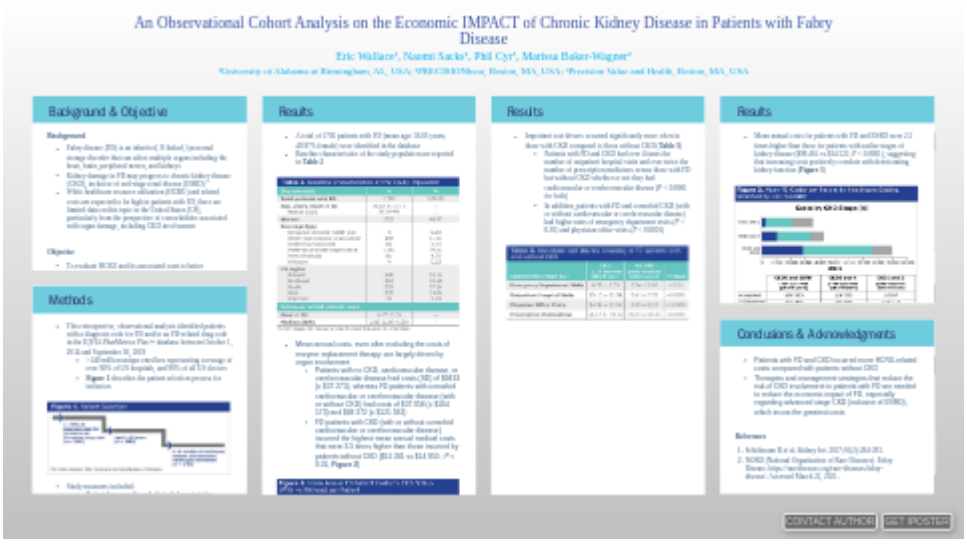
An Observational Cohort Analysis on the Economic IMPACT of Chronic Kidney Disease in Patients with Fabry Disease



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BACKGROUND & OBJECTIVE

Background

- Fabry disease (FD) is an inherited, X-linked, lysosomal storage disorder that can affect multiple organs including the heart, brain, peripheral nerves, and kidneys
- Kidney damage in FD may progress to chronic kidney disease (CKD), inclusive of end-stage renal disease (ESRD)[1,2]
- While healthcare resource utilization (HCRU) and related costs are expected to be high in patients with FD, there are limited data on this topic in the United States (US), particularly from the perspective of comorbidities associated with organ damage, including CKD involvement

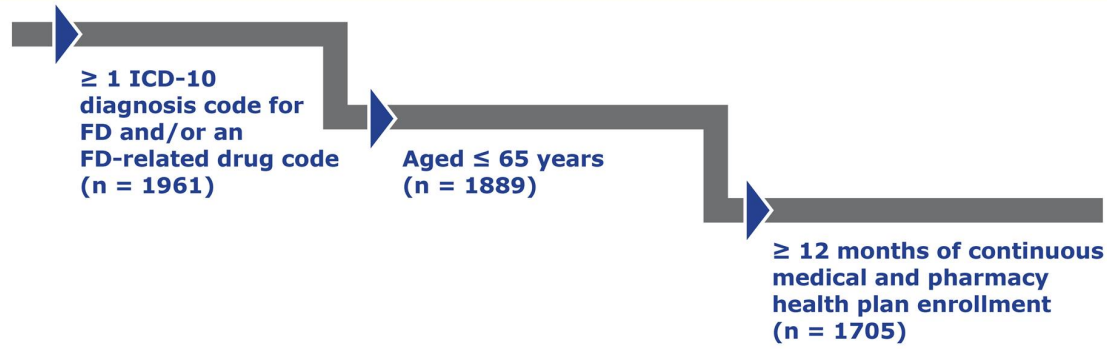
Objective

- To evaluate HCRU and its associated costs to better understand the economic impact of CKD in patients with FD

METHODS

- This retrospective, observational analysis identified patients with a diagnosis code for FD and/or an FD-related drug code in the IQVIA PharMetrics Plus™ database between October 1, 2014 and September 30, 2019
  - >140 million unique enrollees representing coverage of over 90% of US hospitals, and 90% of all US doctors
  - **Figure 1** describes the patient selection process for inclusion

Figure 1. Patient Selection



FD, Fabry disease; ICD, International Classification of Diseases

- Study measures included:
  - Patient demographic and clinical characteristics
  - Presence or absence of cardiovascular disease and/or cerebrovascular disease
  - Mean annual per patient rates of healthcare resource use (HCRU) by setting
  - Mean annual per patient costs overall and by setting
- **Table 1** lists the codes used to identify patients with FD

Table 1. Diagnosis Codes for FD and FD-Related Drugs

Diagnosis codes for FD		
ICD edition	**ICD-9**	**ICD-10**
Code	272.7	E75.21
Disease description	Lipidoses (includes Fabry, Anderson's, Gaucher, I-cell (mucolipidosis I), lipid storage NOS, Niemann-Pick, pseudo-Hurler or mucolipidosis III, triglyceride storage type I or II, Wolman or triglyceride storage type III	Fabry (-Anderson) disease
FD-Related drug codes		
Drug	**J-code**	**National Drug Code**
FABRAZYME (algasidase beta)	J0180	54868-0041-xx, 54868-0040-xx
GALAFOLD (migalastat)	Not applicable	71904-0100

FD, Fabry disease; ICD, International Classification of Diseases; NOS, not otherwise specified.

- Patients with FD were also classified by the presence of comorbid cardiovascular disease, cerebrovascular disease, or CKD (patients could fall into more than one of these groups)
- Patient demographics, comorbid diagnoses, and per-patient HCRU and related costs were analyzed during the follow-up period (which was a minimum of 12 months for each patient)

RESULTS

- A total of 1705 patients with FD (mean age: 36.03 years; 48.97% female) were identified in the database
- Baseline characteristics of the study population are reported in **Table 2**

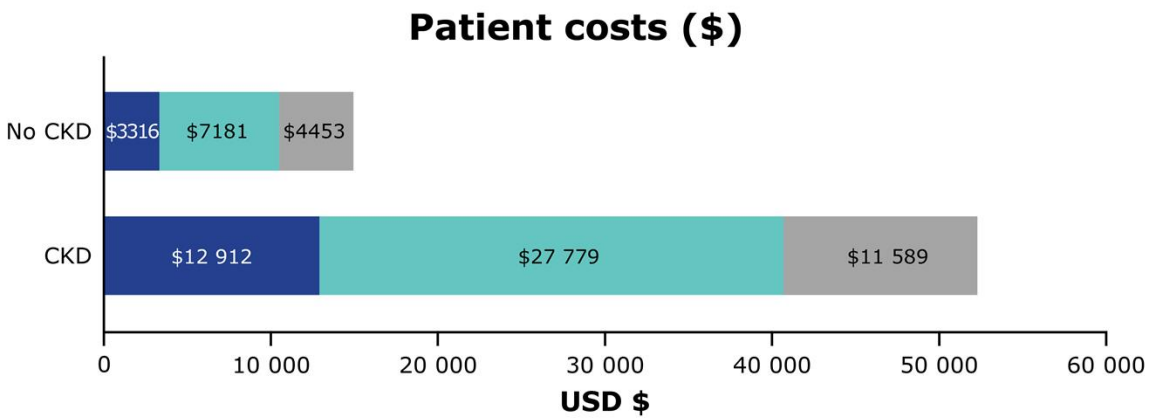
Table 2. Baseline Characteristics of the Study Population

Characteristic	N	%
Total patients with FD	1705	100.00
Age, years, mean ± SD	36.03 ± 17.12	–
Median (IQR)	38 (24–49)	–
Women	835	48.97
Coverage type		
Consumer directed health plan	4	0.23
Health maintenance organization	198	11.61
Indemnity/traditional	64	3.75
Preferred provider organization	1354	79.41
Point of service	81	4.75
Unknown	4	0.23
US region		
Midwest	548	32.14
Northeast	264	15.48
South	635	37.24
West	236	13.84
Unknown	22	1.29
Follow-up period, patient-years		
Mean ± SD	4.75 (3.3)	–
Median (IQR)	3.83 (2.08–6.58)	–

FD, Fabry disease; IQR, interquartile range; SD, standard deviation; US, United States.

- Mean annual costs, even after excluding the costs of enzyme replacement therapy, are largely driven by organ involvement
  - Patients with no CKD, cardiovascular disease, or cerebrovascular disease had costs (SD) of $9433 (± $37 273), whereas FD patients with comorbid cardiovascular or cerebrovascular disease (with or without CKD) had costs of $37 058 (± $104 173) and $48 372 (± $121 582)
  - FD patients with CKD (with or without comorbid cardiovascular or cerebrovascular disease) incurred the highest mean annual medical costs that were 3.5 times higher than those incurred by patients without CKD ($52 281 vs $14 950; $P < 0.01$; **Figure 2**)

Figure 2. Mean Annual FD Patient Costs by CKD Status (With vs Without) per Patient



	CKD (1235 observed patient years)	No CKD (6858 observed patient years)
Inpatient	$12 912	$3316
Outpatient	$27 779	$7181
Pharmacy	$11 589	$4453

Inpatient Outpatient Pharmacy

[a]Total costs for patients with CKD compared with patients without CKD ($52 281 vs $14 950) excluded enzyme replacement therapy.

CKD, chronic kidney disease.

RESULTS

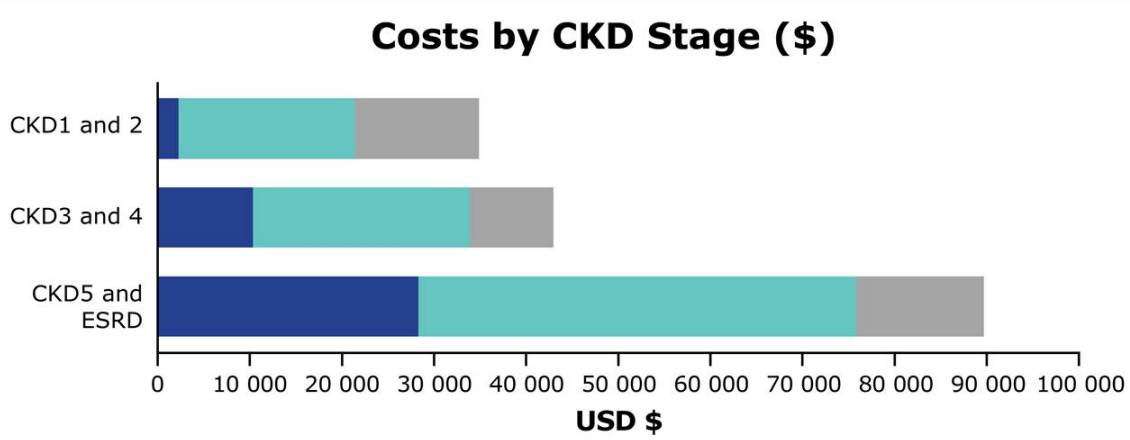
- Important cost drivers occurred significantly more often in those with CKD compared to those without CKD (**Table 3**)
  - Patients with FD and CKD had over 4 times the number of outpatient hospital visits and over twice the number of prescription medicines versus those with FD but without CKD whether or not they had cardiovascular or cerebrovascular disease ($P < 0.0001$ for both)
  - In addition, patients with FD and comorbid CKD (with or without cardiovascular or cerebrovascular disease) had higher rates of emergency department visits ($P < 0.01$) and physician office visits ($P < 0.0001$)

Table 3. Important cost drivers occurring in FD patients with and without CKD

Cost Drivers (Mean SD)	CKD (1235 observed patient years)	No CKD (6858 observed patient years)	*P*-value
Emergency Department Visits	0.77 ± 3.53	0.54 ± 2.03	<0.01
Outpatient Hospital Visits	15.16 ± 31.94	3.41 ± 7.78	<0.0001
Physician Office Visits	14.92 ± 13.51	9.75 ± 12.8	< 0.0001
Prescription Medications	58.47 ± 70.72	22.81 ± 30.23	<0.0001

- Mean annual costs for patients with FD and ESRD were 2.5 times higher than those for patients with earlier stages of kidney disease ($98 461 vs $34 521; $P < 0.0001$), suggesting that increasing costs positively correlate with deteriorating kidney function (**Figure 3**)

Figure 3. Mean FD Costs[a] per Patient by Healthcare Setting, Stratified by CKD Severity



	CKD5 and ESRD (324 observed patient years)	CKD3 and 4 (465 observed patient years)	CKD1 and 2 (336 observed patient years)
Inpatient	$28 330	$10 333	$2287
Outpatient	$47 493	$23 520	$19 118
Pharmacy	$13 846	$9103	$13 479

Inpatient Outpatient Pharmacy

[a]Costs excluded enzyme replacement therapy.

CKD, chronic kidney disease; ESRD, end-stage renal disease; FD, Fabry disease.

CONCLUSIONS & REFERENCES

- Patients with FD and CKD incurred more HCRU-related costs compared with patients without CKD
- Therapies and management strategies that reduce the risk of CKD involvement in patients with FD are needed to reduce the economic impact of FD, especially regarding advanced stage CKD (inclusive of ESRD), which incurs the greatest costs

References

1. Schiffmann R et al. *Kidney Int*. 2017;91(2):284-293.
2. NORD (National Organization of Rare Diseases). Fabry Disease. https://rarediseases.org/rare-diseases/fabry-disease/. Accessed March 22, 2021.

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