



ADPKD Annual Report

BC RENAL ADPKD NETWORK
2024

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1 Introduction

Autosomal dominant polycystic kidney disease (ADPKD) is the most common inherited renal disorder and is the fourth leading cause of end-stage renal disease in Canada. A lifelong disease, patients develop clusters of cysts -- noncancerous round sacs containing fluid. The disease is quite variable, from minimal impact on kidney function to rapidly progressive disease that results in kidney failure at a young age. There is no cure for ADPKD at this time, but research has led to improved tools for diagnosing and predicting the renal outcome of the disease, as well as treatments that may slow the progression of ADPKD in some people.

As part of BC Renal's overarching priorities of optimizing patient experience and outcomes as well as innovation and research in renal care, we have developed and implemented a provincial ADPKD strategy in BC's renal programs that supports equitable and sustainable care for patients and families living with ADPKD.

As a first in kind comprehensive provincial registry, the BC ADPKD registry was established to enhance the ability to identify ADPKD patients in BC and gain a better understanding of the burden of disease, current management, and outcomes of these patients. The granular data in the registry informs continuous quality improvement initiatives including facilitation of practice audits wherein individual clinicians can use the data to get a better understanding of their current ADPKD management and identify areas for improvement.

2 Data Sources

Early identification, assessment of renal progression and implementation of appropriate treatments are key components of modern ADPKD care.

The ADPKD patient registry was created to enhance identification and understanding of ADPKD in BC including basic demographics, management patterns and clinical outcomes of this specific group of patients. A main focus in the registration efforts has been identification and registration of patients seen in nephrologists' private offices, prior to enrollment in other BC Renal administered services. Over time there is a noted increase in the number of ADPKD patients registered in PROMIS which is most prominent in early-stage patients not on dialysis or transplant.

The ADPKD registry includes data that will capture patterns of treatment use, specifically tolvaptan which has been funded by BC Renal since January 2020. Cost of BCR-funded tolvaptan is being captured through a separate tolvaptan utilization report. Further enhancements to PROMIS for capture of tolvaptan approval criteria are pending.

As part of the BC ADPKD strategy and with support from nephrology offices across the province, imaging reports (MRI, CT, U/S) reports are being obtained from PKD patients as far as 5-6 years back to ensure TKV and classification data is captured in the ADPKD registry in PROMIS. This will be an annual process for ongoing TKV and classification data capture in PROMIS until an automated process is established.

3 ADPKD Overview

3.1 ADPKD in BC

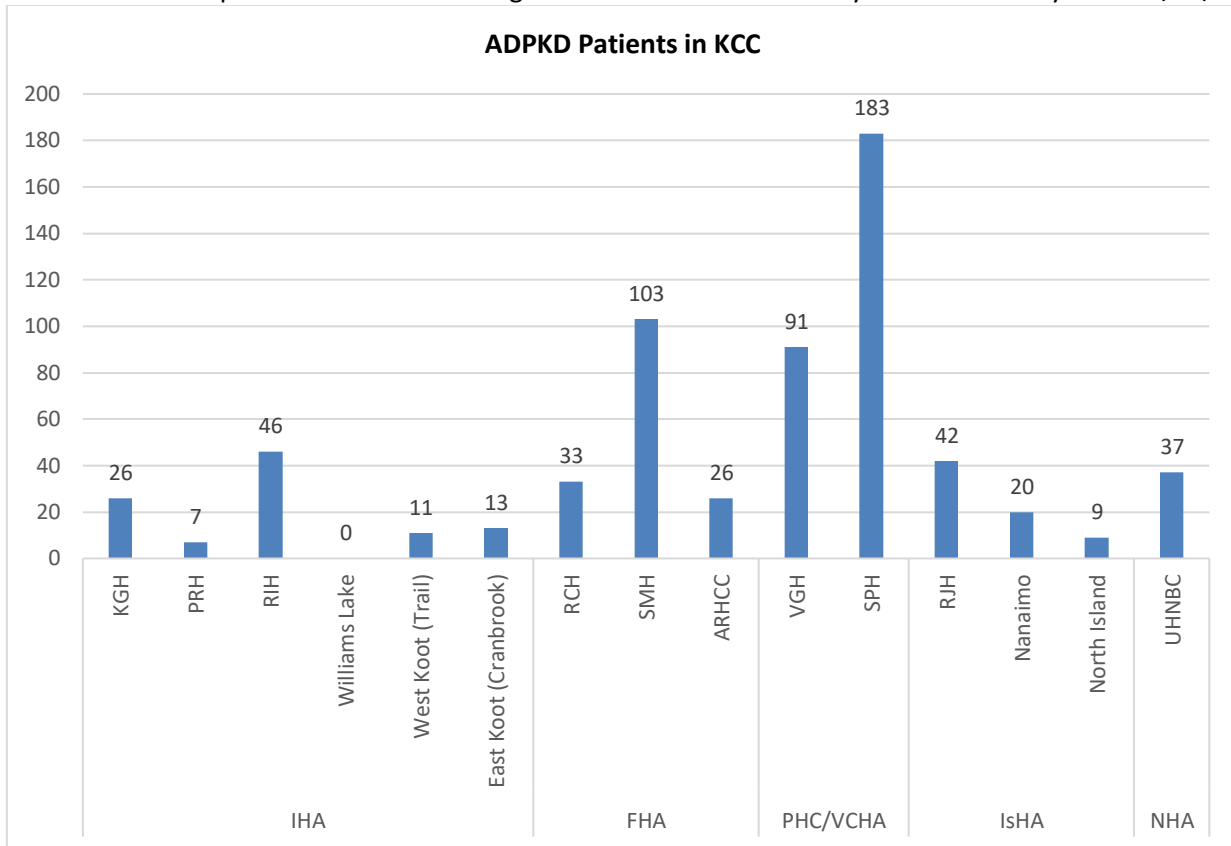
Total number of active patients with ADPKD diagnosis registered as of March 31 each year (2015 to 2024) regardless of setting (KCC, MD office, dialysis, transplant).

Report Date As Of	2015	2016	2017	2018	2019	2020	2021	2022	2023	2024
Total number of registered ADPKD patients	709	772	1041	1121	1179	1292	1352	1381	1431	1496
ADPKD patients followed in KCC as of report date	211 (30%)	214 (28%)	256 (25%)	282 (25%)	342 (29%)	452 (35%)	519 (38%)	554 (40%)	597 (42%)	647 (43%)

Total number of active patients with ADPKD diagnosis in BC regardless of setting (KCC, MD office, dialysis, transplant) as of March 31, 2024

	#
Total number of active patients with ADPKD diagnosis in BC as of period end	1496
By Health Authority:	
IHA	154
FHA	365
VCH+PHC	629
IsHA	254
NHA	57
Patients whose primary nephrologist does not belong to any HARP/unknown	37

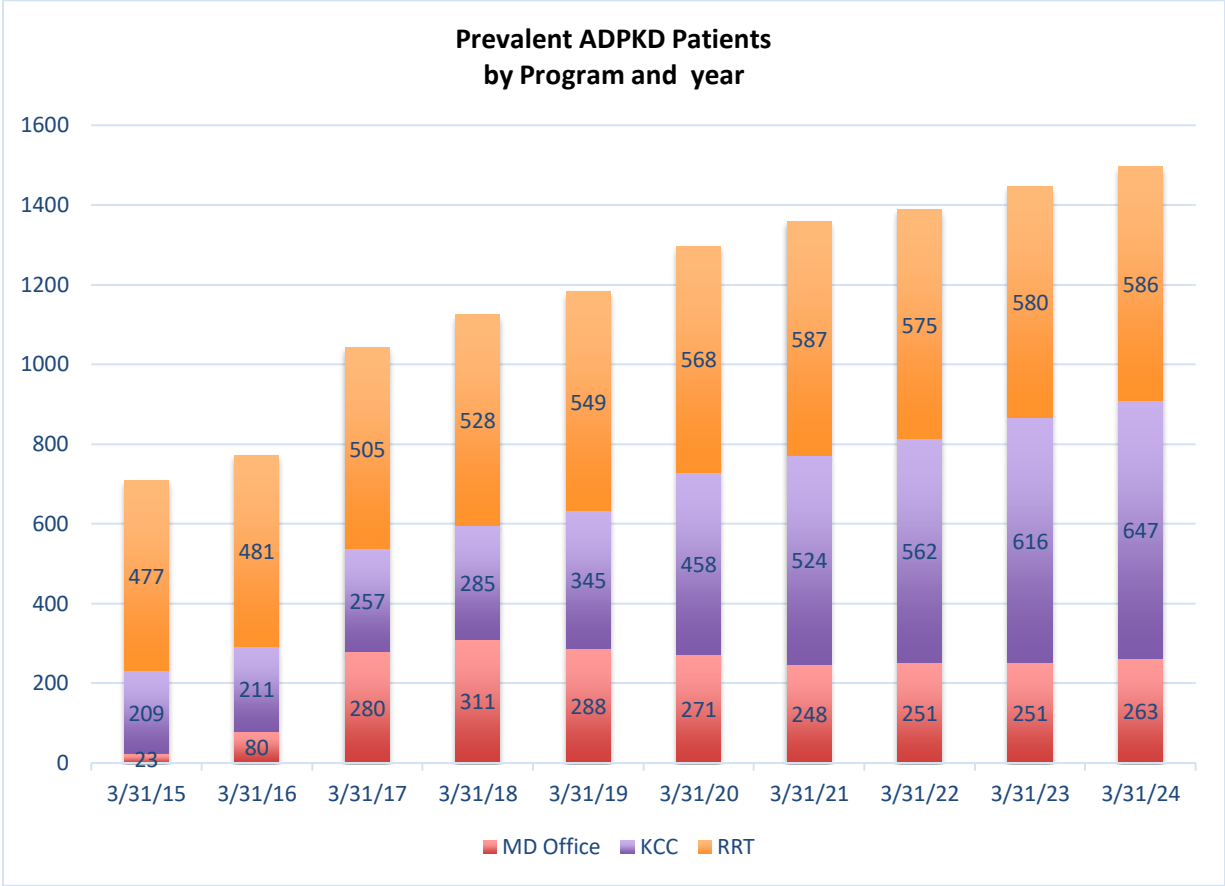
Number of active patients with ADPKD diagnosis followed in KCC and by health authority as of 03/31/2024.



Number/proportion of active patients with ADPKD diagnosis by modality as of 03/31/2024.

	#(%)
Total number of active patients with ADPKD diagnosis in BC as of period end	1496
By modality:	
Active patients in pre-ESKD care	910 (61%)
Active patients in KCC care	647 (43%)
Active patients whose initial registration occurred in MD office	719 (67%)
Active patients on RRT	586 (39%)

Number/proportion of active patients with ADPKD diagnosis in pre-ESKD care (KCC or MD offices) and renal replacement therapy (RRT) = dialysis modalities and transplant. Historical data shown from 2015 to 2024:



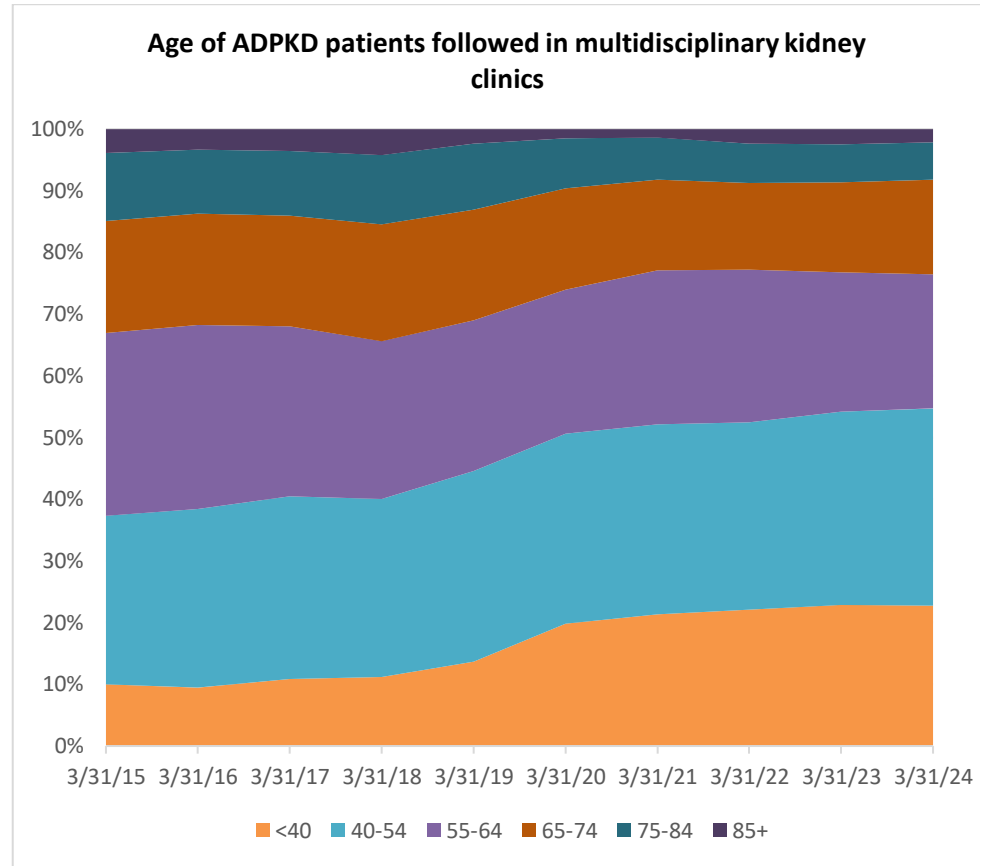
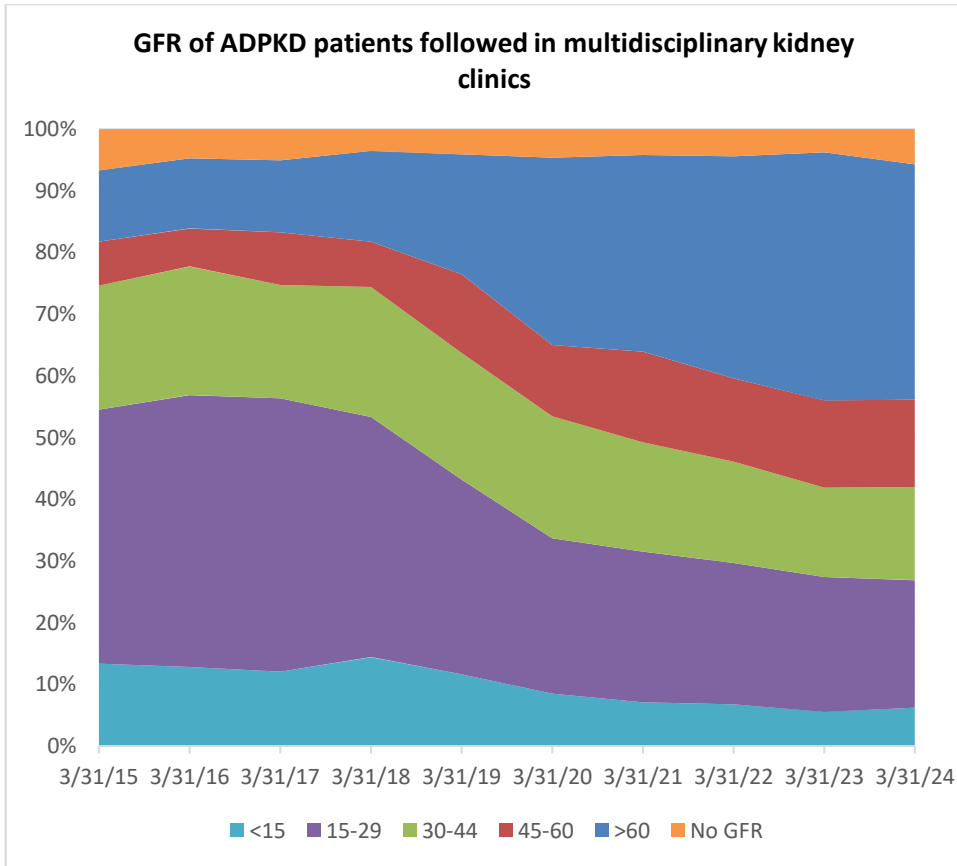
3.2 Clinical Characteristics of KCC patients with ADPKD Diagnosis

	#(%)
Total number of KCC patients with ADPKD diagnosis	647
Sex:	
Female	305 (47)
Male	342 (53)
Age (years):	
<40	147 (23)
40-54	207 (32)
55-64	141 (22)
65-74	99 (15)
75-84	39 (6)
>=85	14 (2)
Latest GFR (mL/min) during last 12 months:	
>60	247 (38)
45-60	91 (14)
30-44	98 (15)
15-29	134 (21)
<15	40 (6)
No value	37 (6)

Number/proportion of active patients with ADPKD diagnosis in BC as of March 31, 2024

	Unit	
	#	
Total number of active patients with ADPKD diagnosis in BC as of period end	1496	
Patients with blood pressure measurements during last 12 months	#(%)	969 (65)
Mean number of blood pressure measurements per Patient year	Mean #	3.2
Mean blood pressure systolic/diastolic	Mean BP systolic/diastolic	130/78
Median systolic (IQR)	Median systolic (IQR)	128 (120-138)
Median diastolic (IQR)	Mean diastolic (IQR)	78 (72-84)
<110/75	%	5.3
110/75 – 120/80	%	14.6
120/80 – 140/90	%	49.2
>140/90	%	31.0
Number of Patients with BP measurements who meet HALT-PKD trial criteria (age 15-49, GFR>60)	#(%)	157 (16.2)
Proportion with blood pressure <110/75	#(%)	11 (7.0)

The following graphs show historical data from 2015 to 2024. These data demonstrate that since inception of the ADPKD Network, there is a trend towards inclusion of greater proportions of younger and higher eGFR patients with ADPKD diagnosis in BC KCCs.

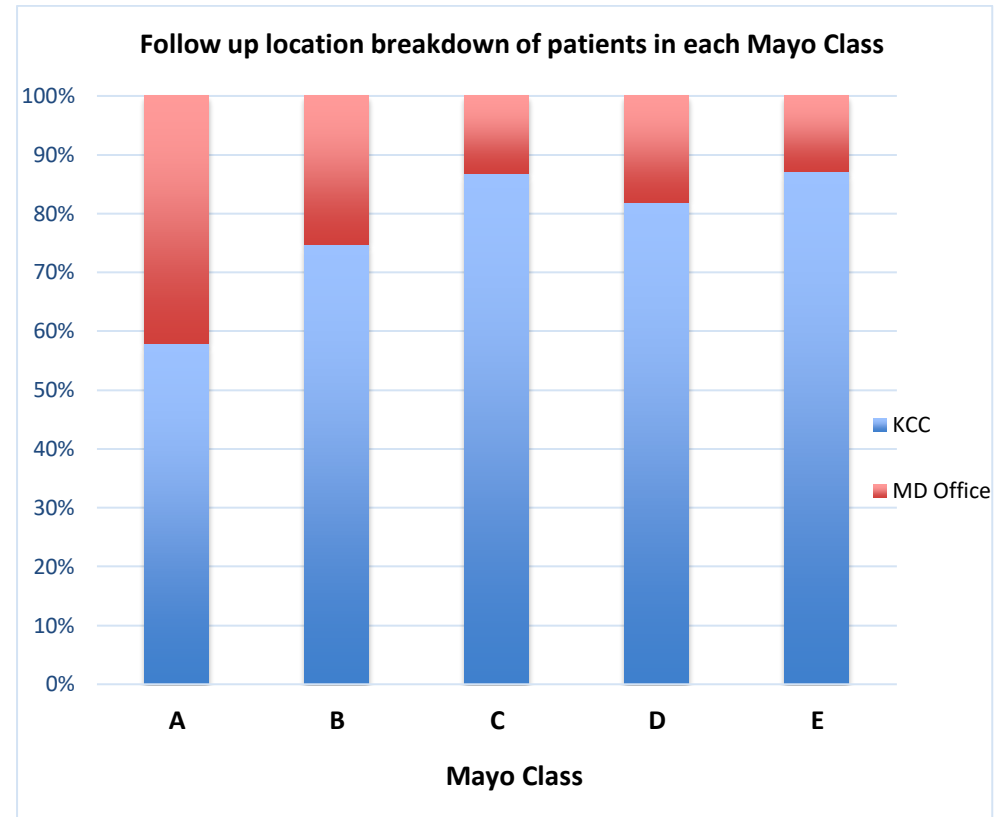
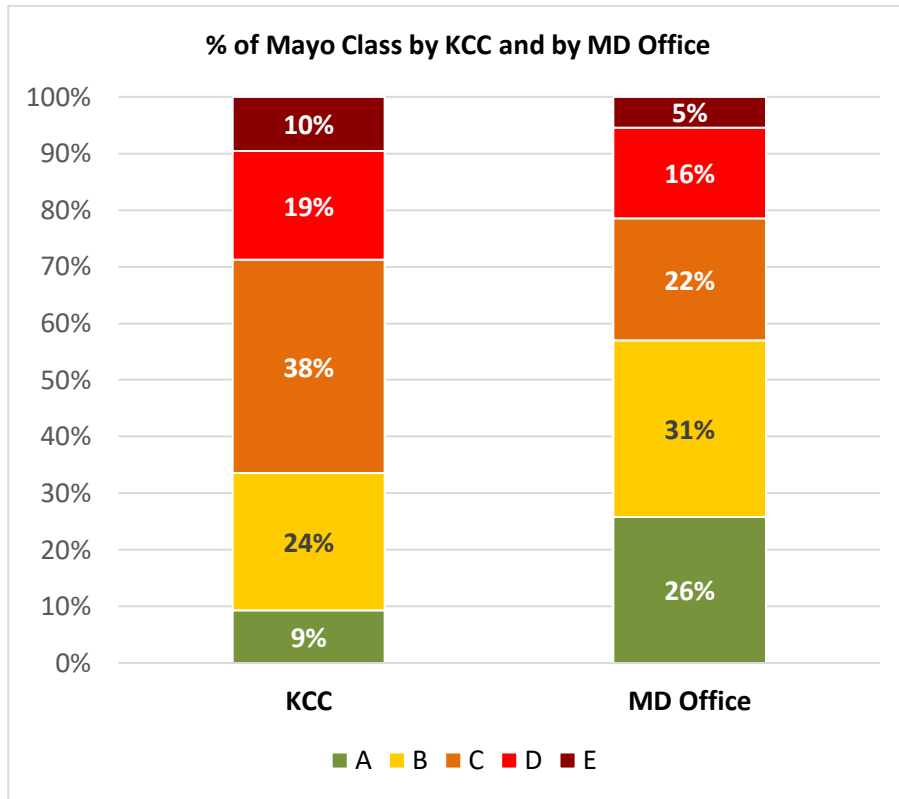


3.3 ADPKD Imaging

Total number and percentage of number of non-RRT patients in each health authority within reporting period.

	KCC (#/%)	MD office (#/%)
Total number of active non RRT patients with PKD diagnosis in BC with TKV results in PROMIS	354(54)	93(36)
By Health Authority:		
IHA	51(46.8)	2(25)
FHA	83(50)	27(28.1)
VCH+PHC	168(61.1)	19(33.3)
IsHA	33(45.8)	38(47.5)
NHA	19(51.4)	1(33.3)
Patients whose primary nephrologist does not belong to any HARP/unknown	0	6(37.5)

The following graphs are visual representations of table 3.3 above. The figure on the left shows the Mayo classification distribution of patients in KCC versus MD offices. The figure on the right considers all of the patients in each Mayo Class and shows the proportion of each class that are followed in KCC versus MD offices. Note that Mayo Class is normally distributed in the ADPKD population. Taken together, these data demonstrate that a greater portion of the more rapidly progressing categories (C, D and E) are followed in KCCs compared to the more slowly progressing categories (A and B).

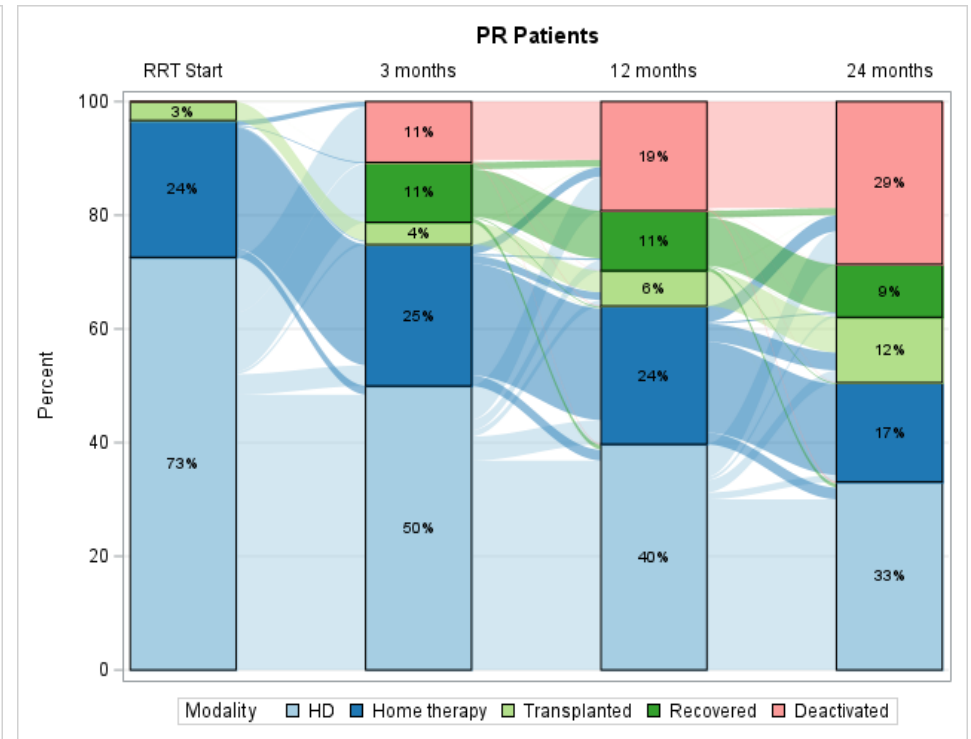
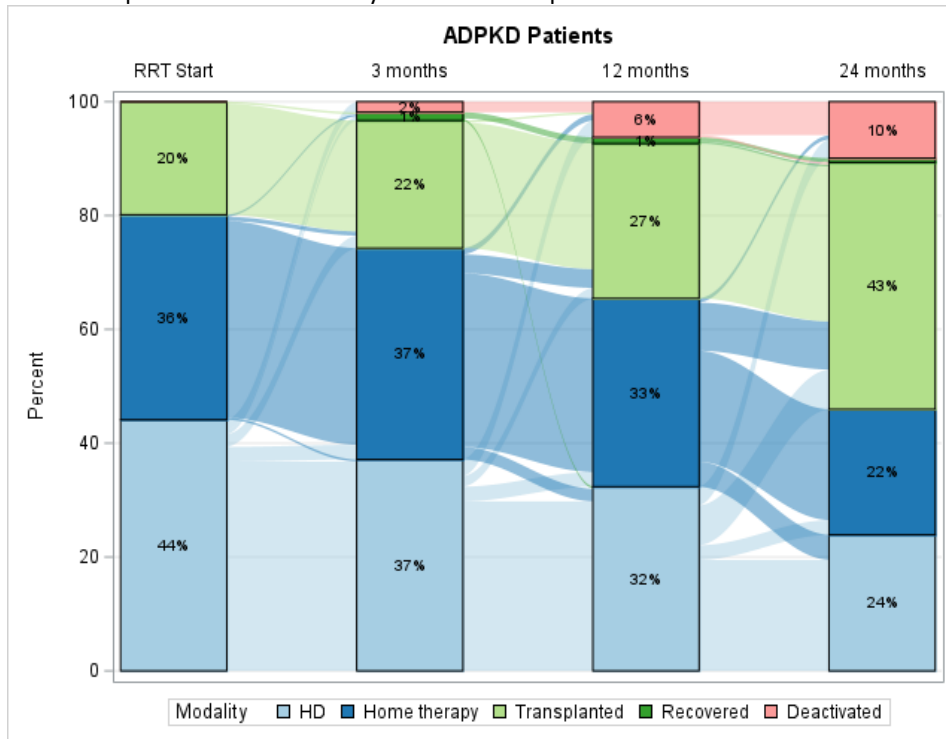


3.4 Trajectory of ADPKD patients after reaching End-Stage Kidney Disease

The following Sankey Plot shows the percentage of incident ADPKD RRT patients by treatment modality at RRT start (0 month) and status at 3 months, 1 year and 2 years post-RRT start. The same is shown for a comparator cohort of provincial renal (PR) patients. Incident RRT patients refer to patients reaching end-stage kidney disease and starting renal replacement therapy (RRT, i.e. Dialysis/transplant) for the first time. This first start is denoted by time 0 and each patient's trajectory is tracked at 3 months, 1 year, and 2 years after first RRT initiation.

Cohorts: ADPKD and PR patients, respectively, who started RRT between April 1, 2015, and March 31, 2022, with follow-up data collected for 2 years on each patient.

*Time period above allows 2 years of follow-up



4 Tolvaptan in ADPKD

4.1 Tolvaptan Utilization

Report Period 10/01/2020 to 03/31/2024 for the following tables:

Report Date:	Unit	*10/01/2020-03/31/2021	04/01/2021-03/31/2022	04/01/2022-03/31/2023	04/01/2023-03/31/2024
Total patients on tolvaptan	#	125	153	198	243

Report Date:	Unit	*10/01/2020-03/31/2021	04/01/2021-03/31/2022	04/01/2022-03/31/2023	04/01/2023-03/31/2024
Tolvaptan applications received	#	45	47	75	68
Tolvaptan applications approved	#(%)	38 (84.4)	39 (82.9)	66 (88.0)	62 (91.1)
Criteria for tolvaptan use in ADPKD approval as per tolvaptan application form:					
Group 1	#(%)	31 (81.6)	36 (92.3)	57 (86.4)	53 (85.6)
Group 2	#(%)	2 (5.3)	0	5 (7.6)	2 (3.2)
Group 3	#(%)	5 (13.2)	3 (7.7)	4 (6.1)	5 (8.1)
Not provided on application	#(%)	0	0	0	2 (3.2)
Tolvaptan applications declined	#(%)	5 (11.1)	8 (17.0)	7 (9.3)	1 (1.5)
Criteria for tolvaptan use in ADPKD declined as per tolvaptan application form:					
Group 1	#(%)	1 (20.0)	3 (37.5)	2 (28.6)	0
Group 2	#(%)	1 (20.0)	1 (12.5)	2 (28.6)	0
Group 3	#(%)	1 (20.0)	2 (25.0)	3 (42.9)	0
Not provided on application	#(%)	2 (40.0)	2 (25.0)	0	1 (100.0)
Withdrawn applications	#(%)	2 (4.4)	0	2 (2.7)	5 (7.4)
On tolvaptan, but do not meet any of the criteria as per tolvaptan application form	#	0	0	0	0

*6 months of data available from 10/01/2020 to 03/31/2021

**Group 1: Patients 18-55 years old who are similar to those in clinical trials

Group 2: Patients 55-65 years old who would have met criteria for the REPRISÉ trial², and who also have evidence of rapid disease progression.

Group 3: Patients ≥ 18 years of age with eGFR ≥ 25 mL/min/1.73 m² who do not otherwise fit into the criteria above but display other clear markers of renal progression related to their ADPKD.

Report Period 04/01/2022 to 03/31/2024 for the following tables:

	#
Number of patients on tolvaptan at any time during report period	243
FHA	81
VCH+PHC	81
IsHA	31
NHA	13
IHA	31
Patients whose primary nephrologist does not belong to any HARP	6

4.2 Clinical Parameters of ADPKD patients treated with Tolvaptan

Report Period 04/01/2022 to 03/31/2024 for the following tables:

Demographics:	Unit	
Total number of patients on tolvaptan	#	198
Age at start of treatment	years	45.0 (11.2)
Median continuous duration on treatment	Months (IQR)	20.9 (7.9, 40.9)
On treatment for \leq 3 months	#(%)	28 (11.5)
On treatment for 3-6 months	#(%)	21 (8.6)
On treatment for 6-12 months	#(%)	36 (14.8)
On treatment for 12-18 months	#(%)	26 (10.7)
On treatment for 18+ months	#(%)	132 (54.3)
Height	cm	171.4 (15.3)
eGFR, closest to start of therapy (median, IQR)	mL/min	57 (40,83)
Blood pressure within 12 months	mean systolic/diastolic	127/80
TKV - Distribution of Mayo class for current tolvaptan patients in all modalities with TKV, at most recent measurement		
A	#(%)	1 (0.4)
B	#(%)	28 (11.5)
C	#(%)	81 (33.3)
D	#(%)	58 (23.9)
E	#(%)	28 (11.5)
Missing	#(%)	47 (19.3)
% followed by KCC (at time of latest dispense)	#(%)	196 (81)
Dosage (at time of latest dispense):		
15 + 15	#(%)	8 (3)
30 + 15	#(%)	9 (4)
45 + 15	#(%)	92 (38)
60 + 30	#(%)	70 (29)
90 + 30	#(%)	63 (26)

Monitoring – All patients on tolvaptan within April 2021 to March 2022	Unit	
Total number of patients	#	153
Treatment persistence from time of first start/refill:		
3 months	#(%)	150 (98)
6 months	#(%)	143 (93)
12 months	#(%)	133 (87)
18 months	#(%)	122 (80)
24 months	#(%)	114 (75)

*Time period different to allow 2 years of follow-up

Monitoring – All patients on tolvaptan within April 2023 to March 2024	Unit	
Patients who had urine osmolality test done during report period	#	217
Proportion of patients who had had urine osmolality result less than 250	#(%)	198 (91)
Patients who did not have any AST nor ALT labs during monitoring period	#(%)	4 (2)
Patients who have had an AST or ALT above normal during report period	#(%)	50 (21)
Patients with labs that have higher AST/ALT		
LFTs > 1-3 x ULN	#	44
LFTs > 3-5 x ULN	#	3
LFTs > 5 x ULN	#	3
Out of patients who had lab results >3x ULN, how many were rechallenged and stayed on treatment until report end	#	3
Out of patients who were rechallenged, how many had another >3x abnormal lab result	#	0

Treatment eligibility	Unit	
Total number of active non RRT patients with PKD diagnosis and TKV results in BC as of report date	#	447
Proportion who satisfy criteria for tolvaptan treatment initiation based on BC application criteria	#(%)	189 (42.3)
Group 1	#	178
Group 2	#	11
Patients in each Mayo class who satisfy a criterion for tolvaptan treatment initiation based on BC application criteria:		
A	#	0
Patients in this group who have ever been prescribed tolvaptan	#(%)	N/A
B	#	19
Patients in this group who have ever been prescribed tolvaptan	#(%)	7(36.8)
C	#	80
Patients in this group who have ever been prescribed tolvaptan	#(%)	50(62.5)
D	#	64
Patients in this group who have ever been prescribed tolvaptan	#(%)	48(75)
E	#	26
Patients in this group who have ever been prescribed tolvaptan	#(%)	20(76.9)
Distribution of Mayo class among active non RRT patients who satisfy criteria for treatment initiation and who were ever prescribed tolvaptan	#(%)	125(66.1)
A	#(%)	0
B	#(%)	7(5.6)
C	#(%)	50(40)
D	#(%)	48(38.4)
E	#(%)	20(16)