept Document that was circulated nationally on December 31, 2010[7]. After reading the document, readers were asked to answer a brief survey. A total of 227 responses were received, from every Region and almost 40 states. While 70% of respondents identified themselves as being associated with a transplant center, the remaining respondents were either affiliated with an OPO, or were recipients, candidates, donors, or family members. The Committee reviewed the results of the Concept Paper survey during the February conference call and March meeting. There were 227 responses, with 70% identified as being affiliated with a liver transplant program, and the remainder as either OPO personnel, or recipients, candidates, donors, or families of candidates/recipients/donors. Responses were received from every region and 36 states. A tabulation of the responses is shown in Table 1. Nearly three quarters of the respondents supported a national share 15 policy. Similarly, the majority supported some sort of broader regional sharing at higher MELD scores. Because respondents could select multiple thresholds for Question 4, the percentages sum to greater than 100%. Some of the responses were difficult to interpret; for example, 26 answered that they would support a threshold of 32 only, making it unclear whether those would also support a higher threshold of 35 if it was proposed. Further, some individuals selected "none of the above" but in the text response indicated that a lower threshold or full regional sharing for all MELD/PELD scores should be considered. A total of 164 respondents (72%) selected some form of regional sharing (35, 32, 29, or other).

Figures 1 and 2 provide a side-by-side comparison of the polling results obtained at the Atlanta Forum and by the RFI and Concept Paper Surveys.

[7] http://optn.transplant.hrsa.gov/SharedContentDocuments/LiverConcept_123010.pdf

Table 1

Question	Yes	No
1. Would you support a national share 15 policy?	170 (74.9%)	57 (25.1%)
2. Is there a subgroup of liver transplant candidates with low MELD/PELD scores who may be unduly disadvantaged by a National Share 15 policy?	107 (47.1%)	120 (52.9%)
3. Do you think broader sharing for patients with high waiting list mortality is reasonable?	178 (78.4)	49 (21.6)
4. Would you support regional sharing for a MELD/PELD threshold of (check all that apply):		
• 35	74 (32.6%)	
• 32	57 (25.1%)	
• 29	68 (30.0%)	
• None of the above	47 (20.7%)	
• Other	24 (10.6%)	
➢ Selected 29, 32, **or** 35, above	143 (63.0%)	
5. Should the Sharing Threshold (ST) concept be incorporated if tiered MELD/PELD sharing is endorsed?	185 (80.5%)	42 (18.5%)
6. Would you support a national policy for facilitated placement of donor livers that are not used locally or regionally?	208 (91.6%)	19 (8.4%)

Share 15 National

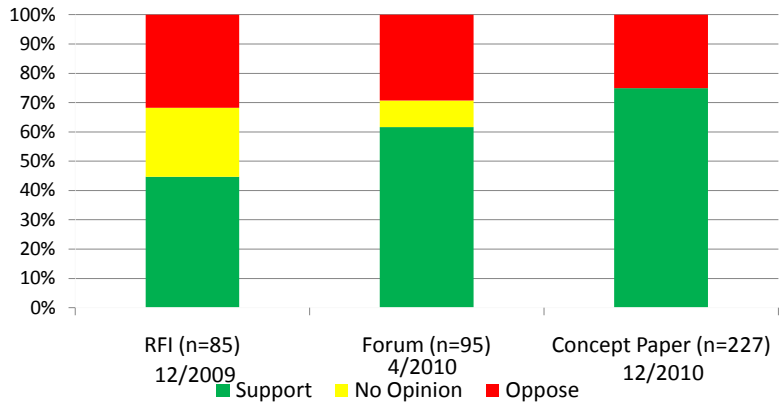

Figure 1

Tiered Regional Sharing

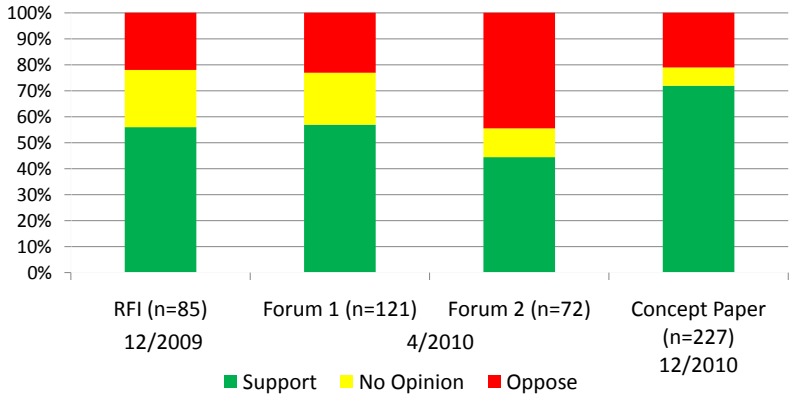

Figure 2