

1 Year Monitoring Report Pediatric National Heart Review Board

DHHS Contract No. 250-2019-00001C
Date Completed: 12/20/2022

Prepared for:
Heart Committee
Committee Meeting
Date of Meeting: 12/20/2022

By:
Keighly Bradbrook, PhD // Jesse Howell, MPH
UNOS Research Department

Contents

Background/Purpose	3
Strategic Plan Goal or Committee Project Addressed	3
Committee Request	3
Data and Methods	3
Results	5
Figure 1. Map of Heart Programs with an Approved Pediatric Component	5
Waiting List	6
Figure 2. Waitlist additions by medical urgency status and era	6
Figure 3. Waitlist additions by age at listing and era	7
Figure 4. Waitlist additions by medical urgency status, age at listing and era	8
Table 1. Waitlist additions by medical urgency status, age at listing and era	9
Figure 5. Waitlist additions by OPTN region and era	10
Figure 6. Waitlist additions by medical urgency status and OPTN region	11
Table 2. Waitlist additions by medical urgency status and OPTN region	12
Figure 7. Waitlist additions by diagnosis and era	13
Table 3. Waitlist additions by diagnosis and era	13
Figure 8. Waitlist additions by medical urgency status, diagnosis and era	14
Table 4. Waitlist additions by medical urgency status, diagnosis and era	15
Figure 9. Waitlist additions by exception vs. standard criteria at listing and era	16
Figure 10. Waitlist additions by medical urgency status, exception vs. standard criteria and era	17
Figure 11. Waitlist additions by exception vs. standard criteria and OPTN region	18
Table 5. Waitlist additions by exception vs. standard criteria and OPTN region	19
Transplants	20
Figure 12. Transplants by medical urgency status and era	20
Table 6. Transplants by medical urgency status and era	20

Figure 13. Transplants by age at transplant and era	21
Figure 14. Transplants by medical urgency status, age at transplant, and era	22
Figure 15. Transplants by OPTN region and era	23
Figure 16. Transplants by medical urgency status and OPTN region	24
Table 7. Transplants by medical urgency status and OPTN region	25
Figure 17. Transplants by diagnosis at transplant and era	26
Table 8. Transplants by diagnosis at transplant and era	26
Figure 18. Transplants by medical urgency status, diagnosis at transplant and era	27
Table 9. Transplants by medical urgency status, diagnosis at transplant and era	28
Figure 19. Transplants by exception vs. standard criteria at transplant and era	29
Figure 20. Transplants by medical urgency status, exception vs. standard criteria and era	30
Figure 21. Transplants by exception vs. standard criteria at Transplant and OPTN region	31
Table 10. Transplants by exception vs. standard criteria at Transplant and OPTN region	32
Exception Requests	33
Survival Analysis	34
Figure 22. Post-transplant patient survival rates overall	34
Table 11. Post-transplant patient survival rates overall	34
Figure 23. Post-transplant patient survival rates stratified by status	35
Table 12. Post-transplant patient survival rates stratified by status	35
Rates	36
Figure 24. Waitlist mortality rate	36
Table 13. Waitlist mortality rate	37
Figure 25. Waitlist transplant rate	38
Table 14. Waitlist transplant rate	39
Summary	40

Background/Purpose

Before the implementation of the pediatric National Heart Review Board (NHRB) on June 15, 2021, Regional Review Boards (RRBs) handled and reviewed exception cases for pediatric candidates listed before their 18th birthday. The purpose of the NHRB was to improve quality and consistency in evaluating exceptions for heart candidates listed before their 18th birthday. Pediatric heart candidates can be listed as Status 1A, Status 1B, Status 2 or Inactive. Active pediatric candidates not meeting the criteria for Statuses 1A and 1B are put in Status 2 by default. The NHRB is comprised of representatives from pediatric heart programs from across the country. Reviewers are randomly assigned to review exception requests.

Strategic Plan Goal or Committee Project Addressed

1. Improve equity in access to transplants.
2. Improve waitlisted patient, living donor, and transplant recipient outcomes.

Committee Request

As outlined in the monitoring plan in the board briefing report, the Committee will monitor metrics as they relate to the pediatric NHRB. This includes, but is not limited to:

- Examine changes in the number and percent of pediatric candidates by status, exception, age group, OPTN region, and diagnosis
- Examine changes in the number and percent of pediatric transplant recipients by status, exception, age group, OPTN region, and diagnosis
- Evaluate changes in waiting list mortality rate for pediatric candidates by status and exception
- Evaluate changes in transplant rate for pediatric candidates by status and exception
- Report the percent of approvals and denials for exception requests by status
- Examine changes in post-transplant patient survival rates overall and stratified by status

This policy will be formally evaluated approximately 6 months, 1 year, and 2 years post-implementation. The aforementioned metrics, and any subsequently requested by the committee, will be evaluated as data become available (Appropriate lags will be applied, per typical UNOS conventions, to account for time delay in institutions reporting data to UNet)

Data and Methods

Data Sources:

These analyses use data from the OPTN waiting list, the Deceased Donor Registration (DDR) form, the Transplant Candidate Registration (TCR) form, and the Transplant Recipient Registration (TRR) form.

Analyses are based on OPTN data as of December 16, 2022 and are subject to change based on future data submission or correction.

Methods:

Pediatric (age < 18 at listing) candidates added to the heart waiting list between June 15, 2020 and June 14, 2021 (pre) or between June 15, 2021 and June 14, 2022 (post) were stratified by medical urgency status, exception vs standard criteria, age group, OPTN region, and diagnosis. For the diagnosis section, the "other" category contains valvular heart disease, muscular dystrophy: other specify, arrhythmogenic right ventricular dysplasia, COVID-19: dilated myopathy, cancer, and the other category. The acronym CHD stands for congenital heart defect and CAD stands for coronary artery disease.

Pediatric (age < 18 at transplant) heart recipients transplanted between June 15, 2020 and June 14, 2021 (pre) or between June 15, 2021 and June 14, 2022 (post) were stratified by medical urgency status, exception vs standard

criteria, age group, OPTN region, and diagnosis. The “other” category is defined the same as with waiting list candidates above.

Waiting list mortality rates and transplant rates were calculated based on a cohort of pediatric (age < 18) candidates ever waiting between June 15, 2020 and June 14, 2021 (pre) or between June 15, 2021 and June 14, 2022 (post). Rates were calculated as the ratio of death or transplant to patient-years of exposure, and are displayed as deaths or transplants per 100 patient-years. The OPTN database was supplemented with deaths from verified external sources. Since candidates may be removed from the waiting list shortly prior to death as their health deteriorates, the waiting list mortality rate calculation included deaths within seven days of waiting list removal and those removed from the waiting list as a result of becoming too sick to transplant. Exceptions were calculated by whether the candidate ever had an exception in each policy era. Candidates who received any previous transplant were excluded from the waiting list mortality and transplant rate analyses.

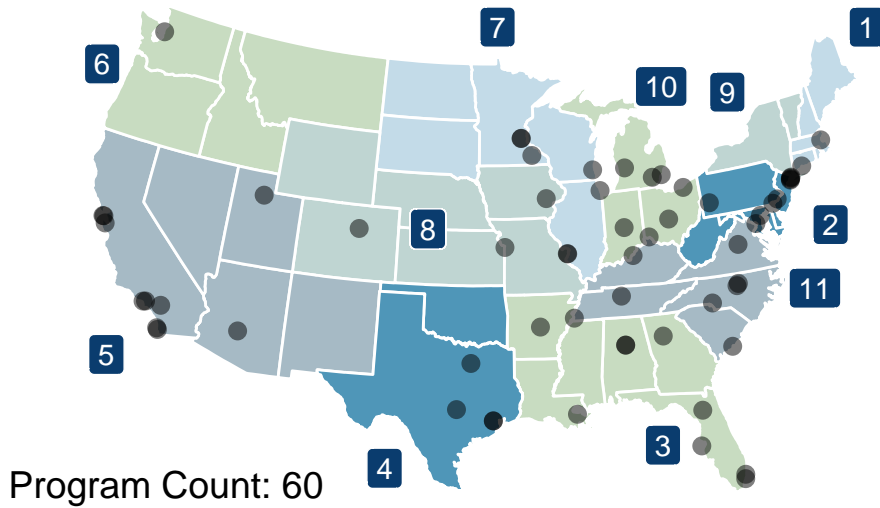
Outcomes analyses were performed on a subset of pediatric (age < 18 at transplant) heart transplant recipients with the potential for at least six months of follow-up plus a two-month data lag, which included recipients transplanted between December 14, 2020 and June 14, 2021 in the pre-implementation cohort and between June 15, 2021 and December 14, 2021 in the post-implementation cohort. 6 month survival curves were constructed using unadjusted Kaplan-Meier methodology and compared using the log-rank test.

Statistical analyses were performed using SAS v9.3 (SAS Institute, Inc., Cary, NC.) and R Version 4.1.3 (R: A language and environment for statistical computing. R Foundation for Statistical Computing, Vienna, Austria. URL: <https://www.R-project.org/>).

Results

At the same time that the ped NHRB was implemented the pediatric bylaws also went into effect. The bylaws established requirements for pediatric components and minimum qualifications for primary pediatric transplant surgeons and physicians for kidney, liver, pancreas, heart and lung programs. Figure 1 shows all programs with an approved pediatric program per the OPTN pediatric bylaws both pre and post ped NHRB implementation. There were a total of 59 active programs with approved pediatric components as of December 16, 2022.

Figure 1. Map of Heart Programs with an Approved Pediatric Component

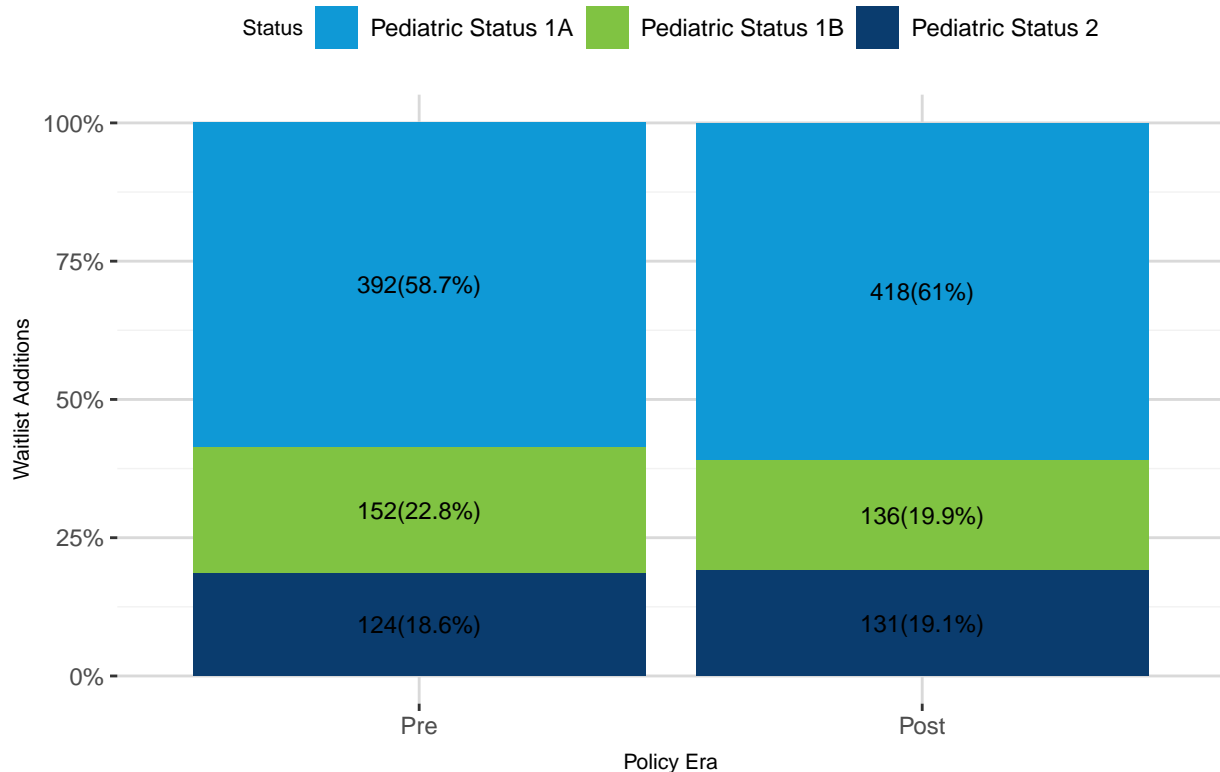


Waiting List

The following metrics summarize waiting list additions by medical urgency status, exception vs. standard criteria, age group, OPTN region, and diagnosis.

Figure 2 shows pediatric waitlist additions by medical urgency status and era. There is slight variation between the statuses in the pre and post-policy eras. There was an increase in the percent of status 1A waitlist additions from 58.7% (n=392) in the pre-policy era to 61% (n=418) in the post-policy era. There was a decrease in the percent of status 1B waitlist additions from 22.8% (n=152) in the pre-policy era to 19.9% (n=136) in the post-policy era.

Figure 2. Waitlist additions by medical urgency status and era



Note: Only categories with more than 5% are labelled.
Temporarily inactive medical urgency status was removed from this figure (n=14)

Figure 3 shows the pediatric waitlist additions by age at listing and era. The pre and post-policy eras had a similar distribution across the age groups.

Figure 3. Waitlist additions by age at listing and era

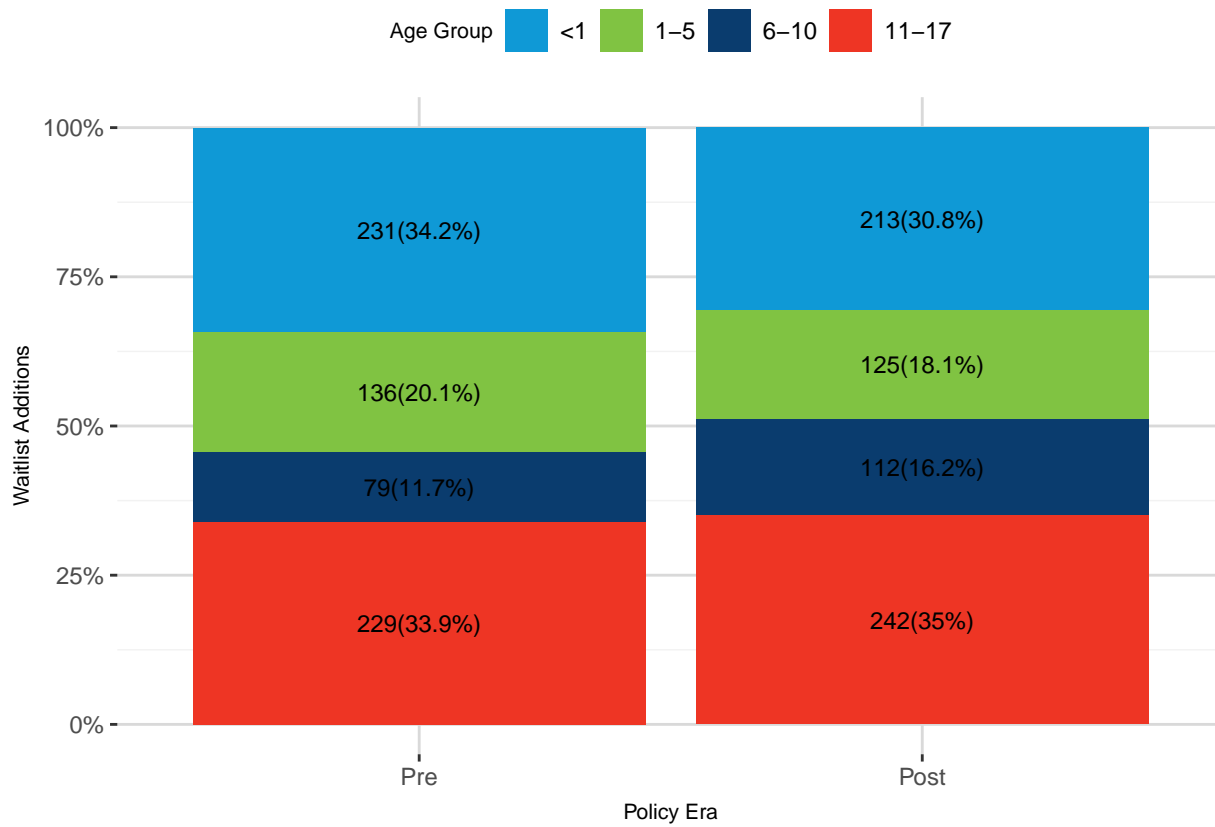
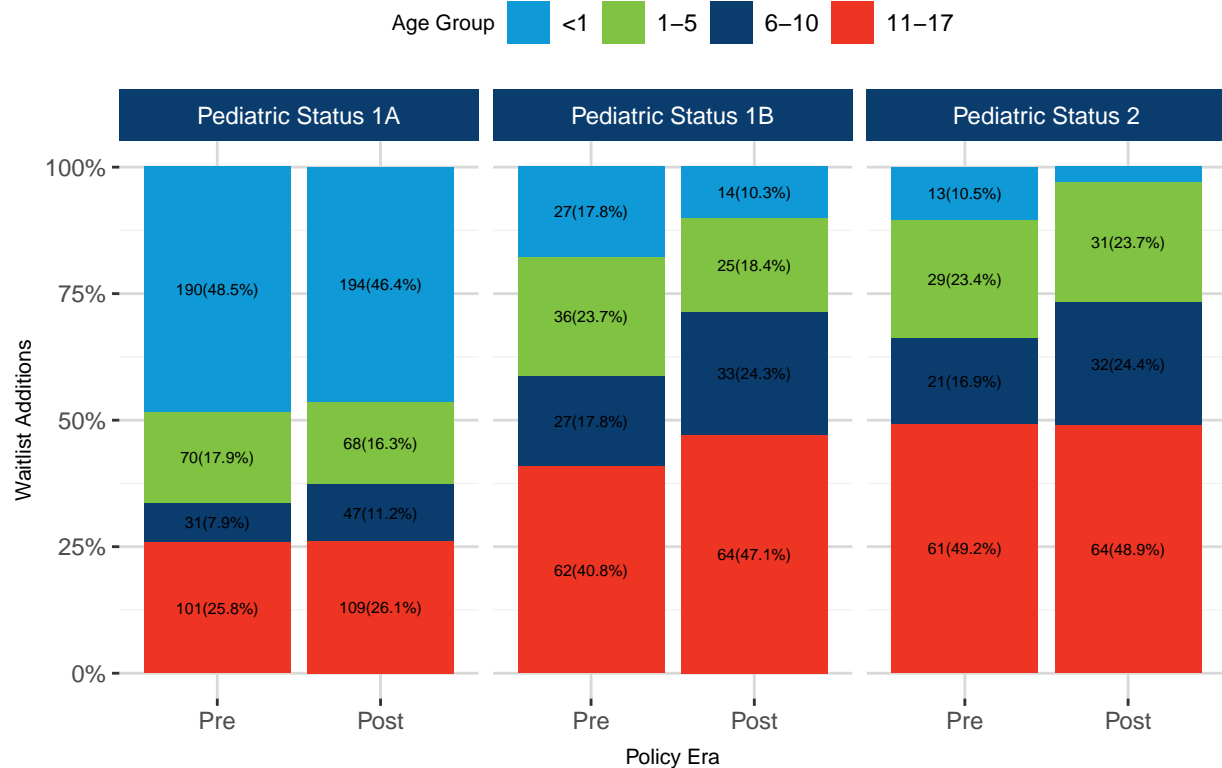


Figure 4 and table 1 show pediatric waitlist additions by medical urgency status, age at listing, and era. Pediatric status 1A candidates were more likely to be in the <1 age group, while pediatric status 1B and status 2 candidates were more likely to be in the 11-17 age group, followed by the 6-10 age group. In the post-policy era, there was a slightly higher percent of candidates in the 6-10 age group in status 1A, from 7.9% (n=31) in the pre-policy era to 11.2% (n=47) in the post-policy era. Pediatric status 1B had a slightly higher percent of waitlist additions in the 11-17 and 6-10 age groups in the post-policy era. Pediatric status 2 had a slightly higher percent of waitlist additions in the 6-10 age group in the post-policy era.

Figure 4. Waitlist additions by medical urgency status, age at listing and era



Note: Only categories with more than 5% are labelled. Temporarily inactive medical urgency status was removed from this figure (n=14)

Table 1. Waitlist additions by medical urgency status, age at listing and era

Status	Age Group	Pre	Post
Pediatric Status 1A	<1	190 (48.5%)	194 (46.4%)
	1-5	70 (17.9%)	68 (16.3%)
	6-10	31 (7.9%)	47 (11.2%)
	11-17	101 (25.8%)	109 (26.1%)
Pediatric Status 1B	<1	27 (17.8%)	14 (10.3%)
	1-5	36 (23.7%)	25 (18.4%)
	6-10	27 (17.8%)	33 (24.3%)
	11-17	62 (40.8%)	64 (47.1%)
Pediatric Status 2	<1	13 (10.5%)	4 (3.1%)
	1-5	29 (23.4%)	31 (23.7%)
	6-10	21 (16.9%)	32 (24.4%)
	11-17	61 (49.2%)	64 (48.9%)

Note:

Temporarily inactive medical urgency status was removed from this table (n=14)

Figure 5 shows the pediatric waitlist additions by OPTN region and era. Most regions recorded a positive change between eras. Region 9 had the highest positive percent change in pediatric waiting list additions from pre-policy to post-policy with a difference of 35.3%. Region 3 had the highest negative percent change in pediatric waiting list additions from pre-policy to post-policy with a difference of -19.1%.

Figure 5. Waitlist additions by OPTN region and era

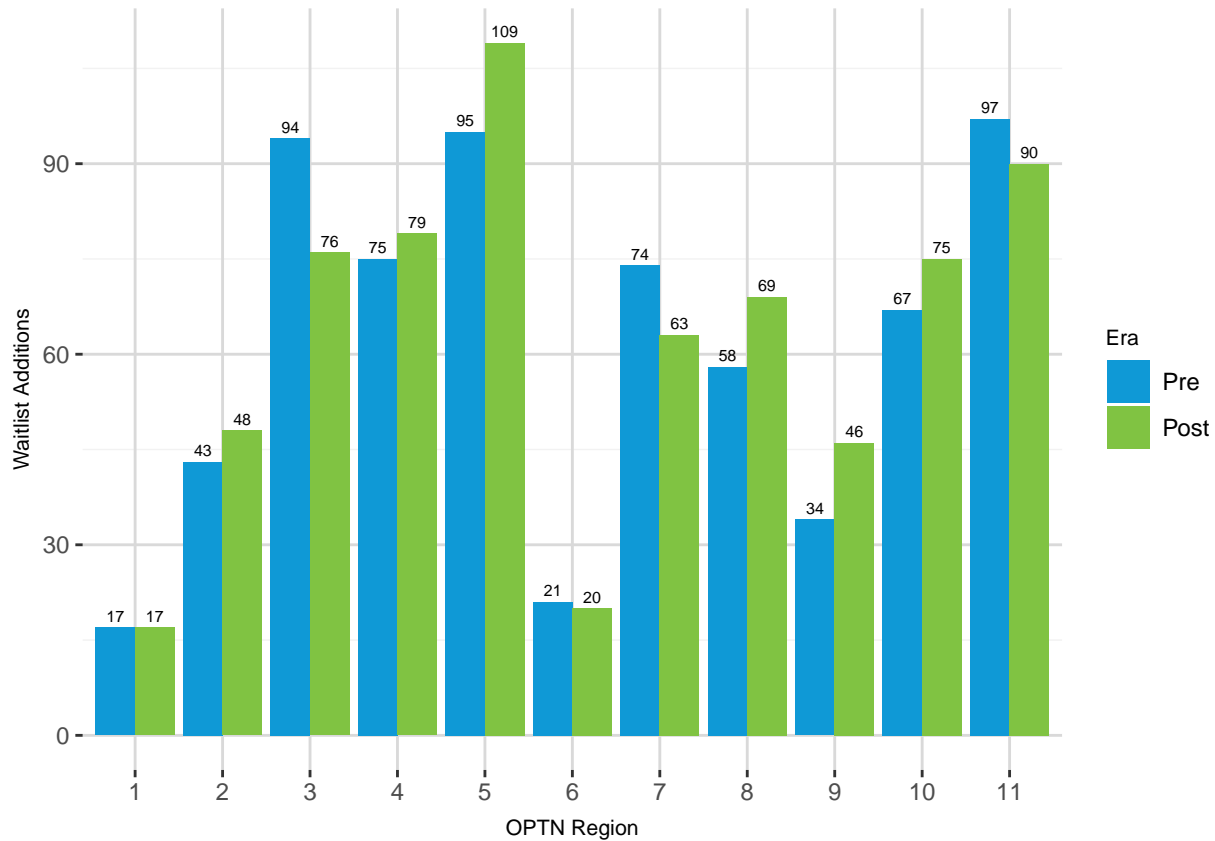
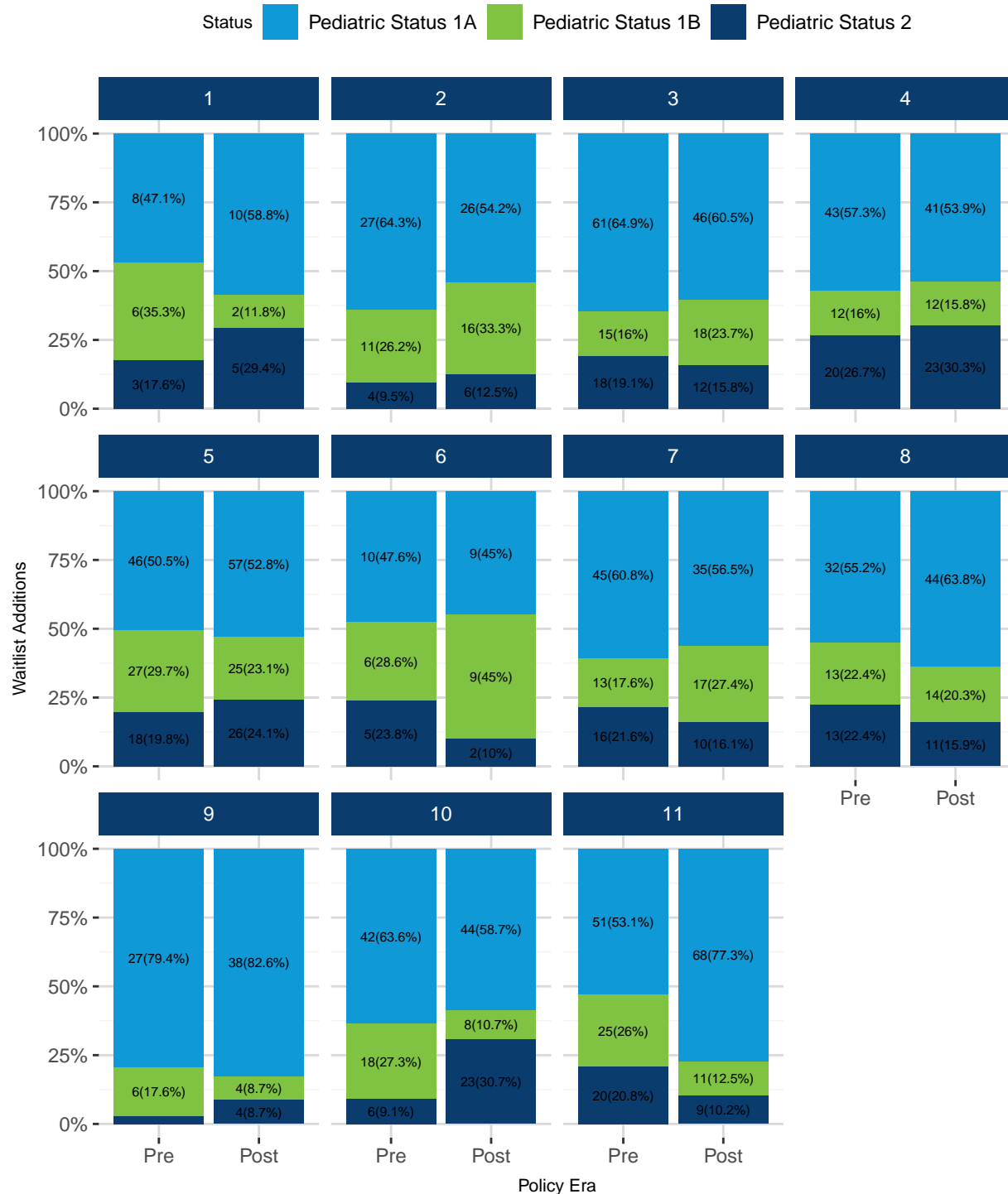


Figure 6 and table 2 show pediatric waitlist additions by medical urgency status and OPTN region. Most regions had a decrease in pediatric status 1A waitlist additions. Region 2 had the greatest percent decrease from 64.3% in the pre-policy era to 54.2% in the post-policy era. Over half of the regions recorded a percent increase in pediatric status 2 waitlist additions from the pre-policy to the post-policy era.

Figure 6. Waitlist additions by medical urgency status and OPTN region



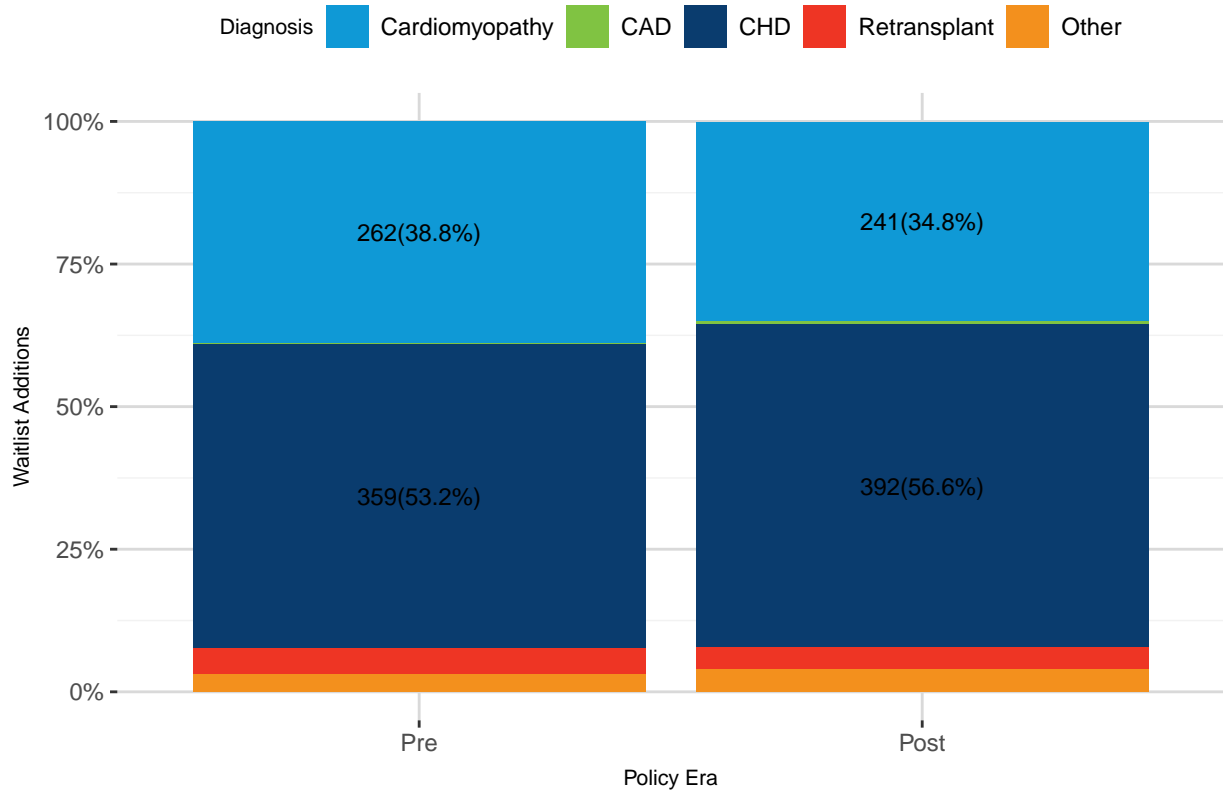
Note: Only categories with more than 5% are labelled. Temporarily inactive medical urgency status was removed from this figure (n=14)

Table 2. Waitlist additions by medical urgency status and OPTN region

OPTN Region	Status	Pre	Post
1	Pediatric Status 1A	8(47.1%)	10(58.8%)
	Pediatric Status 1B	6(35.3%)	2(11.8%)
	Pediatric Status 2	3(17.6%)	5(29.4%)
2	Pediatric Status 1A	27(64.3%)	26(54.2%)
	Pediatric Status 1B	11(26.2%)	16(33.3%)
	Pediatric Status 2	4(9.5%)	6(12.5%)
3	Pediatric Status 1A	61(64.9%)	46(60.5%)
	Pediatric Status 1B	15(16%)	18(23.7%)
	Pediatric Status 2	18(19.1%)	12(15.8%)
4	Pediatric Status 1A	43(57.3%)	41(53.9%)
	Pediatric Status 1B	12(16%)	12(15.8%)
	Pediatric Status 2	20(26.7%)	23(30.3%)
5	Pediatric Status 1A	46(50.5%)	57(52.8%)
	Pediatric Status 1B	27(29.7%)	25(23.1%)
	Pediatric Status 2	18(19.8%)	26(24.1%)
6	Pediatric Status 1A	10(47.6%)	9(45%)
	Pediatric Status 1B	6(28.6%)	9(45%)
	Pediatric Status 2	5(23.8%)	2(10%)
7	Pediatric Status 1A	45(60.8%)	35(56.5%)
	Pediatric Status 1B	13(17.6%)	17(27.4%)
	Pediatric Status 2	16(21.6%)	10(16.1%)
8	Pediatric Status 1A	32(55.2%)	44(63.8%)
	Pediatric Status 1B	13(22.4%)	14(20.3%)
	Pediatric Status 2	13(22.4%)	11(15.9%)
9	Pediatric Status 1A	27(79.4%)	38(82.6%)
	Pediatric Status 1B	6(17.6%)	4(8.7%)
	Pediatric Status 2	1(2.9%)	4(8.7%)
10	Pediatric Status 1A	42(63.6%)	44(58.7%)
	Pediatric Status 1B	18(27.3%)	8(10.7%)
	Pediatric Status 2	6(9.1%)	23(30.7%)
11	Pediatric Status 1A	51(53.1%)	68(77.3%)
	Pediatric Status 1B	25(26%)	11(12.5%)
	Pediatric Status 2	20(20.8%)	9(10.2%)

Figure 7 and table 3 show the pediatric waitlist additions by diagnosis and era. There was a small drop in waitlist additions reporting a primary diagnosis of cardiomyopathy from 38.8%(n=262) in the pre-policy era to 34.8%(n=241) in the post-policy era. There was a small increase in waitlist additions reporting a primary diagnosis of CHD from 53.2%(n=359) in the pre-policy era to 56.6%(n=392) in the post-policy era.

Figure 7. Waitlist additions by diagnosis and era



Note: Only categories with more than 5% are labelled.

Table 3. Waitlist additions by diagnosis and era

Diagnosis	Pre	Post
Cardiomyopathy	262(38.8%)	241(34.8%)
CAD	2(0.3%)	5(0.7%)
CHD	359(53.2%)	392(56.6%)
Retransplant	32(4.7%)	27(3.9%)
Other	20(3%)	27(3.9%)

Figure 8 and table 4 show pediatric waitlist additions by medical urgency status, diagnosis, and era. Between the two eras, the pediatric status 1A additions were similarly distributed across the diagnoses, there was a decrease in pediatric status 1B cardiomyopathy diagnoses from 55.3%(n=84) to 47.8%(n=65), a decrease in pediatric status 2 cardiomyopathy diagnoses from 29%(n=36) to 22.1%(n=29), and an increase in pediatric status 2 CHD diagnoses from 64.5%(n=80) to 68.7%(n=90).

Figure 8. Waitlist additions by medical urgency status, diagnosis and era

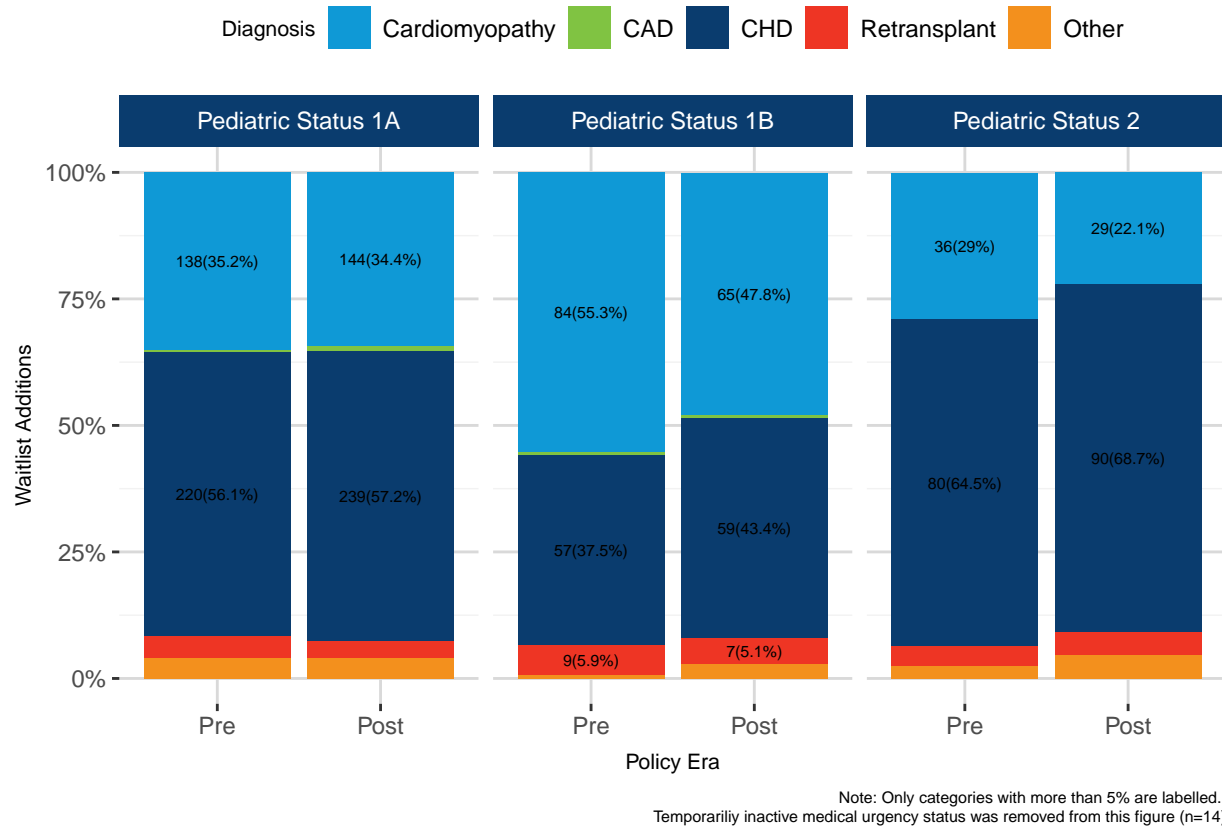


Table 4. Waitlist additions by medical urgency status, diagnosis and era

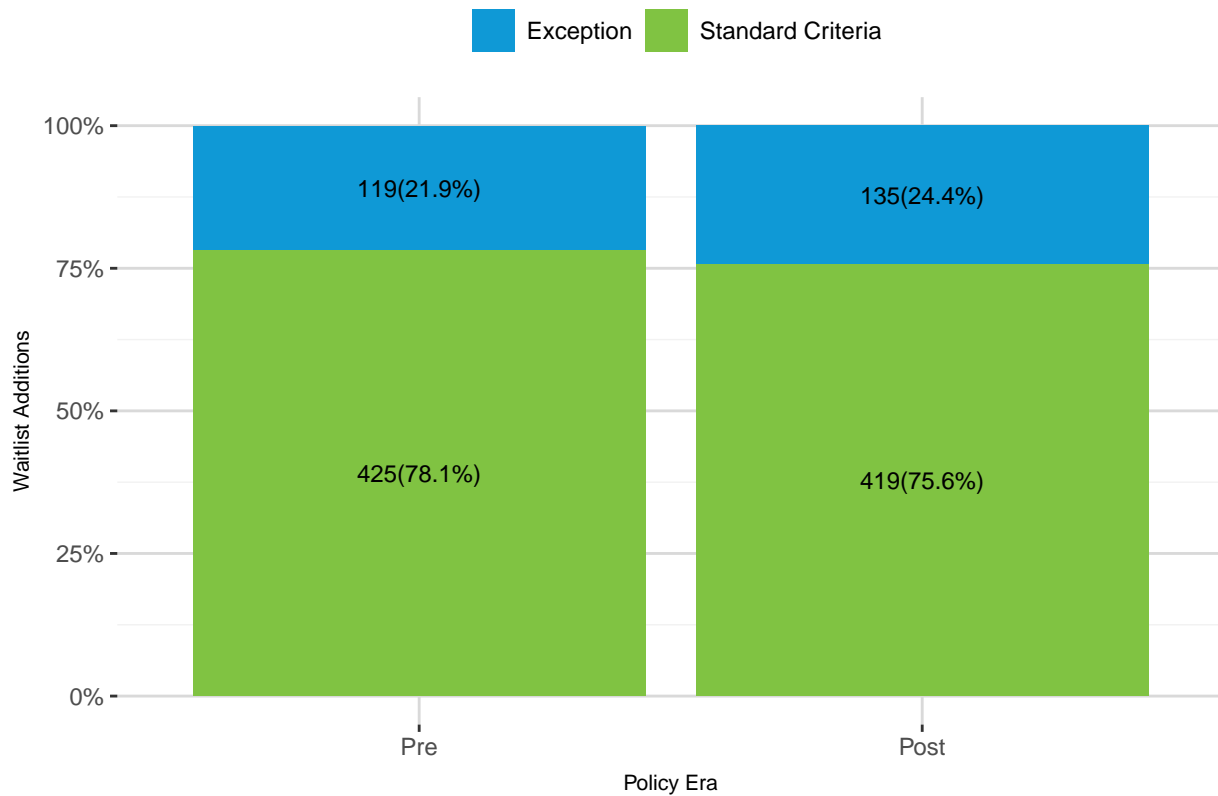
Status	Diagnosis	Pre	Post
Pediatric Status 1A	Cardiomyopathy	138(35.2%)	144(34.4%)
	CAD	1(0.3%)	4(1%)
	CHD	220(56.1%)	239(57.2%)
	Retransplant	17(4.3%)	14(3.3%)
	Other	16(4.1%)	17(4.1%)
	Not Reported	0(0%)	0(0%)
Pediatric Status 1B	Cardiomyopathy	84(55.3%)	65(47.8%)
	CAD	1(0.7%)	1(0.7%)
	CHD	57(37.5%)	59(43.4%)
	Retransplant	9(5.9%)	7(5.1%)
	Other	1(0.7%)	4(2.9%)
	Not Reported	0(0%)	0(0%)
Pediatric Status 2	Cardiomyopathy	36(29%)	29(22.1%)
	CAD	0(0%)	0(0%)
	CHD	80(64.5%)	90(68.7%)
	Retransplant	5(4%)	6(4.6%)
	Other	3(2.4%)	6(4.6%)
	Not Reported	0(0%)	0(0%)

Note:

Temporarily inactive medical urgency status was removed from this table (n=14)

Figure 9 shows pediatric waitlist additions by exception vs. standard criteria at listing and era. The exceptions were similarly distributed across eras.

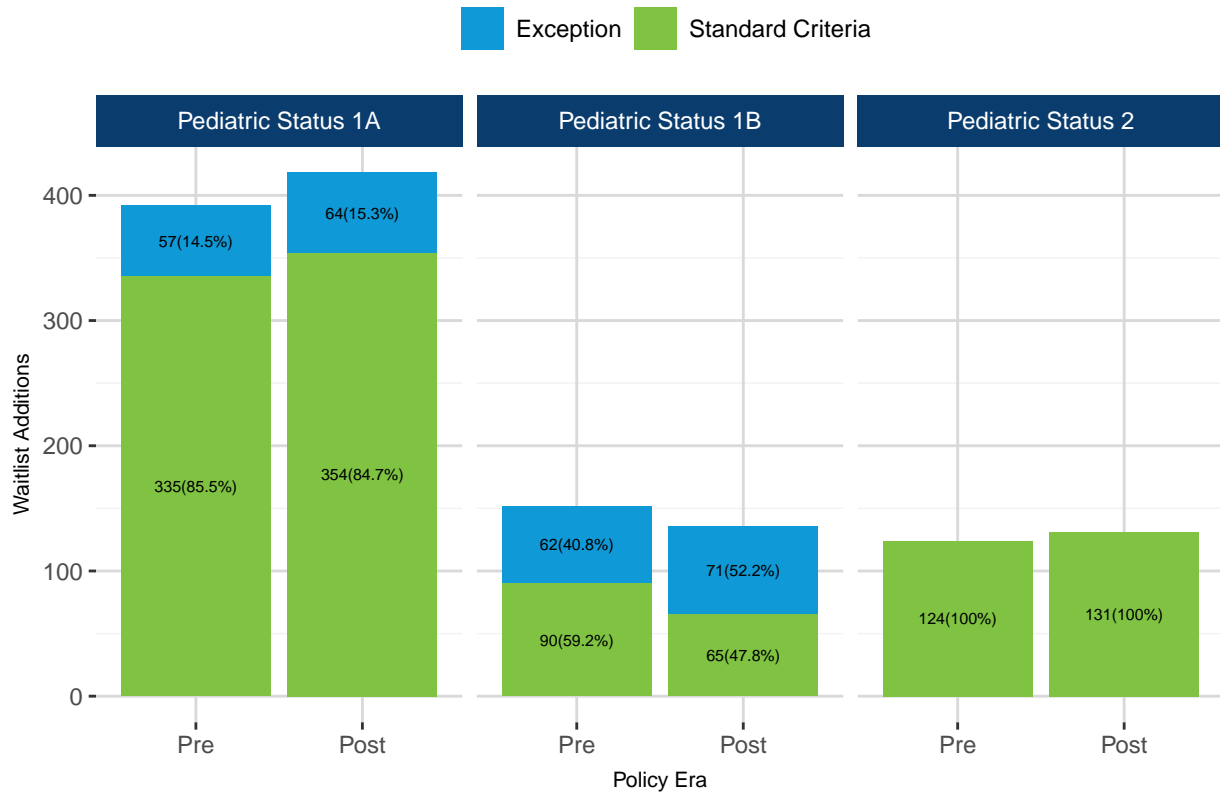
Figure 9. Waitlist additions by exception vs. standard criteria at listing and era



Both the temporarily inactive and pediatric status 2 waitlist additions were removed from the figure (n= 269).

Figure 10 shows pediatric waitlist additions by medical urgency status, exception vs. standard criteria, and era. Pediatric status 1A exceptions are similarly distributed across era. Pediatric status 1B exceptions rose from 40.8%(n=62) in the pre-policy era to 52.2%(n=71) in the post-policy era. Pediatric status 2 is not eligible for exception, so 100% of those waitlist additions were standard criteria.

Figure 10. Waitlist additions by medical urgency status, exception vs. standard criteria and era



Temporarily inactive medical urgency status was removed from this figure (n=14)

Figure 11 and table 5 show the breakdown of waitlist additions by exception vs. standard criteria stratified by OPTN region. The majority of regions saw an increase in waitlist additions with exceptions. Region 6 had the biggest percent increase in exceptions from 23.8% in the pre-policy era to 45% in the post-policy era.

Figure 11. Waitlist additions by exception vs. standard criteria and OPTN region



Note: Only categories with more than 5% are labeled.

Table 5. Waitlist additions by exception vs. standard criteria and OPTN region

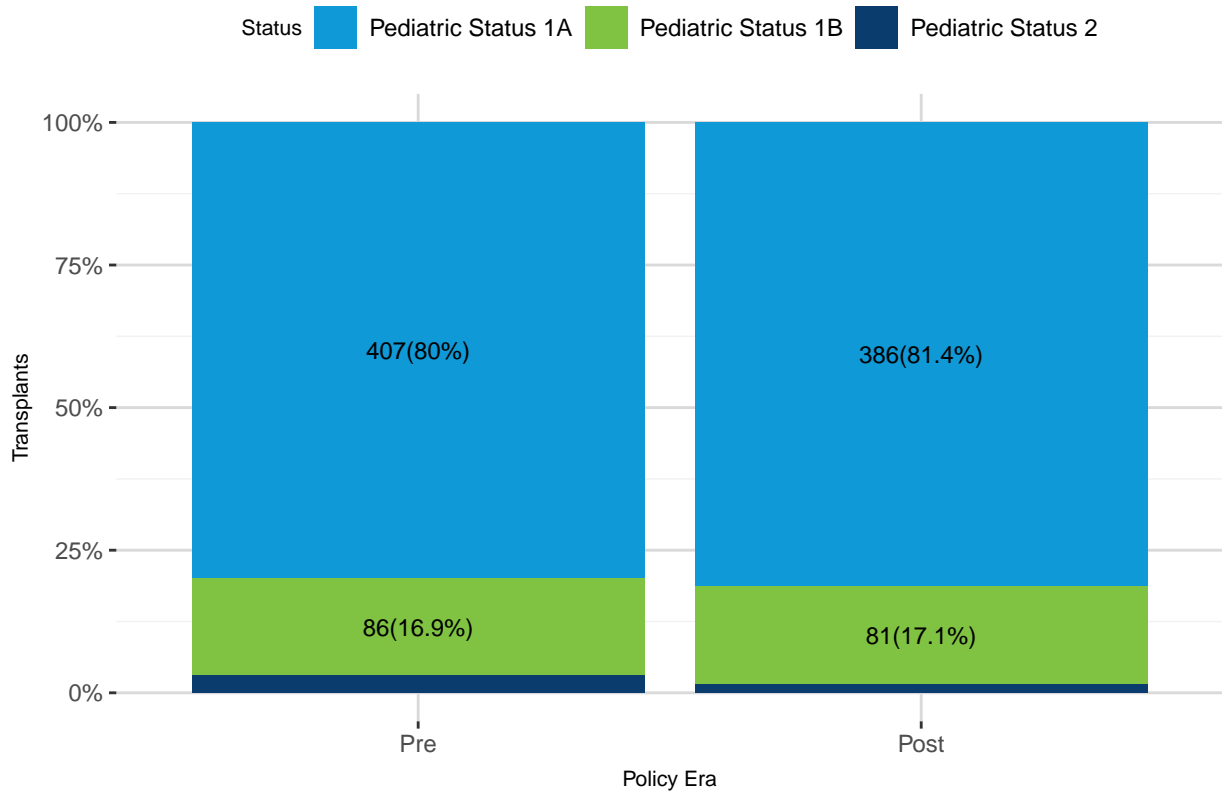
OPTN Region	Exception vs. Standard Criteria	Pre	Post
1	Exception	3(17.6%)	5(29.4%)
	Standard Criteria	11(64.7%)	7(41.2%)
	Not Reported	3(17.6%)	5(29.4%)
2	Exception	12(27.9%)	14(29.2%)
	Standard Criteria	26(60.5%)	28(58.3%)
	Not Reported	5(11.6%)	6(12.5%)
3	Exception	16(17%)	16(21.1%)
	Standard Criteria	60(63.8%)	48(63.2%)
	Not Reported	18(19.1%)	12(15.8%)
4	Exception	4(5.3%)	5(6.3%)
	Standard Criteria	51(68%)	48(60.8%)
	Not Reported	20(26.7%)	26(32.9%)
5	Exception	16(16.8%)	20(18.3%)
	Standard Criteria	57(60%)	62(56.9%)
	Not Reported	22(23.2%)	27(24.8%)
6	Exception	5(23.8%)	9(45%)
	Standard Criteria	11(52.4%)	9(45%)
	Not Reported	5(23.8%)	2(10%)
7	Exception	14(18.9%)	19(30.2%)
	Standard Criteria	44(59.5%)	33(52.4%)
	Not Reported	16(21.6%)	11(17.5%)
8	Exception	7(12.1%)	13(18.8%)
	Standard Criteria	38(65.5%)	45(65.2%)
	Not Reported	13(22.4%)	11(15.9%)
9	Exception	10(29.4%)	6(13%)
	Standard Criteria	23(67.6%)	36(78.3%)
	Not Reported	1(2.9%)	4(8.7%)
10	Exception	12(17.9%)	9(12%)
	Standard Criteria	48(71.6%)	43(57.3%)
	Not Reported	7(10.4%)	23(30.7%)
11	Exception	20(20.6%)	19(21.1%)
	Standard Criteria	56(57.7%)	60(66.7%)
	Not Reported	21(21.6%)	11(12.2%)

Transplants

The following metrics summarize transplant recipients by medical urgency status, exception vs. standard criteria, age group, OPTN region, and diagnosis.

Figure 12 and table 6 show pediatric heart transplants by medical urgency status and era. The medical urgency statuses are similarly distributed across era.

Figure 12. Transplants by medical urgency status and era



Note: Only categories with more than 5% are labelled.

Table 6. Transplants by medical urgency status and era

Status	Pre	Post
Pediatric Status 1A	407(80%)	386(81.4%)
Pediatric Status 1B	86(16.9%)	81(17.1%)
Pediatric Status 2	16(3.1%)	7(1.5%)

Figure 13 shows the pediatric transplants by age at transplant an era. The pre and post-policy eras had a similar distribution across the age groups.

Figure 13. Transplants by age at transplant and era

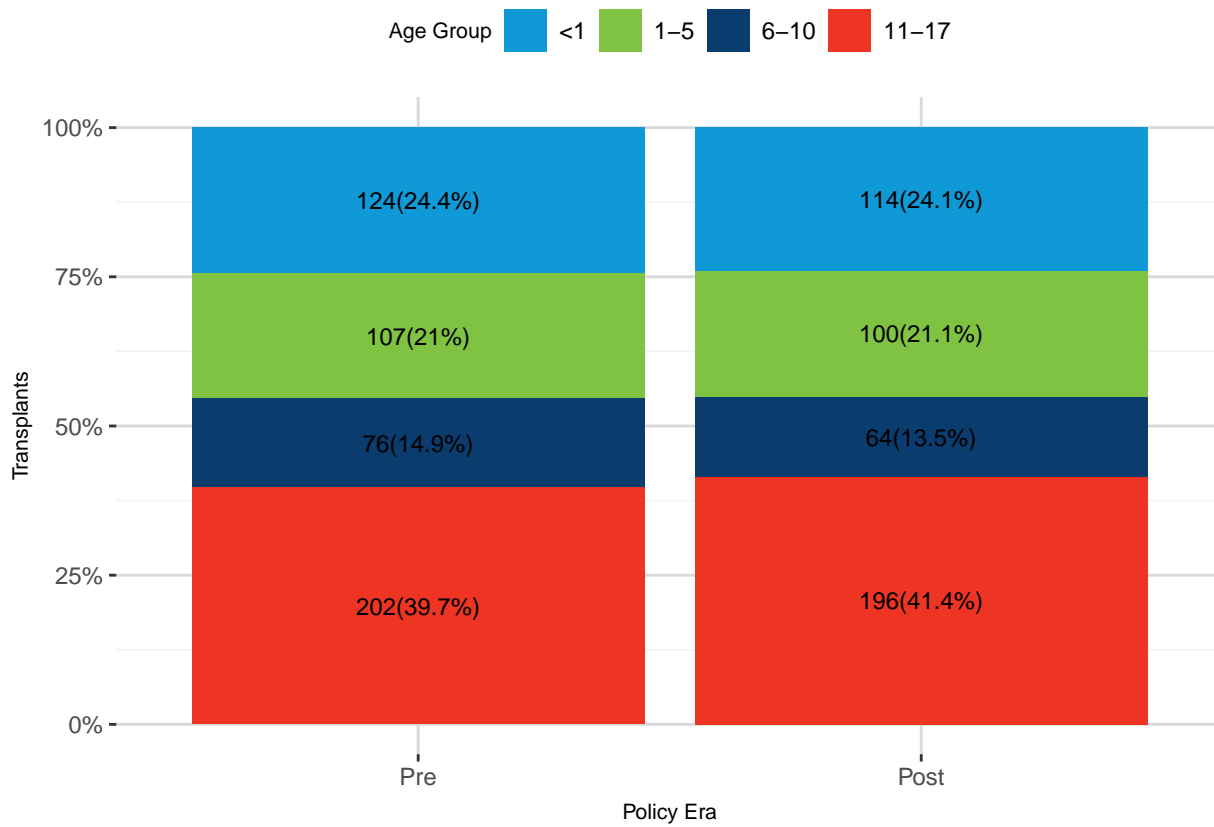
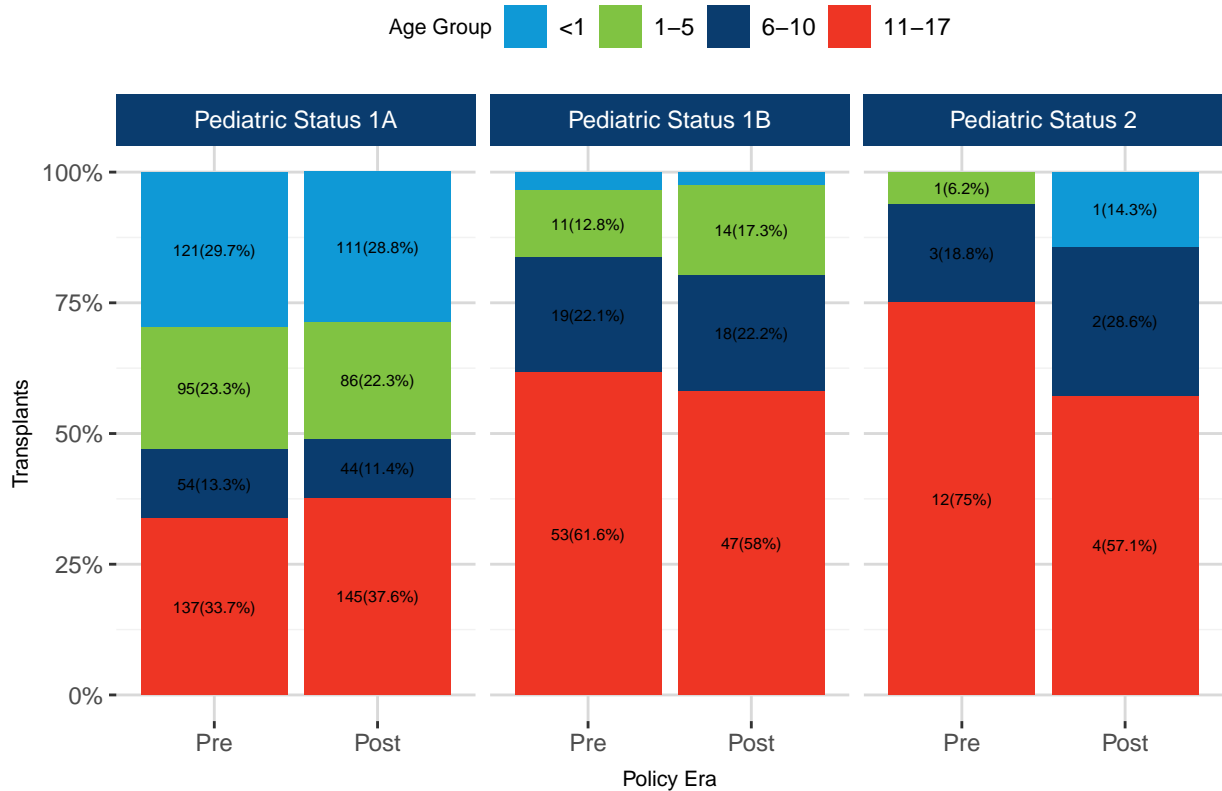


Figure 14 shows pediatric heart transplants by medical urgency status, age at transplant, and era. Pediatric status 1A transplants increased within the 11-17 age group from 33.7%(n=137) to 37.6%(n=145) Pediatric status 1B had an increase in transplants within age group 1-5 from 12.8%(n=11) to 17.3%(n=14) and a decrease in transplants within age group 11-17 from 61.6%(n=53) to 58%(n=47). Status 2 had a decrease in transplants within age group 11-17 from 75%(n=12) to 57.1%(n=4). The numbers are small in these groups, so true distributions may be difficult to compare.

Figure 14. Transplants by medical urgency status, age at transplant, and era



Note: Only categories with more than 5% are labelled.

Figure 15 shows pediatric heart transplants by OPTN region and era. Most regions recorded a negative change between the eras. Region 9 had the highest positive percent change in pediatric heart transplants from pre-policy to post-policy with a difference of 32%. Region 7 had the highest negative percent change in pediatric heart transplants from pre-policy to post-policy with a difference of -32.1%.

Figure 15. Transplants by OPTN region and era

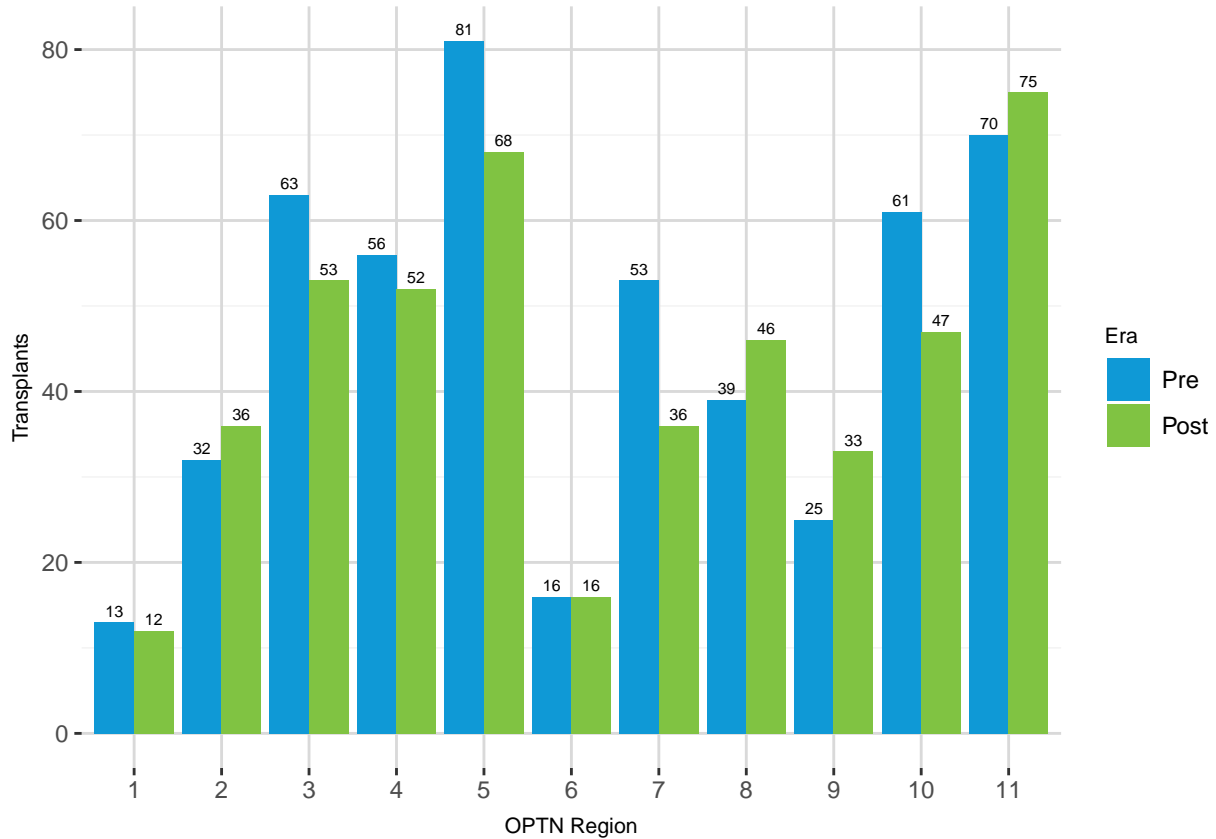
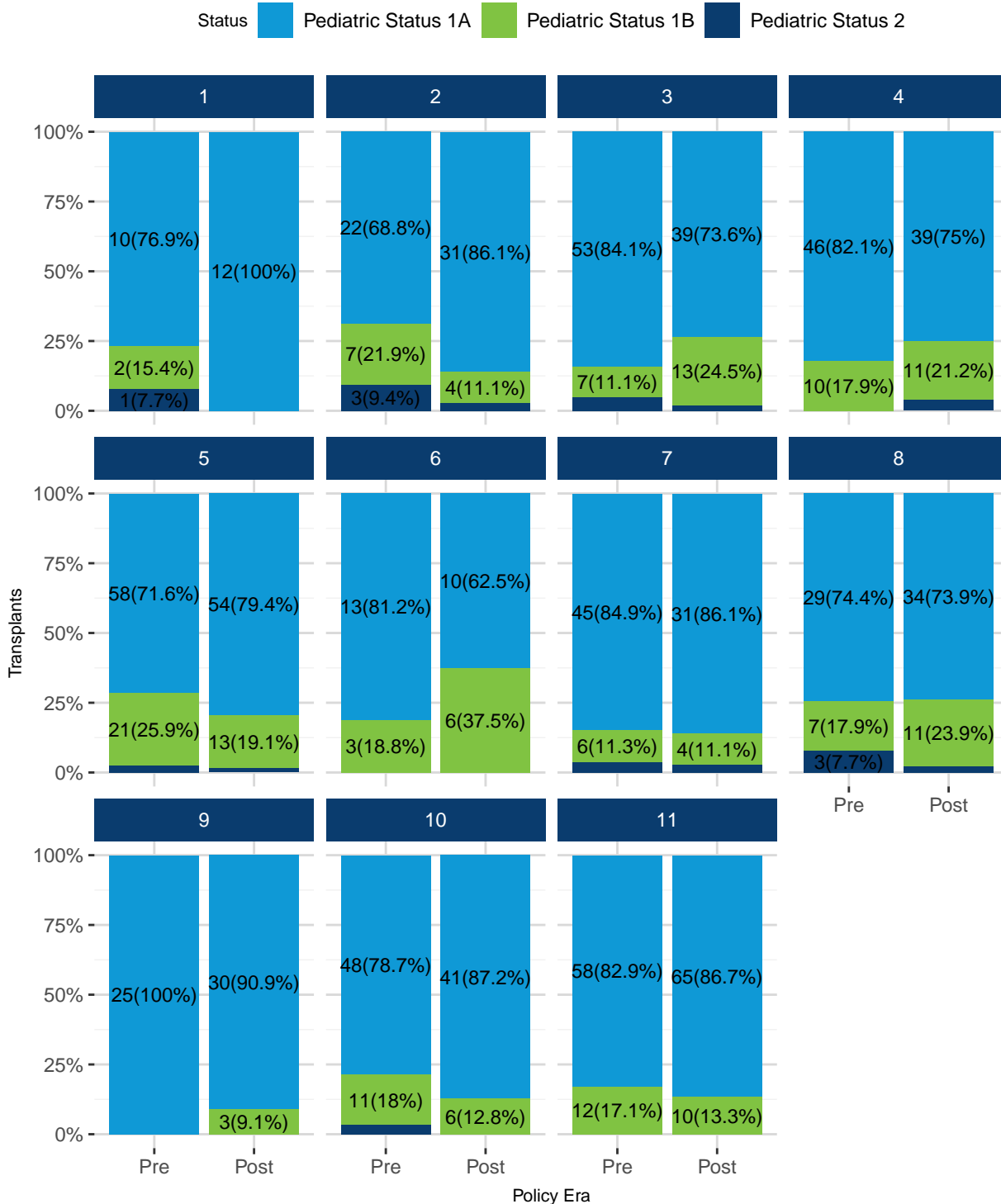


Figure 16 and table 7 show pediatric heart transplants by medical urgency status and OPTN region. The majority of regions saw a percent increase in pediatric status 1A transplants. Region 1 had an increase from 76.9% in the pre-policy era to 100% in the post-policy era. The majority of regions saw a percent decrease in pediatric status 2 transplants.

Figure 16. Transplants by medical urgency status and OPTN region



Note: Only categories with more than 5% are labelled.

Table 7. Transplants by medical urgency status and OPTN region

Region	Status	Pre	Post
1	Pediatric Status 1A	10(76.9%)	12(100%)
	Pediatric Status 1B	2(15.4%)	0(0%)
	Pediatric Status 2	1(7.7%)	0(0%)
2	Pediatric Status 1A	22(68.8%)	31(86.1%)
	Pediatric Status 1B	7(21.9%)	4(11.1%)
	Pediatric Status 2	3(9.4%)	1(2.8%)
3	Pediatric Status 1A	53(84.1%)	39(73.6%)
	Pediatric Status 1B	7(11.1%)	13(24.5%)
	Pediatric Status 2	3(4.8%)	1(1.9%)
4	Pediatric Status 1A	46(82.1%)	39(75%)
	Pediatric Status 1B	10(17.9%)	11(21.2%)
	Pediatric Status 2	0(0%)	2(3.8%)
5	Pediatric Status 1A	58(71.6%)	54(79.4%)
	Pediatric Status 1B	21(25.9%)	13(19.1%)
	Pediatric Status 2	2(2.5%)	1(1.5%)
6	Pediatric Status 1A	13(81.2%)	10(62.5%)
	Pediatric Status 1B	3(18.8%)	6(37.5%)
7	Pediatric Status 1A	45(84.9%)	31(86.1%)
	Pediatric Status 1B	6(11.3%)	4(11.1%)
	Pediatric Status 2	2(3.8%)	1(2.8%)
8	Pediatric Status 1A	29(74.4%)	34(73.9%)
	Pediatric Status 1B	7(17.9%)	11(23.9%)
	Pediatric Status 2	3(7.7%)	1(2.2%)
9	Pediatric Status 1A	25(100%)	30(90.9%)
	Pediatric Status 1B	0(0%)	3(9.1%)
10	Pediatric Status 1A	48(78.7%)	41(87.2%)
	Pediatric Status 1B	11(18%)	6(12.8%)
	Pediatric Status 2	2(3.3%)	0(0%)
11	Pediatric Status 1A	58(82.9%)	65(86.7%)
	Pediatric Status 1B	12(17.1%)	10(13.3%)

Figure 17 and Table 8 show pediatric heart transplants by diagnosis at transplant and era. The diagnosis groups are similarly distributed across era.

Figure 17. Transplants by diagnosis at transplant and era

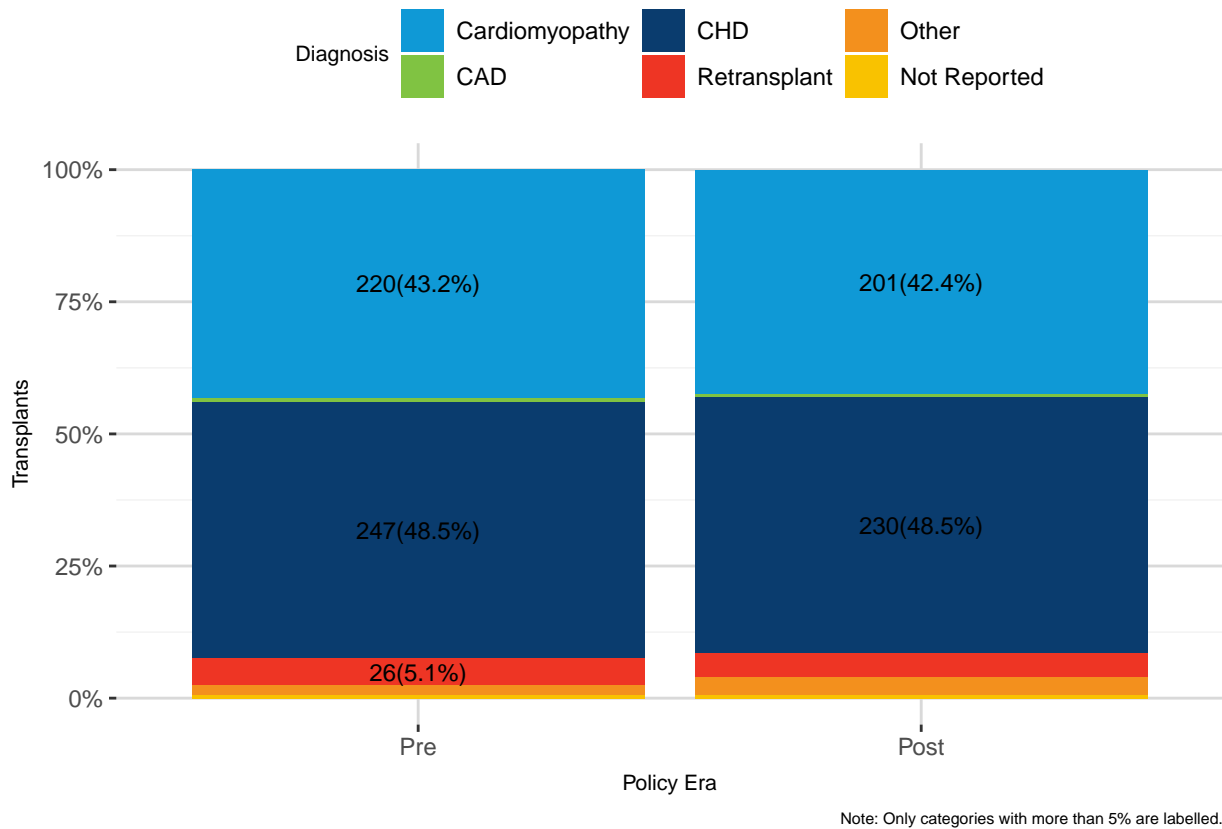
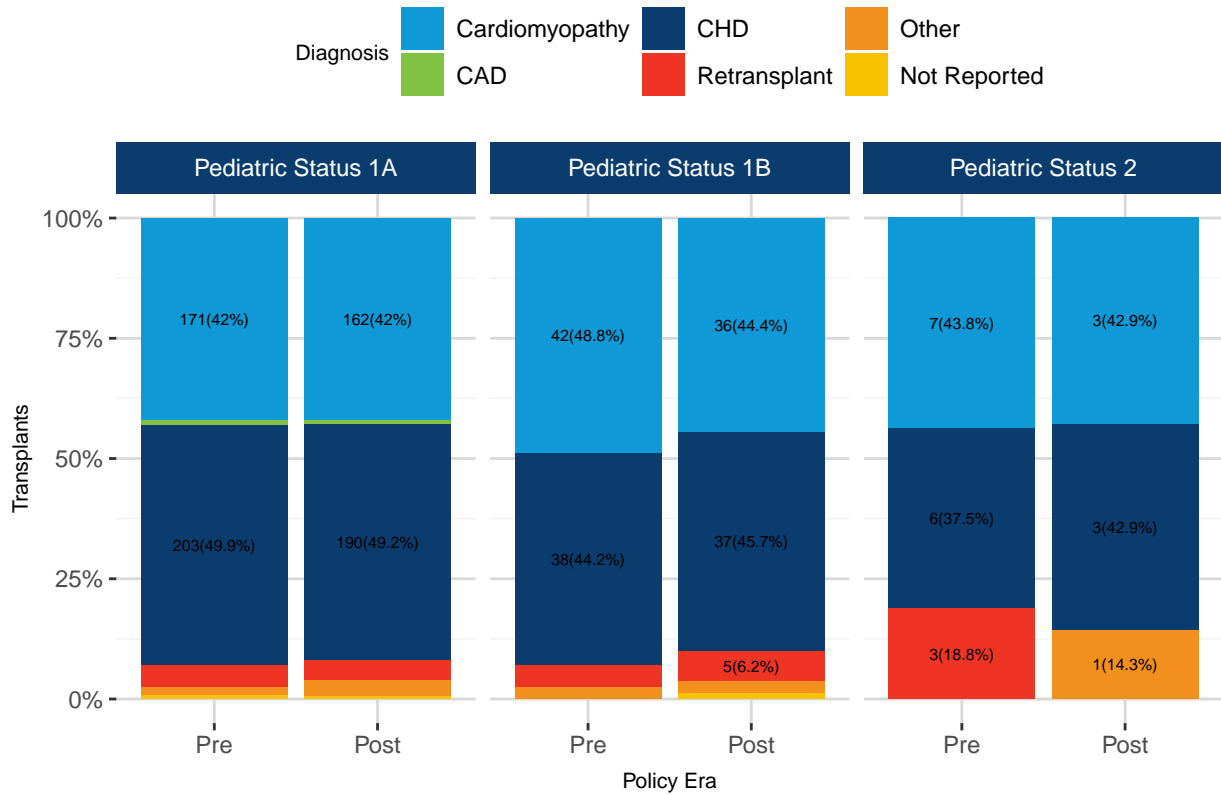


Table 8. Transplants by diagnosis at transplant and era

Diagnosis	Pre	Post
Cardiomyopathy	220(43.2%)	201(42.4%)
CAD	4(0.8%)	3(0.6%)
CHD	247(48.5%)	230(48.5%)
Retransplant	26(5.1%)	21(4.4%)
Other	9(1.8%)	16(3.4%)
Not Reported	3(0.6%)	3(0.6%)

Figure 18 shows pediatric heart transplants by medical urgency status, diagnosis at transplant, and era. Pediatric status 1A transplants were similarly distributed across diagnoses and eras. A larger percent of pediatric status 1B transplants reported primary diagnosis of cardiomyopathy at transplant from 48.8%(n=42) in the pre-policy era to 44.4%(n=36) in the post policy era, and a decrease in transplants reporting a primary diagnosis of CHD from 44.2%(n=38) in the pre-policy era to 45.7%(n=37) in the post-policy era. Pediatric status 2 had a very small sample size.

Figure 18. Transplants by medical urgency status, diagnosis at transplant and era



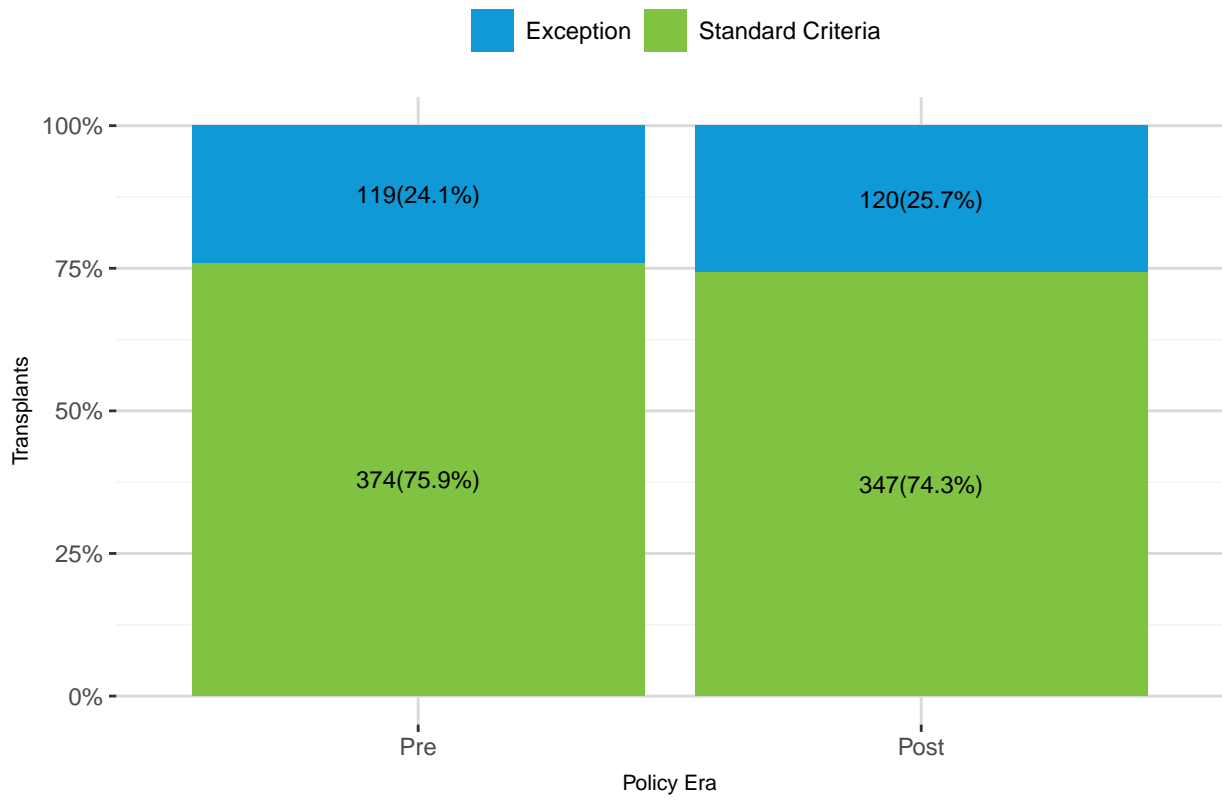
Note: Only categories with more than 5% are labelled.

Table 9. Transplants by medical urgency status, diagnosis at transplant and era

Status	Diagnosis	Pre	Post
Pediatric Status 1A	Cardiomyopathy	171(42%)	162(42%)
	CAD	4(1%)	3(0.8%)
	CHD	203(49.9%)	190(49.2%)
	Retransplant	19(4.7%)	16(4.1%)
	Other	7(1.7%)	13(3.4%)
	Not Reported	3(0.7%)	2(0.5%)
	Pediatric Status 1B	Cardiomyopathy	42(48.8%)
CAD		0(0%)	0(0%)
CHD		38(44.2%)	37(45.7%)
Retransplant		4(4.7%)	5(6.2%)
Other		2(2.3%)	2(2.5%)
Not Reported		0(0%)	1(1.2%)
Pediatric Status 2	Cardiomyopathy	7(43.8%)	3(42.9%)
	CAD	0(0%)	0(0%)
	CHD	6(37.5%)	3(42.9%)
	Retransplant	3(18.8%)	0(0%)
	Other	0(0%)	1(14.3%)
	Not Reported	0(0%)	0(0%)

Figure 19 shows pediatric heart transplants by exception vs. standard criteria at transplant and era. The exceptions were similarly distributed across eras.

Figure 19. Transplants by exception vs. standard criteria at transplant and era



Pediatric status 2 were removed from the figure (n=23)

Figure 20 shows pediatric heart transplants by medical urgency status, exception vs. standard criteria, and era. Pediatric status 1A and 1B exceptions were similarly distributed across eras. Pediatric status 2 is not eligible for exception, so 100% of those transplants were standard criteria.

Figure 20. Transplants by medical urgency status, exception vs. standard criteria and era

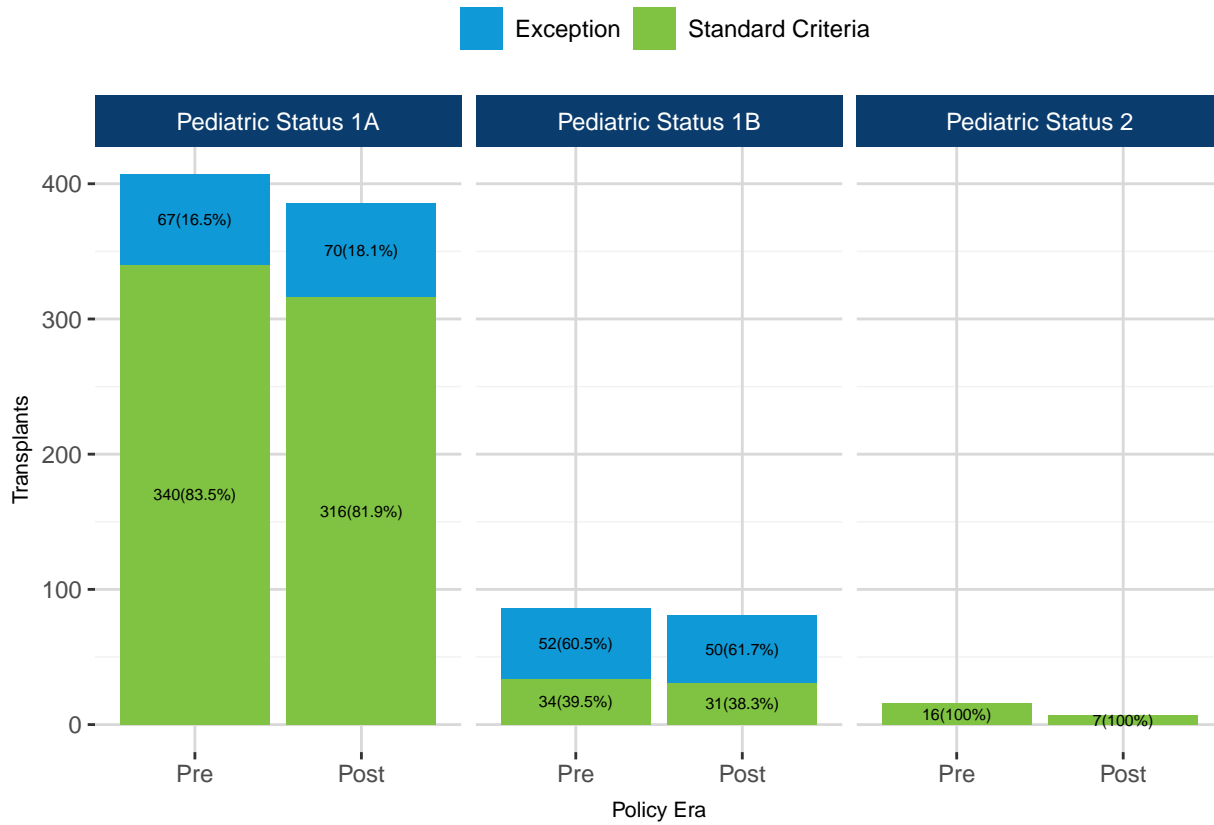
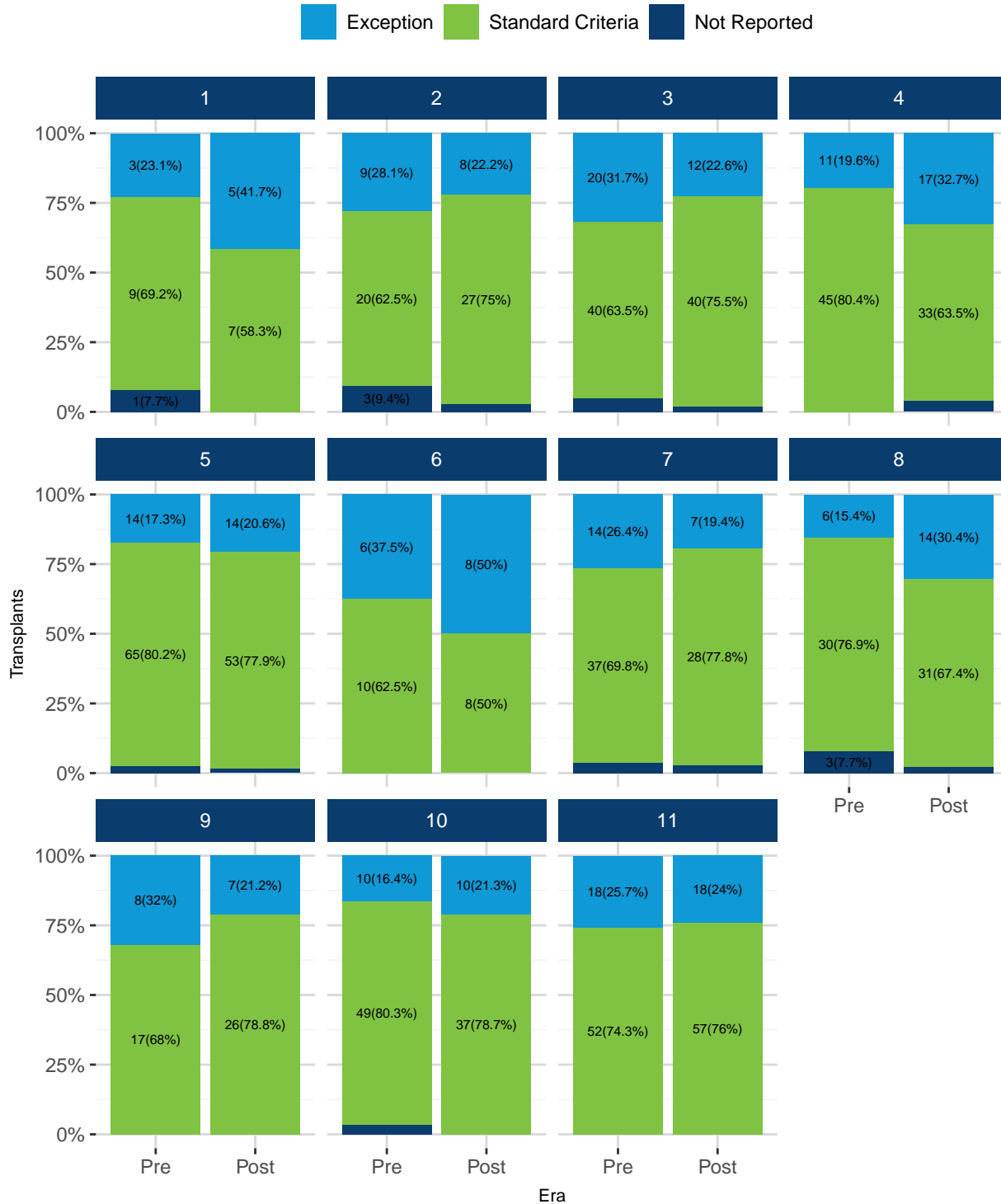


Figure 21 and table 10 show pediatric heart transplants by exception vs. standard criteria at transplant and OPTN region. The majority of regions saw an increase in transplants to patients with exceptions. The greatest increase in transplants to patients with exceptions was in region 1 from 23.1% in the pre-policy era to 41.7% in the post-policy era.

Figure 21. Transplants by exception vs. standard criteria at Transplant and OPTN region



Note: Only categories with more than 5% are labelled.

Table 10. Transplants by exception vs. standard criteria at Transplant and OPTN region

OPTN Region	Exception vs. Standard Criteria	Pre	Post
1	Exception	3(23.1%)	5(41.7%)
	Standard Criteria	9(69.2%)	7(58.3%)
	Not Reported	1(7.7%)	0(0%)
2	Exception	9(28.1%)	8(22.2%)
	Standard Criteria	20(62.5%)	27(75%)
	Not Reported	3(9.4%)	1(2.8%)
3	Exception	20(31.7%)	12(22.6%)
	Standard Criteria	40(63.5%)	40(75.5%)
	Not Reported	3(4.8%)	1(1.9%)
4	Exception	11(19.6%)	17(32.7%)
	Standard Criteria	45(80.4%)	33(63.5%)
	Not Reported	0(0%)	2(3.8%)
5	Exception	14(17.3%)	14(20.6%)
	Standard Criteria	65(80.2%)	53(77.9%)
	Not Reported	2(2.5%)	1(1.5%)
6	Exception	6(37.5%)	8(50%)
	Standard Criteria	10(62.5%)	8(50%)
7	Exception	14(26.4%)	7(19.4%)
	Standard Criteria	37(69.8%)	28(77.8%)
	Not Reported	2(3.8%)	1(2.8%)
8	Exception	6(15.4%)	14(30.4%)
	Standard Criteria	30(76.9%)	31(67.4%)
	Not Reported	3(7.7%)	1(2.2%)
9	Exception	8(32%)	7(21.2%)
	Standard Criteria	17(68%)	26(78.8%)
10	Exception	10(16.4%)	10(21.3%)
	Standard Criteria	49(80.3%)	37(78.7%)
	Not Reported	2(3.3%)	0(0%)
11	Exception	18(25.7%)	18(24%)
	Standard Criteria	52(74.3%)	57(76%)

Exception Requests

The following section examines changes in the percent of approvals and denials for exception requests.

In the pre-policy era, there were a total of 568 exceptions and extensions filed. Of those, 564 were approved/completed, 3 were withdrawn, and 1 was denied. In the post-policy era, there were a total of 620 exceptions and extensions filed. Post-policy, pediatric status 1A had 93.9% (n = 417) of exceptions approved and 5.6% (n = 25) of exceptions denied. The other 0.4% were either withdrawn or not-required - patient transplanted. Pediatric status 1B had 95.5% (n = 168) of exceptions approved and 4.5% (n = 8) of exceptions denied.

Survival Analysis

The following figures examine changes in post-transplant patient survival rates overall and stratified by status. The cohort for these analyses are patients transplanted in the immediate 6 months prior to implementation for the pre-policy era, December 14, 2020 to June 14, 2021, and the immediate 6 month after implementation for the post-policy era, June 15, 2021 to December 14, 2021. All survival analyses reflect 6 month survival.

Figure 22 and table 11 show post-transplant patient survival rates overall by era. Overall patient survival was higher post-policy at 95% when compared to pre-policy at 94%.

Figure 22. Post-transplant patient survival rates overall

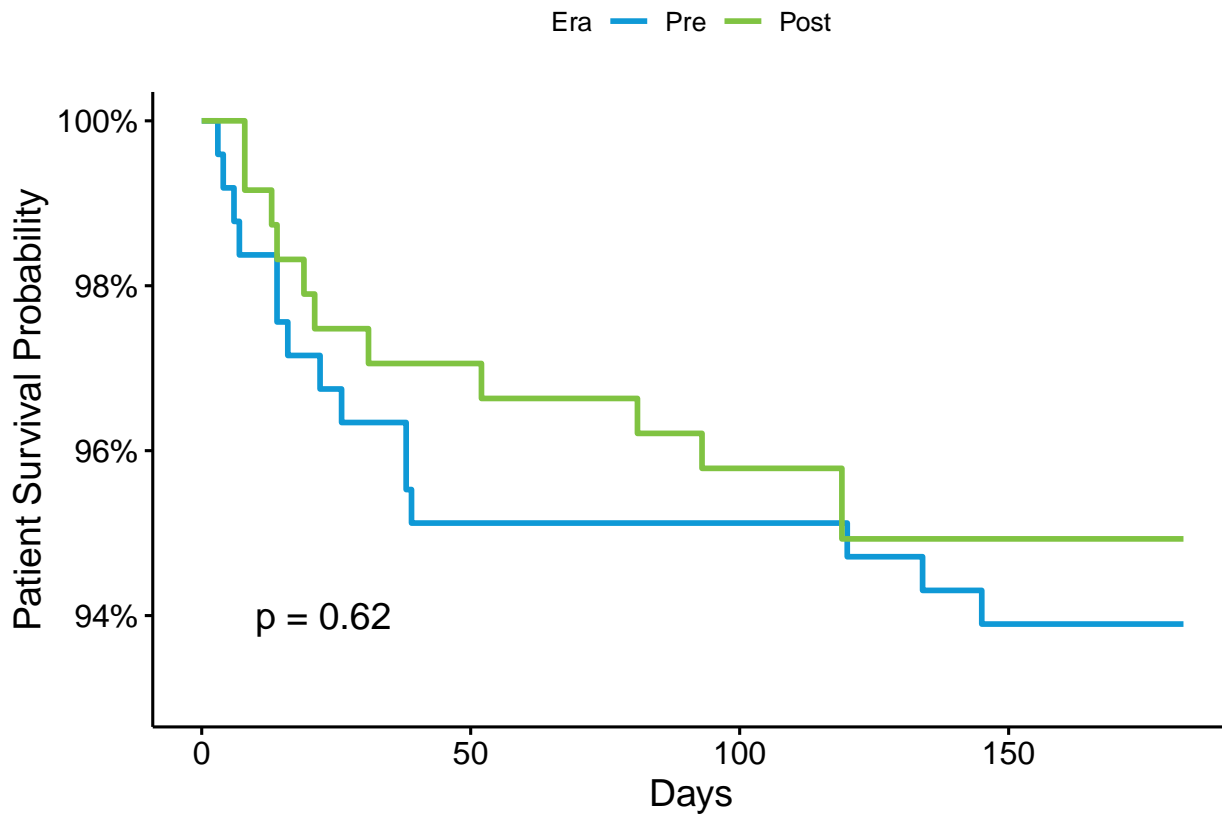


Table 11. Post-transplant patient survival rates overall

Era	N at Risk	N Events	Estimate	95% Confidence Interval	
				Lower CL	Upper CL
Pre	230	15	0.94	0.90	0.96
Post	195	12	0.95	0.91	0.97

Figure 23 and table 12 show post-transplant patient survival rates stratified by status and by era. Post-transplant survival for pediatric status 1A patients was higher post policy at 96% than pre-policy at 94%. Post-transplant survival for pediatric status 1B patients was lower post policy at 93% than pre-policy at 94%. Neither of these differences were statistically significant.

Figure 23. Post-transplant patient survival rates stratified by status

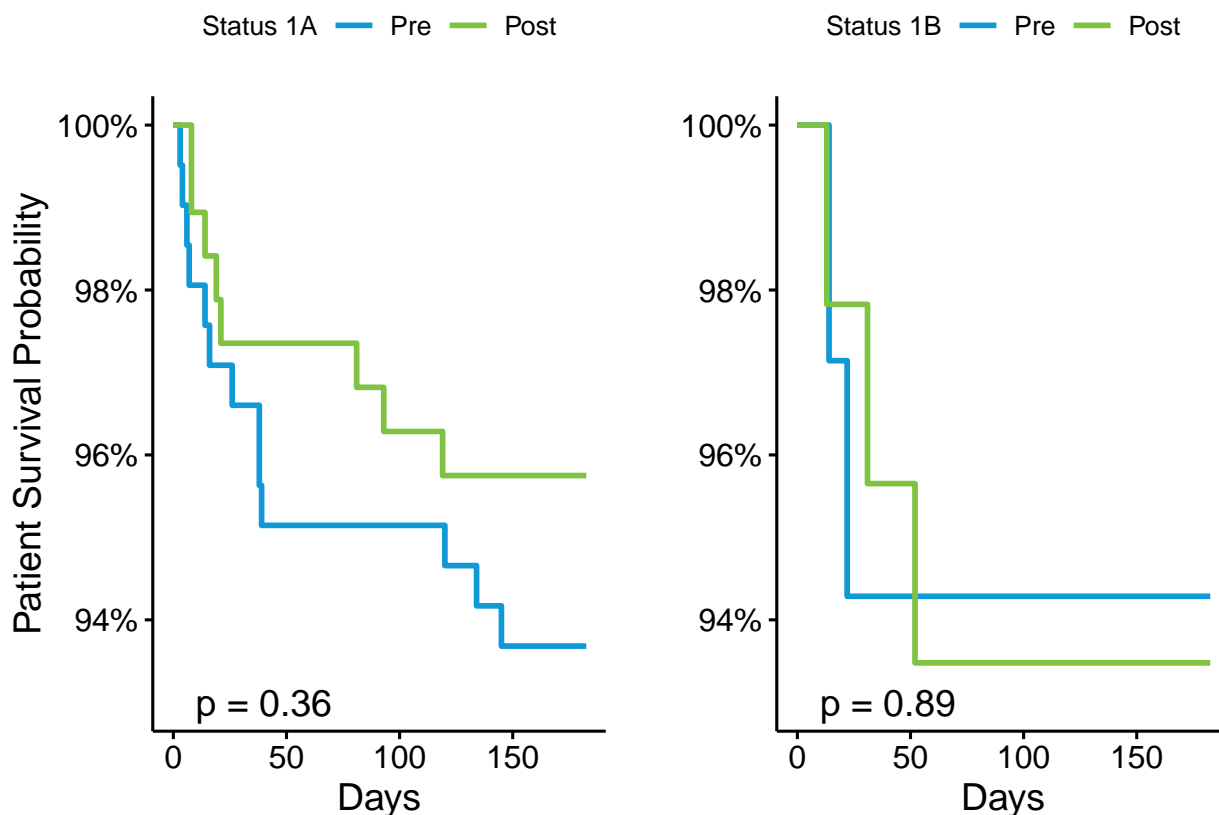


Table 12. Post-transplant patient survival rates stratified by status

Era	Status	N at Risk	N Events	Estimate	95% Confidence Interval	
					Lower CL	Upper CL
Pre	Pediatric Status 1A	192	13	0.94	0.89	0.96
	Pediatric Status 1B	33	2	0.94	0.79	0.99
Post	Pediatric Status 1A	158	8	0.96	0.92	0.98
	Pediatric Status 1B	35	3	0.93	0.81	0.98

Rates

Figure 24 and table 13 show the waitlist mortality rate for pediatric heart candidates broken out by pediatric heart status and whether the candidate ever had an exception in each era. In each status across eras, except for pediatric status 1b in the pre-policy era, those candidates that ever had an exception had a lower mortality rate than those without. Mortality increased from pre-policy in pediatric status 1A candidates with an exception, pediatric status 1B candidates with or without an exception, and pediatric status 2 candidates without an exception. There was a decrease in mortality for pediatric status 1A candidates that never had an exception from 57.81 deaths per 100 patient-years pre-policy to 50.24 deaths per 100 patient-years post policy.

Figure 24. Waitlist mortality rate

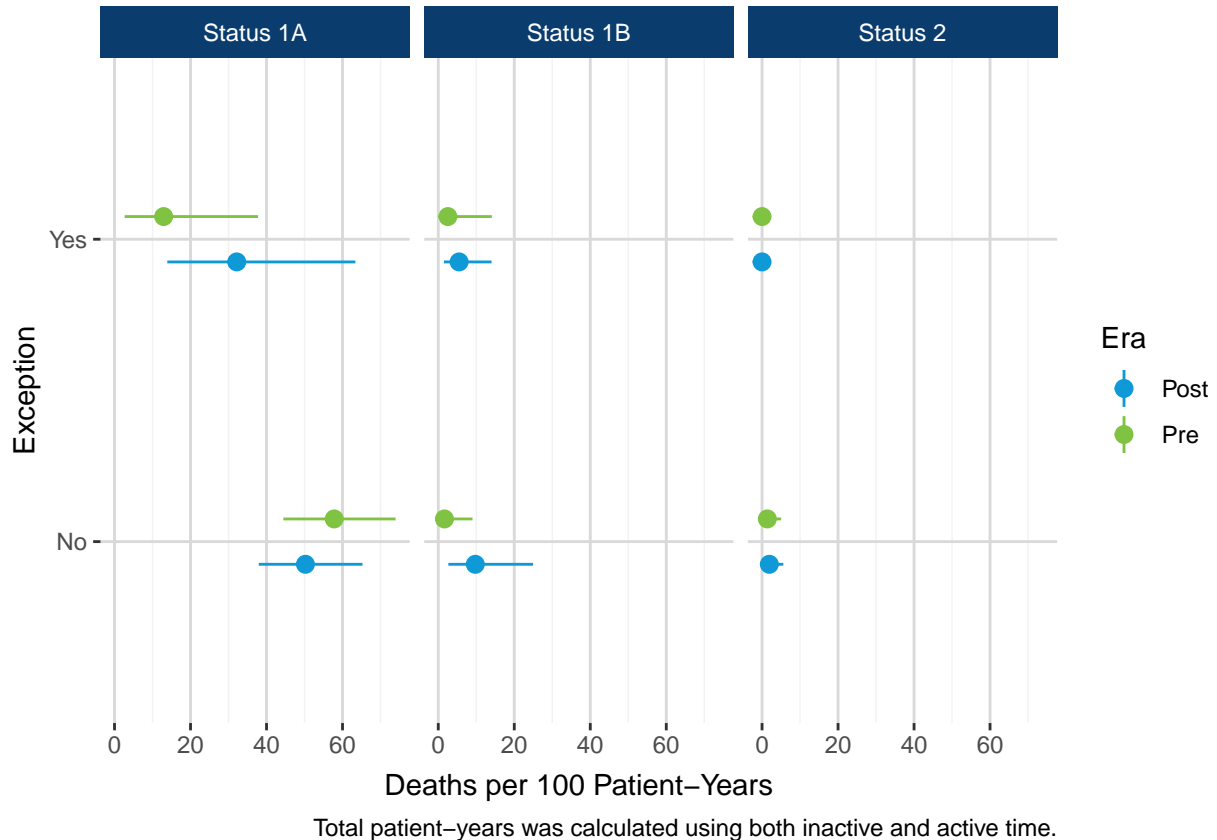


Table 13. Waitlist mortality rate

Era	Status	Exception	N Patients	N Events	Total Patient-Years	Deaths per 100 Patient-Years	95% Confidence Interval	
							Lower CL	Upper CL
Pre	Status 1A	Yes	123	3	23.21	12.93	2.67	37.78
	Status 1A	No	510	63	108.98	57.81	44.42	73.96
	Status 1B	Yes	150	1	39.54	2.53	0.06	14.09
	Status 1B	No	184	1	61.85	1.62	0.04	9.01
	Status 2	Yes	76	0	30.39	0.00	0.00	0.00
	Status 2	No	235	2	143.77	1.39	0.17	5.03
	Overall	-	1035	70	408.90	17.12	13.35	21.63
Post	Status 1A	Yes	148	8	24.88	32.16	13.88	63.37
	Status 1A	No	498	56	111.45	50.24	37.95	65.25
	Status 1B	Yes	204	4	73.03	5.48	1.49	14.02
	Status 1B	No	145	4	41.08	9.74	2.65	24.93
	Status 2	Yes	62	0	18.73	0.00	0.00	0.00
	Status 2	No	247	3	156.57	1.92	0.40	5.60
	Overall	-	1043	75	425.10	17.64	13.88	22.12

Note:

Total patient-years was calculated using both inactive and active time.

Figure 25 and table 14 show the waitlist transplant rate for pediatric heart candidates broken out by pediatric heart status and whether the candidate ever had an exception in each era. The transplant rate increased from pre-policy in pediatric status 1A candidates with an exception and decreased in every other category except pediatric status 2 candidates with an exception which saw no change.

Figure 25. Waitlist transplant rate

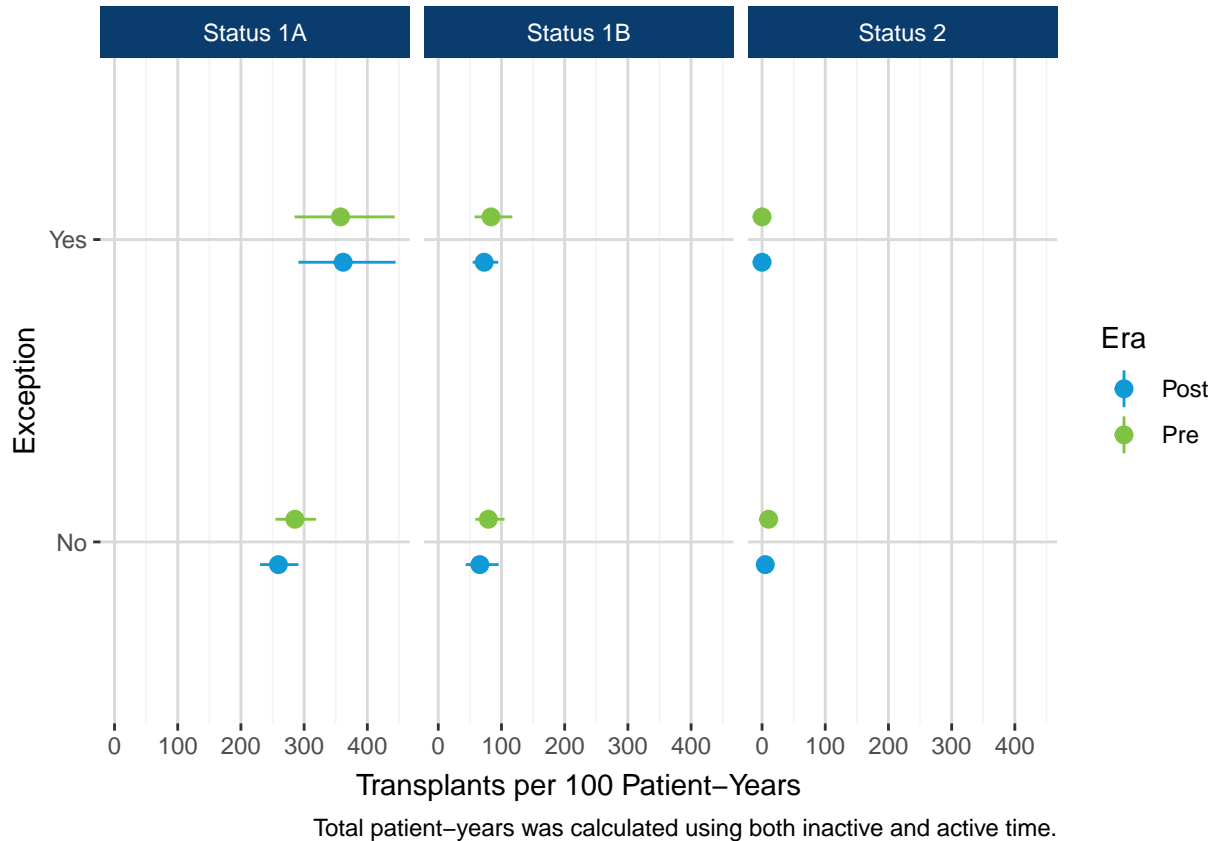


Table 14. Waitlist transplant rate

Era	Status	Exception	N Patients	N Events	Total Patient-Years	Transplants per 100 Patient-Years	95% Confidence Interval	
							Lower CL	Upper CL
Pre	Status 1A	Yes	123	83	23.21	357.63	284.85	443.34
	Status 1A	No	510	311	108.98	285.36	254.53	318.91
	Status 1B	Yes	150	33	39.54	83.47	57.45	117.22
	Status 1B	No	184	49	61.85	79.23	58.61	104.74
	Status 2	Yes	76	0	30.39	0.00	0.00	0.00
	Status 2	No	235	15	143.77	10.43	5.84	17.21
	Overall	-	1035	491	408.90	120.08	109.69	131.19
Post	Status 1A	Yes	148	90	24.88	361.78	290.92	444.69
	Status 1A	No	498	289	111.45	259.30	230.26	290.98
	Status 1B	Yes	204	53	73.03	72.57	54.36	94.93
	Status 1B	No	145	27	41.08	65.73	43.32	95.63
	Status 2	Yes	62	0	18.73	0.00	0.00	0.00
	Status 2	No	247	8	156.57	5.11	2.21	10.07
	Overall	-	1043	467	425.10	109.86	100.12	120.29

Note:

Total patient-years was calculated using both inactive and active time.

Summary

There was an increase in pediatric status 1A waitlist additions and a decrease in pediatric status 1B waitlist additions from the pre-policy era to the post-policy era. Age and exceptions were similarly distributed across eras in both waitlist additions and transplants. Transplants by medical urgency status were also similarly distributed across eras. There was an increase in exception denials from 1 in the pre-policy era to a total of 33 in the post-policy era. Overall post-transplant patient survival increased, especially for pediatric status 1A patients. The transplant rate increased across eras for pediatric status 1A patients with an exception, but decreased for pediatric status 1B patients with an exception. Mortality increased from the pre-policy era to the post-policy era in all categories except pediatric status 1A candidates without an exception and pediatric status 2 candidates with an exception.