



Educational Toolkit for the PSC Community: Communicating Transplant Concerns to UNOS

Overview

PSC patients and caregivers may want to learn more about the current U.S. system of organ allocation or have concerns about how PSCers fare. The United Network for Organ Sharing (UNOS) schedules public comment sessions to hear concerns and other feedback, and this toolkit may be helpful if one plans to comment. PSC Partners prepared it so that the PSC community might understand the liver allocation system and how it works; advocate for a system that fairly prioritizes PSC without creating inequities for other liver disease patients; and be able to clearly articulate concerns during the UNOS public comment sessions.



Key Terms and Background Information



OPTN – In 1984, the U.S. Congress passed the National Organ Transplant Act, which established the Organ Procurement and Transplantation Network (OPTN) to maintain a national registry for organ matching. The act also called for the network to be operated by a private, non-profit organization under federal contract.

UNOS – the United Network for Organ Sharing is the private, non-profit organization that manages the nation's organ transplant system under contract with the federal government.

MELD score – The Model for End-Stage Liver Disease score is designed to calculate the relative severity of a patient's liver disease and the mortality risk over the next three months. It uses a specific formula approved by the OPTN for allocation of livers by the OPTN match system. The MELD considers four blood values: INR, serum creatinine, bilirubin, and serum sodium, and is used for patients 12 years and older.

PELD score – The Pediatric End-Stage Liver Disease score calculates the severity of a pediatric (<12 years old) patient's liver disease and the mortality risk over the next three months. It considers growth factors, blood values, age at listing and diagnosis. It is rare for a PSC patient under 12 years old to need a transplant.

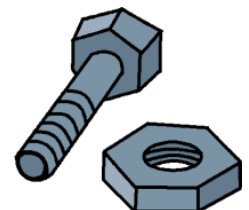
NLRB – In May 2019, the OPTN launched a National Liver Review Board to improve the process of

assessing and determining liver transplant priority for candidates with exceptional medical conditions. It replaces the regional review boards previously used in the 11 OPTN regions. The NLRB is intended to increase consistency in providing exception scores nationwide and better balance transplant access for candidates with and without exception scores.

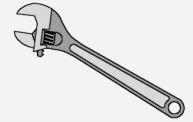
Exception scores (points) – In limited instances, the MELD score doesn't accurately reflect a patient's mortality risk or disease severity. In such a case, the NLRB makes an exception and adds points to the patient's MELD score. Hepatocellular carcinoma and cholangiocarcinoma are examples of diseases that qualify for such points. Certain severe complications of PSC also qualify. The current exception score standards were adopted in 2019.

UNOS public comment periods - UNOS convenes the community and welcomes open debate throughout the policy development process. PSC Partners encourages informed commenting to make concerns known. Public comments are submitted online, and the public can also attend certain UNOS events. More information is on the UNOS and OPTN websites: optn.transplant.hrsa.gov and unos.org.

*Some information is quoted directly from the OPTN and UNOS websites.



Concerns of the PSC community



There is concern among the PSC community that the MELD score does not accurately reflect how seriously ill a PSC patient may be. Many PSCers with advanced disease have a host of symptoms and complications, including cholangitis attacks, biliary strictures, portal hypertension, varices, ascites, jaundice, intractable itching, chronic pain, fatigue and sleep disturbances, hepatic encephalopathy ... the list goes on.

As the disease progresses, a PSC patient may be referred by their physician for a transplant evaluation. Very often, the MELD score is not particularly high, and the patient learns that they will have difficulty attaining a score that puts them near the top of the transplant list.

Why is this? One reason is that the MELD calculation includes measurements of both liver and kidney function. Most of the time, a PSCer's kidneys are healthy, which keeps the total MELD score lower until the patient becomes critically ill. At that point, the transplant surgery may be riskier and recovery lengthier.

The next section shows how the implementation of the NLRB and new guidelines for exception scores has changed the landscape for PSCers.

The OPTN's current stance on PSC and exception scores

When the OPTN moved to a national review system in 2019, specific guidelines regarding exception scores were published ("Guidance to Liver Transplant Programs and the National Liver Review Board for: Adult MELD Exception Review"). The NLRB follows these guidelines when transplant centers appeal for an exception score on behalf of a patient. In part, the guidelines read:

For many patients with chronic liver disease the risk of death without access to liver transplant can be accurately predicted by the MELD score ... However, for some patients the need for liver transplant is not based on the degree of liver dysfunction due to the underlying liver disease but rather a complication of the liver disease ...

The document also specifies criteria that the Review Board should consider when it reviews applications for exception scores for candidates with PSC:

The candidate must meet **both** of the following two criteria:

1. The candidate has been admitted to the intensive care unit (ICU) two or more times over a three-month period for hemodynamic instability requiring vasopressors [unstable blood pressure requiring medication],
AND
2. The candidate has cirrhosis.

In addition, the candidate must have **one** of the following criteria:

- > The candidate has biliary tract strictures which are not responsive to treatment by interventional radiology (PTC) or therapeutic endoscopy (ERCP)
OR
- > The candidate has been diagnosed with a highly resistant infectious organism [several examples are given].

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Continued from previous page —The OPTN's current stance:

Likewise, in the same document, the OPTN states that there is insufficient evidence that **ascites, gastrointestinal bleeding, hepatic encephalopathy, or pruritis (itching)** affect **mortality risk**. Therefore, these conditions do not qualify for exception points under the new standards. Previously, the regional review boards tended to award exception points more subjectively, and PSC patients benefitted from slightly less stringent standards. The complete OPTN document can be accessed at: optn.transplant.hrsa.gov/media/2847/liver_guidance_adult_meld_201706.pdf.

Liver transplant policy continues to change as transplant experts study the system and find ways to improve it for patients ... this system is designed to be flexible and allow improvements ... new studies are taking place all the time to learn how to save more lives and help people live longer and better." ~ OPTN website

Now what?!

As discouraging as the above information about the OPTN's stance may seem, it is vital to understand that the metrics are based on **mortality risk**, not quality of life. The OPTN has already analyzed the effects of PSC and its complications on mortality risk and only recognize the few situations listed above as risks that qualify for exception scores.

Some would like to see these metrics changed. How can the PSC community make a difference? An understanding of OPTN's metrics will help inform comments submitted to UNOS. Read on for information about the UNOS Summer 2020 Public Comment Session and talking points you may wish to use (next page).

The UNOS Summer 2020 Public Comment Session runs from August 4 through Oct. 1, 2020

The UNOS Summer 2020 Public Comment Session

UNOS holds a regular series of public comment sessions (as well as various meetings) to discuss those policies currently under review. All policies come up for review from time to time, but they are not all under review at the same time. **It is important to note** that the exception score guidelines for PSC outlined above are NOT up for review at this time. Nonetheless, PSC Partners encourages the community to submit comments to UNOS about the guidelines whenever an opportunity arises, as the OPTN explicitly encourages such: "The [Liver and Intestinal Organ Transplantation] Committee is seeking public feedback on the proposed changes listed above **as well as any other aspects of the NLRB.**"

The proposals **currently** under review fall in the categories of policy changes and guidance related to other diseases, as well as operational guidelines. You can read the details at: optn.transplant.hrsa.gov/media/3927/further_enhancements_nlrbc_pc.pdf.



Talking points for public comment



Listed below are some talking points for you to consider. These have been informed by conversations with PSC medical advisors.

Commenters should take into account the effect that suggestions would have on waitlist candidates who have other liver diseases. PSCers are a small percentage of liver transplant patients, but changes to the allocation and exception scores systems will affect all patients. No patient-identified or other confidential information should be shared.

Possible talking points:

- > Could the MELD score calculation be weighted differently for PSCers, considering that, for many, the MELD score remains low until they are critically ill and close to death?
- > Is MELD the right metric for PSCers, or should other factors be considered? For PSCers, metrics such as physical frailty, muscle wasting and unintentional weight loss could be more predictive of the need for transplant.
- > Some of the other diseases which present for transplant have alternate treatments that can be tried first, but PSC has no approved first-line treatment option.
- > Should utility and outcomes be considered? PSC transplant patients tend to have excellent outcomes if transplanted before becoming critically ill.
- > UNOS needs to study the data and metrics regularly to ensure that PSCers are fairly represented.

To share your comments on the UNOS Public Comment web page, go to optn.transplant.hrsa.gov/governance/public-comment/further-enhancements-to-the-national-liver-review-board and click on “Provide Feedback.” Public comments will be accepted through Oct. 1, 2020.



Will UNOS read and consider the comments?

Yes! As a government contractor, UNOS is beholden to *everyone* served by the U.S. government. Of course, comments from those who are well-informed and have a personal stake will be most relevant. **However**, changes to the organ allocation system take time, as they require thorough scientific review and discussion.

The OPTN Liver and Intestinal Organ Transplantation Committee reviews all policies governing the allocation of exception points every 2-3 years. Though the committee is not reviewing the PSC exception scores at this time, if the PSC community makes its voice heard at every opportunity, it will have a greater chance of being making a difference!

Also, **please note** that a whole new system of geographical allocation for liver and intestine transplants was just implemented in February 2020. It is now based on “acuity circles” around each transplant center and replaces donation service areas and the regional boundaries previously used. Since the policy has been in effect less than a year, it is probable that UNOS is still gathering data to see if that change affected mortality on the waiting list for PSCers and other liver disease patients. More information is on the UNOS website unos.org/ (search for “acuity circles”).

PSC Partners Seeking a Cure's role

As an organization, PSC Partners is in frequent contact with a variety of expert medical advisors who know the needs of PSC patients. We will continue to communicate with them to find out about any new study results regarding the mortality risks of PSCers on the transplant waiting list. They are in the best position to know the most up-to-date information.

Education is a major part of our mission, and we will continue to keep patients and families informed about transplant issues through our website, conferences, and information pieces such as this toolkit.

Find us online at pscpartners.org.

Other steps that you can take

- ⇒ Continue your involvement with and support of PSC Partners Seeking a Cure. When we find better treatments and, eventually, a cure, fewer patients will require a transplant.
- ⇒ Join the PSC Partners Patient Registry at pscpartnersregistry.org. A robust patient registry attracts researchers and accelerates studies and trials.
- ⇒ Advocate for organ donation. If there were more donors, all patients needing a transplant could get one in a timely fashion.
- ⇒ As always, follow your doctor's advice and take care of your overall health.



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