Final Report

OPTN Liver and Intestinal Transplantation Committee | Descriptive Data Request

National Liver Review Board Enhancements Monitoring

DHHS Contract No. 250-2019-00001C
Date Completed: January 6, 2025

Prepared for:
Liver and Intestinal Transplantation Committee
Committee Meeting
Date of Meeting: January 17, 2025

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Contents

Background/Purpose

This report monitors the impact of seven policy, guideline, or guidance changes for the National Liver Review Board (NLRB). The seven changes occurred across two public comment NLRB enhancement proposals and are listed below:

I.Policy Language: Standard Criteria for Portopulmonary Hypertension Exceptions - **Implemented October 7, 2021**

II.Operational Guidelines: Pediatric Appeals Review Team (ART) - **Implemented October 7, 2021**

III.Guidance: Polycystic Liver Disease - **Implemented February 9, 2021**

IV.Policy Language: Hilar Cholangiocarcinoma (CCA) Standardized Exception Criteria - **Implemented October 7, 2021**

V.Guidance: Pediatric Updates - **Implemented July 15, 2021**

VI.Guidance: Neuroendocrine Tumors (NET) - **Implemented July 15, 2021**

VII.Guidance: Primary Sclerosing Cholangitis and Secondary Sclerosing Cholangitis - **Implemented July 15, 2021**

Details regarding the changes that occurred for each guidance, policy or guideline update can be found in the summary paragraphs prior to the monitoring results provided for each change.

Cohort

The report summarizes all liver exception requests including liver MELD and PELD exception request forms submitted between 02/13/2018 - 07/14/2024. The eras which the data are evaluated over depend on the policy, guidance or guideline implementation date and can be found in the detailed paragraphs prior to the monitoring results for each change.

Candidate information was submitted through OPTN Waiting List and on the Transplant Candidate Registration (TCR). Recipient and transplant data were submitted on the Transplant Recipient Registration (TRR). Donor data were submitted in OPTN Donor Data and Matching System (DonorNet) and on the Deceased Donor Registration (DDR). Multiple organ transplants were excluded from the analysis, with the exception of the cohort utilized to evaluate Polycystic Liver and Kidney Disease transplants. OPTN data as of October 25, 2024 were used for this analysis. Data are subject to change based on future submission or correction.

Results: Further Enhancements to the National Liver Review Board (August 2020 PC)

I.Policy Language: Standard Criteria for Portopulmonary Hypertension Exceptions

Summary of Change:

The changes to policy include updating the criteria for a standardized MELD or PELD exception for portopulmonary hypertension to match updated clinical guidelines. For more explicit information regarding the changes to standard exception criteria, see the policy notice document here: https://optn.transplant.hrsa.gov/media/4251/further_nlrb_enhancements_202012.pdf.

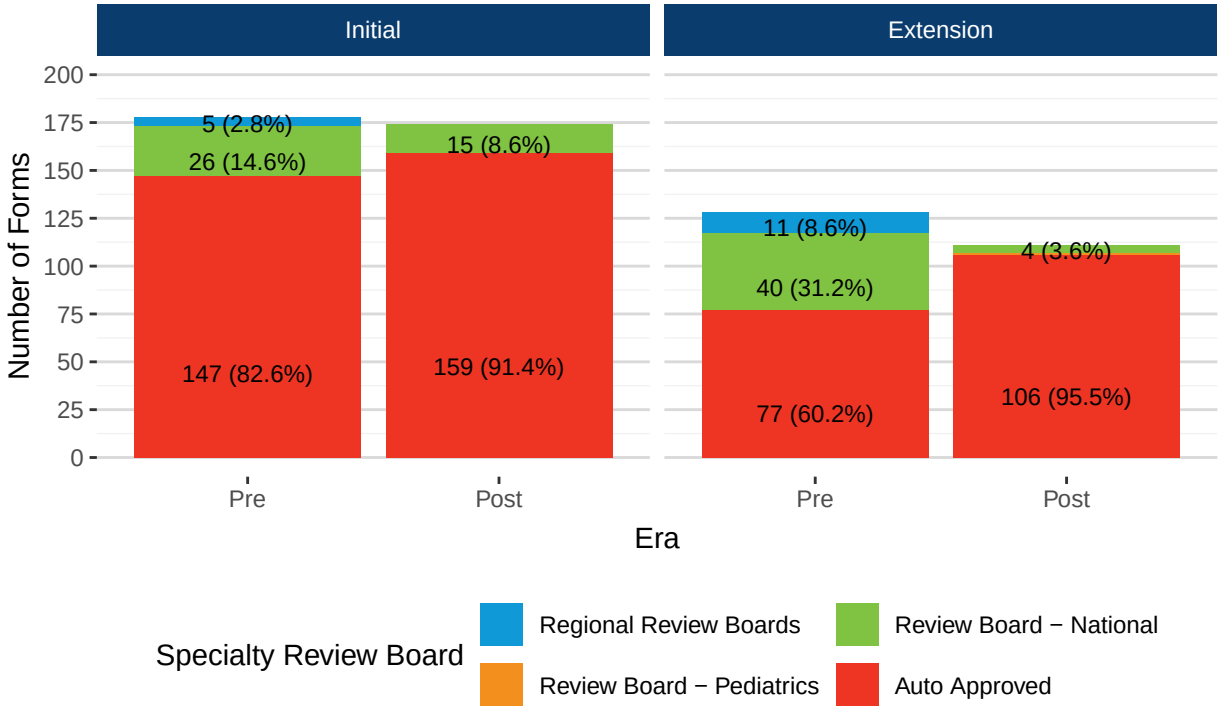
The policy was implemented on 10/07/2021. The policy is evaluated 2.5 years following implementation, with the pre-policy era spanning from April 9, 2019 to October 6, 2021 and post-policy era spanning from October 7, 2021 to April 6, 2024. The following metrics were evaluated:

- Number of exception cases for portopulmonary hypertension (overall, by automatic system approval/NLRB board review, case outcome, and application type)
- Distribution of automatic approval turn-down reasons for portopulmonary hypertension cases (reasons criteria were not met)
- Number of transplant recipients with a portopulmonary hypertension exception

Summary of Results:

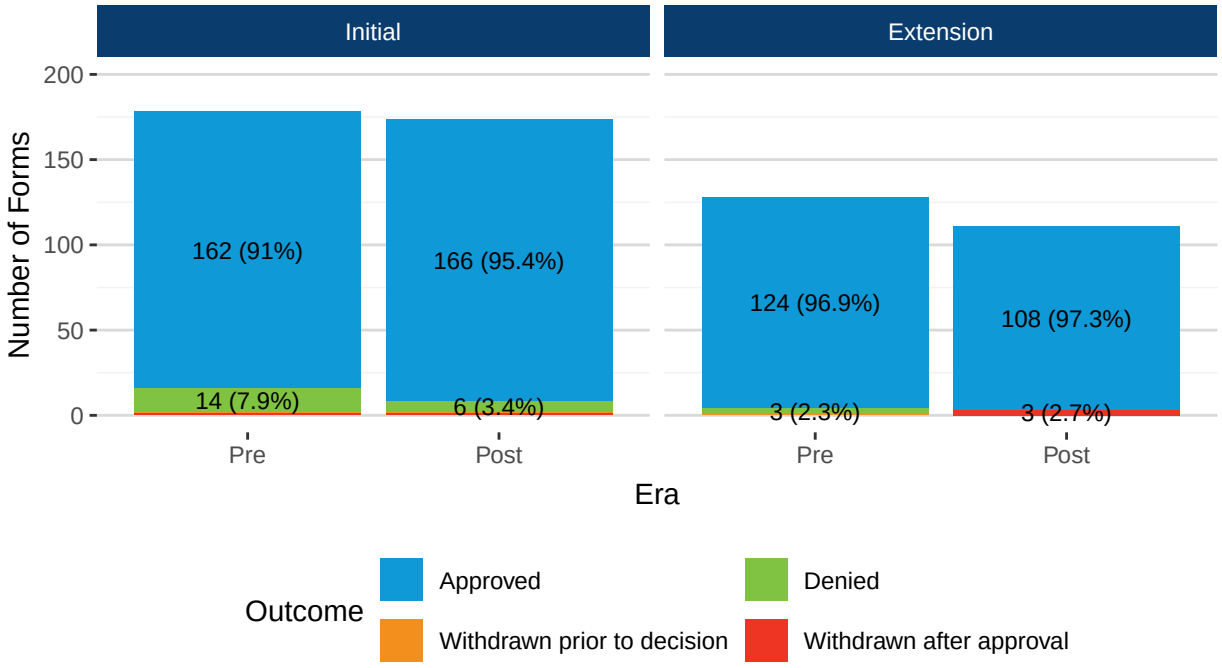
The number of initial and extension forms submitted for portopulmonary hypertension exception cases decreased in the post-policy era. The proportion of portopulmonary hypertension exception cases that were automatically approved increased for both initial and extension cases. Cases that were not automatically approved were reviewed by the NLRB, which then ultimately decided whether the case was approved or denied. The most frequently occurring turn-down reason for automatic approvals in the pre-policy was "Extension is not automatically approved if a previous application did not meet the criteria outlined in policy." The most frequently occurring turn-down reasons for automatic approvals in the post-policy was "Initial MPAP not greater than or equal to 35 mmHg" and "Post-treatment MPAP is greater than or equal to 35 mmHg and less than 45 mmHg and post-treatment PVR is not less than 240 dynes*sec/cm5 or not less than 3 Wood units (WU)." The number of portopulmonary hypertension exception cases transplanted decreased in the post-policy era, but the proportion remained the same.

Figure I.1: Portopulmonary Hypertension Exception Cases by Application and Review Type



There was 1 extension case reviewed by the pediatric review board in the post−policy era, not labelled in the figure.

Figure I.2: Portopulmonary Hypertension Exception Cases by Outcome



In both the Pre− and Post−Policy eras for intial forms, there was 1 form withdrawn prior to decision, and 1 form withdrawn after approval, not labelled in the figure. There was also 1 extension form in the Pre−Policy era withdrawn prior to decision not labelled in the figure

Table I.1: Distribution of Automatic Approval Turn-Down Reasons for Portopulmonary Hypertension Cases (Reasons Criteria were Not Met)

	Policy Era	
Turn-Down Reason	Pre	Post
Extension is not automatically approved if a previous application did not meet the criteria outlined in policy.	30	4
Extensions of pre-NLRB forms for portopulmonary hypertension cannot be auto-approved. Submit a new exception or proceed for NLRB review.	6	0
Heart catheterization date not within 90 days prior to submission.	1	0
Initial pulmonary artery wedge pressure (PAWP) not provided. Initial PVR and TPG cannot be calculated.	2	0
Peak MPAP not less than 35 mmHg.	3	0
Post-treatment MPAP not less than 35 mmHg.	10	0
Post-treatment measurements not within 90 days of submission.	10	1
Post-treatment pulmonary artery wedge pressure (PAWP) not provided. Post-treatment PVR and TPG cannot be calculated.	2	0
Treatment has not been documented.	1	0
Not Reported	8	0
Initial MPAP not greater than or equal to 35 mmHg.	0	5
Initial PVR not greater than or equal to 240 dynes*sec/cm5 or not greater than or equal to 3 Wood units (WU).	0	2
Post-treatment MPAP is greater than or equal to 35 mmHg and less than 45 mmHg and post-treatment PVR is not less than 240 dynes*sec/cm5 or not less than 3 Wood units (WU).	0	5
Post-treatment MPAP is less than 35 mmHg and post-treatment PVR is not less than 400 dynes*sec/cm5 or not less than 5 Wood units (WU).	0	1
Post-treatment mean pulmonary arterial pressure (MPAP) not provided. Post-treatment PVR and TPG cannot be calculated.	0	1
Pulmonary artery wedge pressure (PAWP) not provided. PVR cannot be calculated.	0	1
Total	73	20

Table I.2: Portopulmonary Hypertension Exception Cases Resulting in a Transplant by Era

	Policy Era	
Exception Status	Pre	Post
No Exception	15,008 (78.9%)	17,837 (83.5%)
Other Exception Type	3,953 (20.8%)	3,477 (16.3%)
Portopulmonary Hypertension Exception	66 (0.3%)	55 (0.3%)
Total	19,027 (100.0%)	21,369 (100.0%)

II.Operational Guidelines: Pediatric ART/ART Leader

Summary of Changes:

The improvements to the operational guidelines include creating a separate Appeals Review Team (ART) specifically for pediatric cases and adding an ART leader to each ART. For more explicit information regarding the changes, see the policy notice document here: https://optn.transplant.hrsa.gov/media/4251/further_nlrb_enhancements_202012.pdf.

The guidelines were implemented on 10/07/2021. The guidelines are evaluated 2.5 years following implementation, with no pre-guideline implementation era, since the pediatric appeals review team was created upon implementation of these guidelines. The post-guideline implementation era spanned from October 7, 2021 to April 6, 2024. The following metrics were evaluated:

- Number of pediatric appeals review team cases (overall and by case outcome)

Summary of Results: There were 24 pediatric ART cases in the post-guideline implementation era: 12 were approved or withdrawn prior to decision and 6 were denied. There were no events in the pre-guideline implementation era as the committee was formed upon the implementation date.

Table II.1: Pediatric ART Cases by Outcome between October 7, 2021 and April 6, 2024

Outcome	Cases
Approved	12
Denied	6
Score assigned due to time limit	2
Withdrawn prior to decision	4
Total	24

III.Guidance: Polycystic Liver Disease

Summary of Changes:

The proposal updated the guidance for polycystic liver disease (PLD) to clarify the MELD score recommendation, provide guidance for candidates also requiring a kidney, and add new comorbidities that should be considered for a MELD exception in conjunction with PLD. For more explicit information regarding the changes, see the policy notice document here: https://optn.transplant.hrsa.gov/media/4251/further_nlrb_enhancements_202012.pdf.

The guidance was implemented on February 9, 2021. The guidance is evaluated 3 years following implementation, with the pre-guidance era spanning from February 10, 2018 to February 8, 2021 and post-guidance era spanning from February 9, 2021 to February 9, 2024. After this report, this section will be removed as the 3-year monitoring period has ended. The following metrics were evaluated:

- Number of exception cases for polycystic liver disease/polycystic liver and kidney disease (overall, by case outcome, by application type, and by liver-alone/liver-kidney registration status)
- Number of transplant recipients with polycystic liver disease/polycystic liver and kidney disease

Summary of Results:

Results are summarized by polycystic liver disease for liver alone candidates and polycystic liver and kidney disease for candidates that ever had a liver registration overlap with a kidney registration. There was a decrease in extension exception cases for polycystic liver disease and polycystic liver and kidney disease. All cases of polycystic liver disease or polycystic liver and kidney disease not withdrawn prior to review were reviewed by the National Review Board or Pediatric Review Board in the post-guidance era. The proportion of cases approved increased in the post-guidance era for initial and extension polycystic liver disease cases and extension polycystic liver and kidney disease cases. Overall, the proportion of liver alone transplant recipients with an exception for polycystic liver disease increased for both initial and extension forms. The proportion of simultaneous liver kidney (SLK) transplants with a polycystic liver and kidney disease exception decreased for initial forms and increased for extension forms in the post-guidance era.

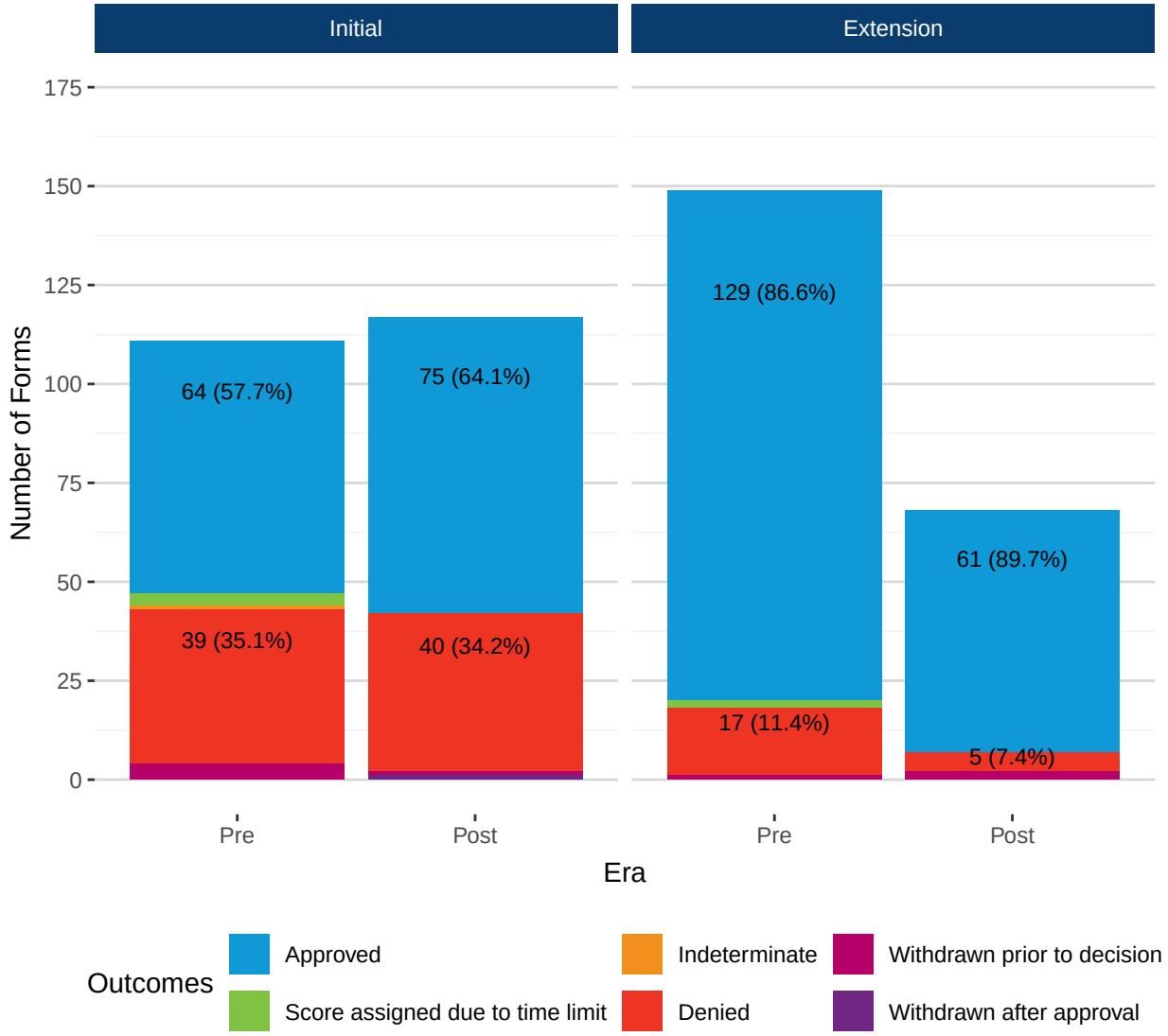
Figure III.1: Polycystic Liver Disease Exception Cases by Application and Review Type

There was 1 initial form withdrawn prior to review board assignment and 1 sent to the Pediatric Review Board in the post−guidance era not labelled here.

Figure III.2: Polycystic Liver and Kidney Disease Exception Cases by Application and Review Type

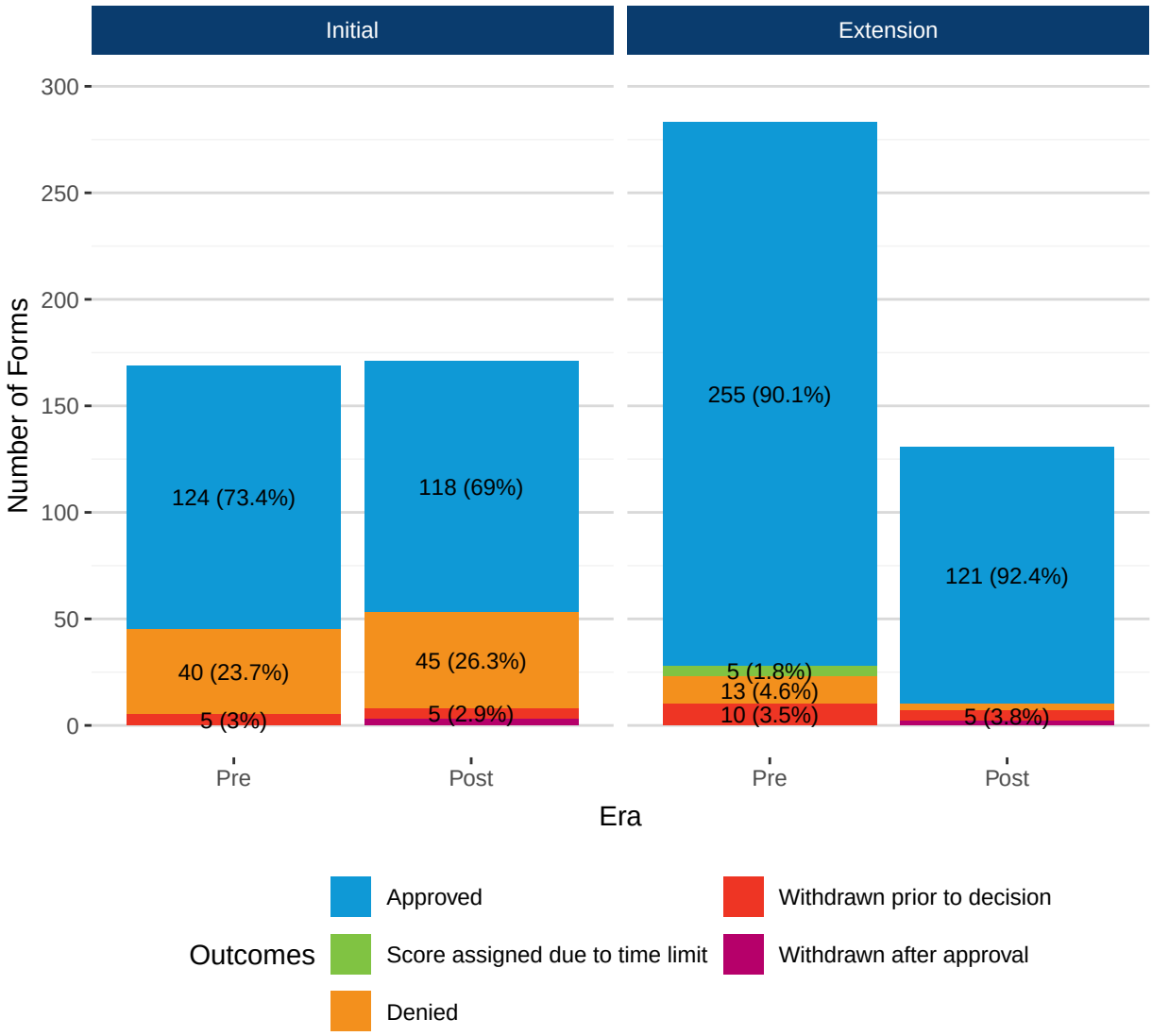
There was 1 initial form withdrawn prior to review board assignment in the post−guidance era not labelled here.

Figure III.3: Polycystic Liver Disease Exception Cases by Outcome



Labels were removed for categories with less than 5 observations. The full labels can be seen in the Liver Alone section of Table III.1.

Figure III.4: Polycystic Liver and Kidney Disease Exception Cases by Outcome



els were removed for categories with less than 5 observations. The full labels can be seen in the Liver−Kidney section of Table III.1.

Table III.1: Polycystic Liver Disease and Polycystic Kidney and Liver Disease Exception Cases by Application and Outcome

		Liver Alone		Liver-Kidney	
Application Type	Outcome	Pre	Post	Pre	Post
Initial	Approved	64 (58%)	75 (64%)	124 (73.37%)	118 (69.01%)
	Score assigned due to time limit	3 (3%)	0 (0.0%)	0 (0%)	0 (0%)
	Indeterminate	1 (1%)	0 (0.0%)	0 (0%)	0 (0%)
	Denied	39 (35%)	40 (34%)	40 (23.67%)	45 (26.32%)
	Withdrawn prior to decision	4 (4%)	1 (1%)	5 (2.96%)	5 (2.92%)
	Withdrawn after approval	0 (0.0%)	1 (1%)	0 (0%)	3 (1.75%)
Extension	Approved	129 (87%)	61 (90%)	255 (90.11%)	121 (92.37%)
	Score assigned due to time limit	2 (1%)	0 (0.0%)	5 (1.77%)	0 (0%)
	Denied	17 (11%)	5 (7%)	13 (4.59%)	3 (2.29%)
	Withdrawn prior to decision	1 (1%)	2 (3%)	10 (3.53%)	5 (3.82%)

Table III.2: Summary of Refusal Comments Provided for Polycystic Liver Disease and Polycycstic Liver and Kidney Disease Exception Form Denied

Each polycystic liver disease or polycystic liver and kidney disease exception request form that is sent to the NLRB for review is reviewed by five randomly selected members of the NLRB Other Diagnosis Board. Forms denied have been refused by at least two of the randomly selected reviewing members, but as many as all five reviewers could indicate a refusal of the exception in the form. Justification for refusal is provided through a free text field by reviewers. The number of refusal comments provided varies per case.

Below the sentiment of reviewers' comments is summarized by form. Sentiments were summarized into one of the three following groups based on main sentiment conveyed:

- Comments that gave any indication that the requested exception score was too high were categorized as "Score Requested Is Too High"
- Comments that referenced the guidance, criteria, or guidelines put forward by the OPTN is not met in some way, excluding those that noted the score requested was to high were categorized as "Guidelines, Criteria or Guidance Referenced in Refusal"
- Comments that indicated neither of the two categories above were categorized as "Other" - comments categorized into 'Other' often referenced the need for additional information to support approving an exception status for the candidate.

Sentiments are summarized in a way that each sentiment only appears once per form if indicated by a reviewer (for example, when two reviewers indicate 'Score Requested Is Too High' and one reviewer indicates 'Other', the 'Score Requested Is Too High' sentiment will be recorded once and the 'Other' sentiment will be recorded once for that form). As a result, the percentages seen will not sum to 100%, however the percentages seen in the table below can be interpreted as the proportion of polycystic liver disease or polycystic liver and kidney disease exception forms denied with that sentiment indicated by at least one reviewer.

		Era	
Exception Type	Refusal Comment Sentiment	Pre	Post
Polycystic Liver Disease	Guidelines, Criteria or Guidance Referenced in Refusal	25 (44.64%)	29 (64.44%)
	Other	34 (60.71%)	31 (68.89%)
	Score Requested Is Too High	15 (26.79%)	8 (17.78%)
	Total Forms Denied	56	45
Polycystic Liver and Kidney Disease	Guidelines, Criteria or Guidance Referenced in Refusal	10 (18.87%)	20 (41.67%)
	Other	25 (47.17%)	32 (66.67%)
	Score Requested Is Too High	19 (35.85%)	21 (43.75%)
	Total Forms Denied	53	48

Figure III.5: Polycystic Liver Disease and Polycystic Liver and Kidney Disease Transplant Recipients by Transplant Type and Exception Case

Results: Updating National Liver Review Board Guidance and Policy Clarification (January 2021 PC)

IV.Guidance: Pediatric Updates

Summary of Changes:

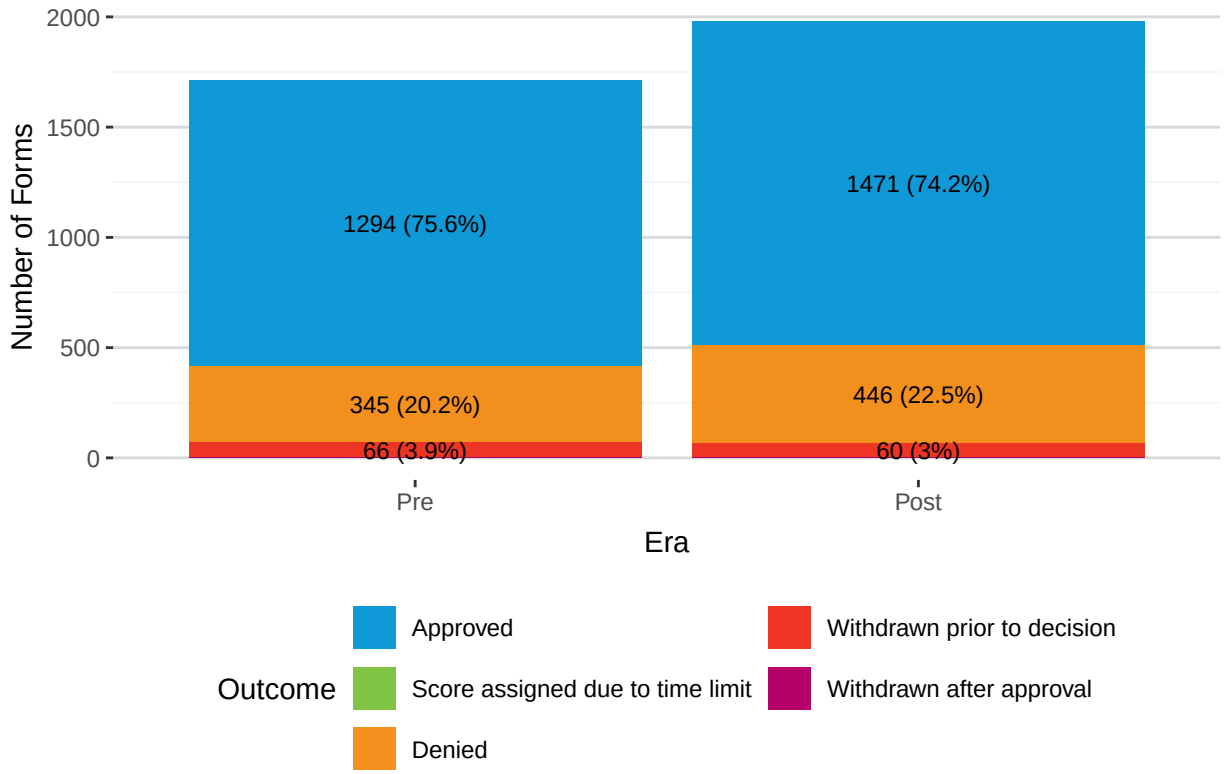
This guidance document included expanded guidance for candidates with growth failure or nutritional insufficiency; added language that outlines information that should be submitted for candidates with gastrointestinal bleeding with ongoing transfusion requirements, and information that should be submitted for candidates with serum sodium less than 130 g/dL on two occasions more than two weeks apart; added language that states a candidate should have at least two thoracenteses in the last 60 days not including the diagnostic thoracentesis; added guidance that recommends an exception for candidates requiring a hospitalization of at least five days with ascites not adequately controlled by oral diuretics and requiring IV diuretic therapy; added guidance for candidates with rare metabolic disorders; updated conclusion section to allow submission and consideration of clinical details not currently included in guidance. For more explicit information regarding the changes, see the policy notice document here: https://optn.transplant.hrsa.gov/media/4694/updating_nlrb_guidance_policy_clarification_june_2021_policy_notice.pdf.

The guidance was implemented on July 15, 2021. The guidance is evaluated 3 years following implementation, with the pre-guidance era spanning from July 16, 2018 to July 14, 2021 and post-guidance era spanning from July 15, 2021 to July 14, 2024. After this report, this section will be removed as the 3-year monitoring period has ended. The following metrics were evaluated:

- Number and percent of pediatric exception requests (overall and by case outcome)

Summary of Results: The number of pediatric exception cases and the proportion of pediatric cases approved remained consistent across eras. It is critical to note the number of forms submitted is larger than the number of candidates requesting an exception as candidates can submit multiple forms to apply for or extend an exception status over their time on the waiting list.

Figure IV.1: Pediatric Cases by Outcome



There were 4 cases pre−guidance and 1 case post−guidance era where a score was assigned due to the time limit and 2 cases pre−guidance and 3 cases post−guidance era where the form was withdrawn after approval, not labelled here

V.Policy Language: Hilar CCA Standardized Exception Criteria

Summary of Changes:

Changes to this policy include expanded diagnostic criteria for standardized exceptions to include candidates with a hilar mass less than three centimeters in radial diameter. For more explicit information regarding the changes, see the policy notice document here: https://optn.transplant.hrsa.gov/media/4694/updating_nlrb_guidance_policy_clarification_june_2021_policy_notice.pdf.

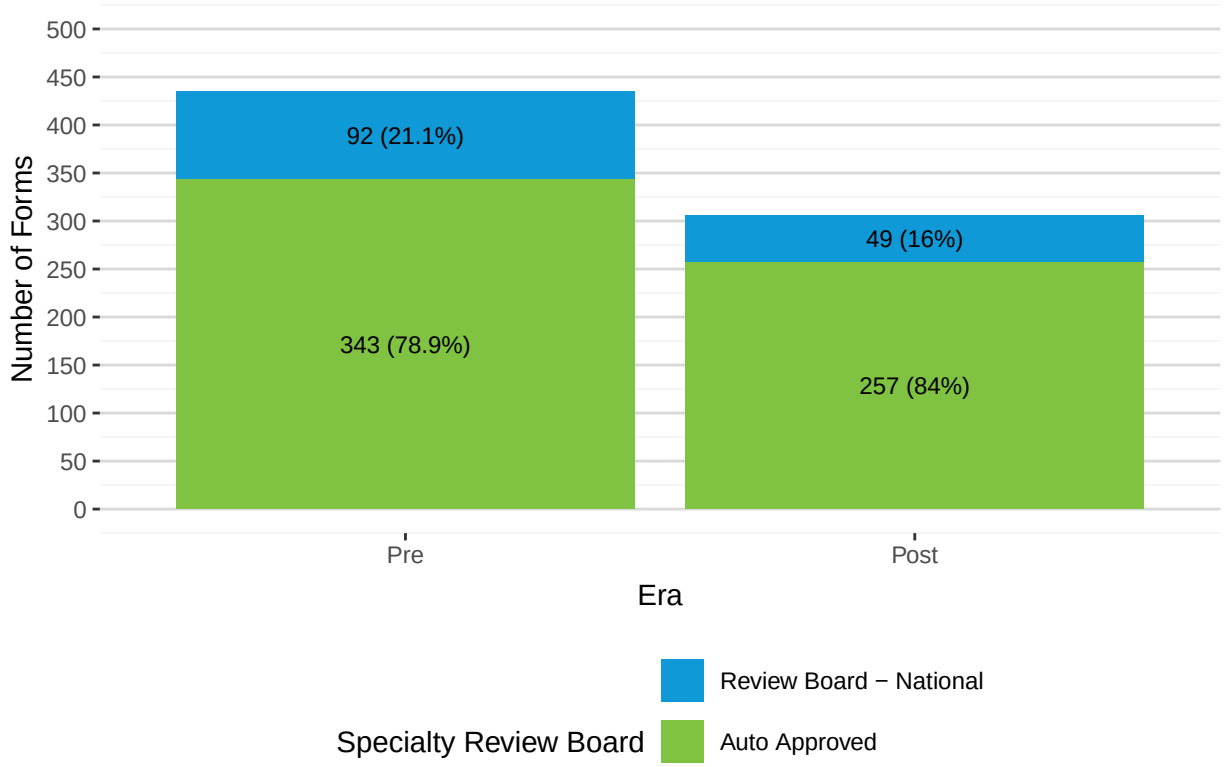
The policy was implemented on October 7, 2021. The policy is evaluated 2.5 years following implementation, with the pre-policy era spanning from April 9, 2019 to October 6, 2021 and post-policy era spanning from October 7, 2021 to April 6, 2024. The following metrics were evaluated:

- Number and percent of Hilar CCA exceptions meeting standard policy criteria versus requiring NLRB review

Summary of Results:

Exception cases evaluated below include all exception forms under Cholangiocarcinoma (CCA) diagnosis. The number of exception forms for CCA decreased in the post-policy era. The proportion of exception cases that were NLRB reviewed also decreased.

Figure V.1: CCA Cases by Review Type



VI.Guidance: Neuroendocrine Tumors (NET)

Summary of Changes:

Changes to this guidance include clarifying language and removing the recommendation that candidates be less than 60 years old. For more explicit information regarding the changes, see the policy notice document here: https://optn.transplant.hrsa.gov/media/4694/updating_nlrb_guidance_policy_clarification_june_2021_policy_notice.pdf.

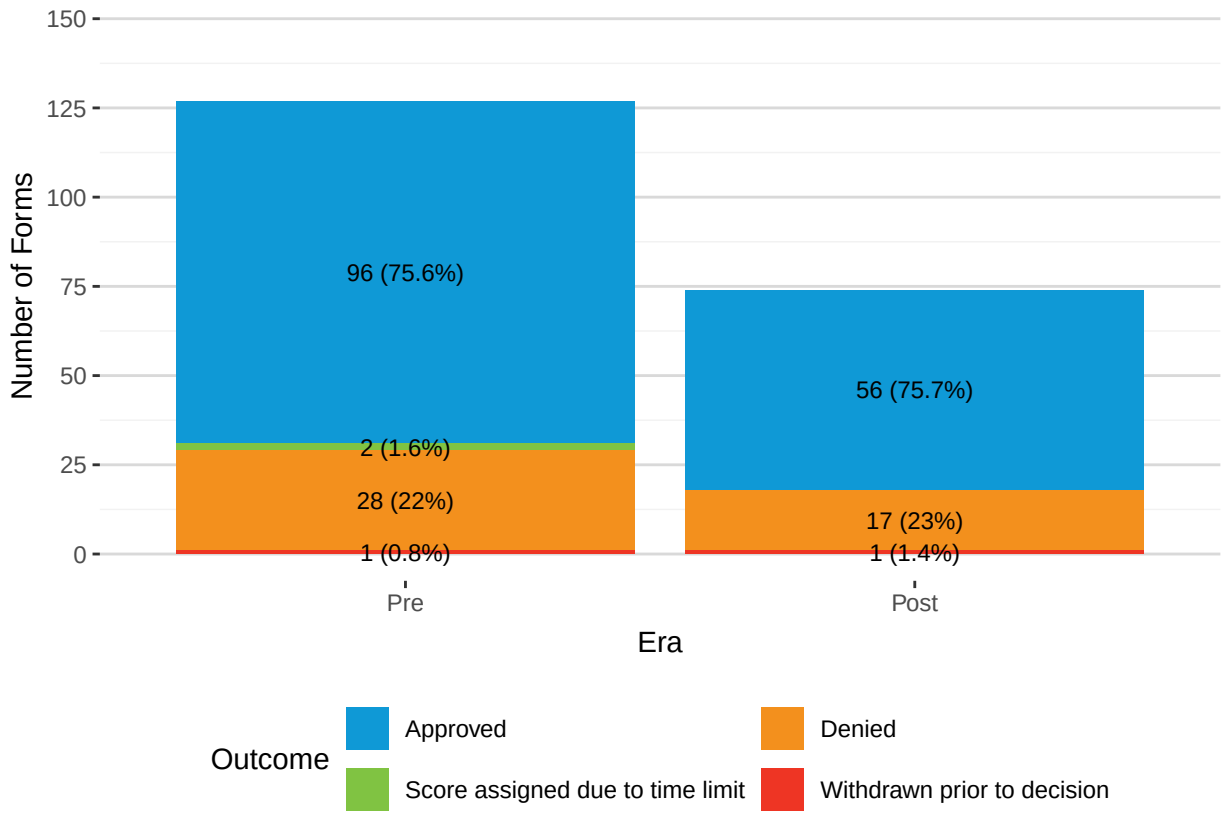
The guidance was implemented on July 15, 2021. The guidance is evaluated 3 years following implementation, with the pre-guidance era spanning from July 16, 2018 to July 14, 2021 and post-guidance era spanning from July 15, 2021 to July 14, 2024. After this report, this section will be removed as the 3-year monitoring period has ended. The following metrics were evaluated:

- Number of exception cases for NET (overall and by case outcome)

Summary of Results:

The number of exception cases for candidates with neuroendocrine tumors decreased in the post-guidance era and the proportion of those approved remained relatively steady.

Figure VI.1: Neuroendocrine Tumors Exception Cases by Case Outcome



VII.Guidance: Primary Sclerosing Cholangitis and Secondary Sclerosing Cholangitis

Summary of Changes:

Guidance includes recommendations that candidates be admitted to hospital two or more times in the previous year with a documented blood stream infection or evidence of sepsis including hemodynamic instability requiring vasopressors. For more explicit information regarding the changes, see the policy notice document here: https://optn.transplant.hrsa.gov/media/4694/updating_nlrb_guidance_policy_clarification_june_2021_policy_notice.pdf.

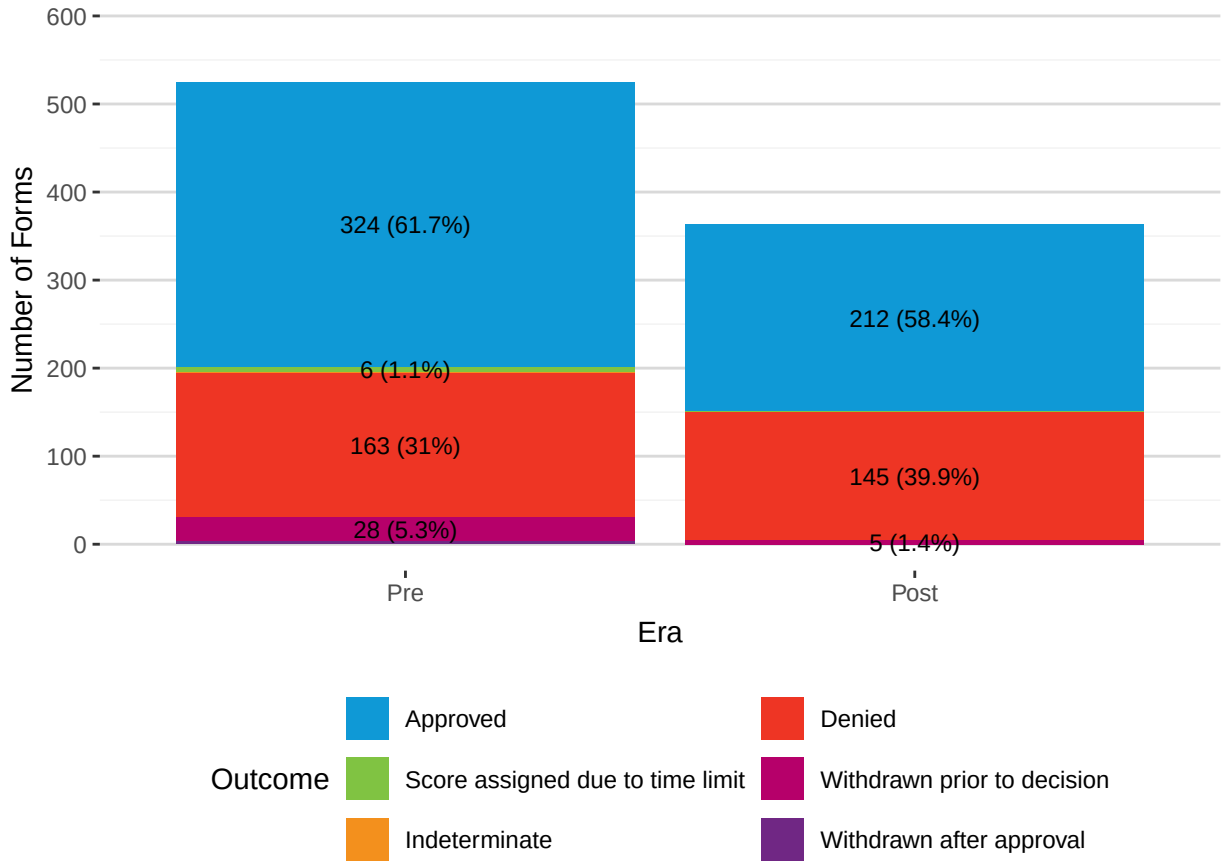
The guidance was implemented on July 15, 2021. The guidance is evaluated 3 years following implementation, with the pre-guidance era spanning from July 16, 2018 to July 14, 2021 and post-guidance era spanning from July 15, 2021 to July 14, 2024. After this report, this section will be removed as the 3-year monitoring period has ended. The following metrics were evaluated.

- Number of exception cases for PSC/SSC (overall and by case outcome)

Summary of Results:

The number of cases regarding primary sclerosing cholangitis or secondary sclerosing cholangitis increased in the post-guidance era. The proportion of cases accepted decreased slightly. For forms denied, the proportion of forms with a refusal sentiment citing guidelines, criteria, or guidance in refusal comments increased.

Figure VII.1: Primary Sclerosing Cholangitis and Secondary Sclerosing Cholangitis Exception Cases by Case Outcome



There were 3 forms in the pre−policy period that were withdrawn after approval, 1 form in the pre−policy period was indeterminate, and 1 form in the post−policy period that had a score assigned due to time limit, all not labelled in the figure

Table VII.1: Summary of Refusal Comments Provided for Primary Sclerosing Cholangitis and Secondary Sclerosing Cholangitis Exception Forms Denied

Each PSC or SSC exception request form that is sent to the NLRB for review is reviewed by five randomly selected members of the NLRB Other Diagnosis board. Forms denied have been refused by at least two of the randomly selected reviewing members, but as many as all five reviewers could indicate a refusal of an exception for the case. Justification for refusal is provided through a free text field by reviewer. The number of refusal comments provided varies per case.

Below the sentiment of reviewers' comments is summarized by form. Sentiments were summarized into one of the three following groups based on main sentiment conveyed:

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Sentiments are summarized in a way that each sentiment only appears once per case if indicated by a reviewer (for example in the case where two reviewers could indicate 'Score Requested Is Too High' and one reviewer indicated 'Other', the 'Score Requested Is Too High' sentiment will be recorded once and the 'Other' sentiment will be recorded once for that case). As a result, the percentages seen will not sum to 100%; however the percentages seen in the table below can be interpreted as the proportion of PSC/SCS forms denied with that sentiment indicated by at least one reviewer.

Refusal Comment Sentiment	Pre	Post
Guidelines, Criteria or Guidance Referenced in Refusal	63 (38.65%)	71 (48.97%)
Other	91 (55.83%)	104 (71.72%)
Score Requested Is Too High	39 (23.93%)	45 (31.03%)
Total Forms Denied	163	145