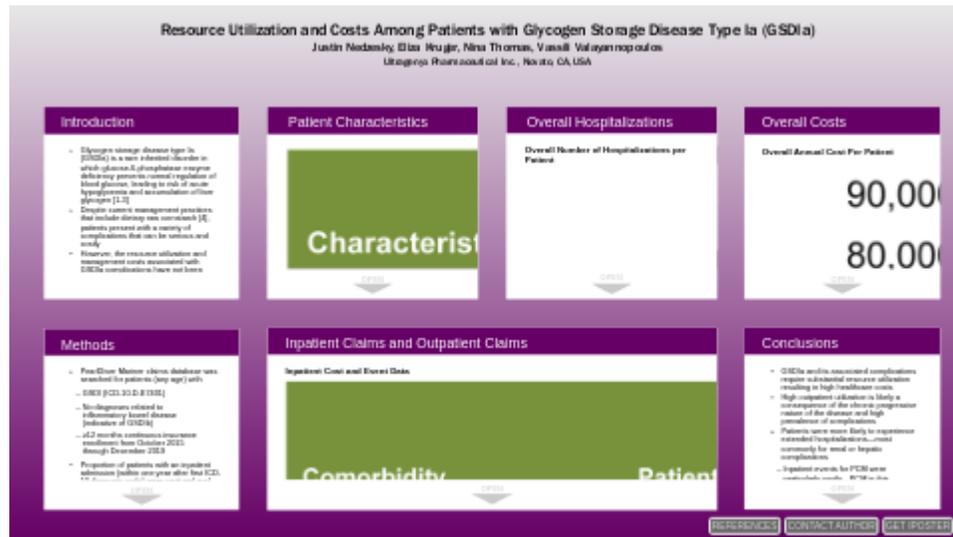


Resource Utilization and Costs Among Patients with Glycogen Storage Disease Type Ia (GSDIa)



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PRESENTED AT:



INTRODUCTION

- Glycogen storage disease type Ia (GSDIa) is a rare inherited disorder in which glucose-6-phosphatase enzyme deficiency prevents normal regulation of blood glucose, leading to risk of acute hypoglycemia and accumulation of liver glycogen [1-3]
- Despite current management practices that include dietary raw cornstarch [4], patients present with a variety of complications that can be serious and costly
- However, the resource utilization and management costs associated with GSDIa complications have not been quantified

PATIENT CHARACTERISTICS

Characteristic	Total (N=2641)	Age <18 Years (n=1223)	Age ≥18 Years (n=1418)
Sex, n (%)			
Female	862 (33)	217 (18)	645 (45)
Male	1779 (67)	1006 (82)	773 (55)
Mean (SD) age, years	26.9 (24.3)	4.7 (4.8)	46.0 (17.1)
Mean (SD) duration of enrollment, years	3.2 (0.9)	3.1 (0.9)	3.3 (0.8)
Type of insurance plan, n (%)			
Commercial	1825 (69)	837 (68)	988 (70)
Medicaid	497 (19)	327 (27)	170 (12)
Medicare	164 (6)	*(<1)	161 (11)
US Department of Veterans Affairs	63 (2)	11 (1)	52 (4)
Not available	92 (3)	45 (4)	47 (3)
Region, n (%)			
Northeast	1069 (40)	628 (51)	441 (31)
South	881 (33)	332 (27)	549 (39)
Midwest	414 (16)	163 (13)	251 (18)
West	240 (9)	69 (6)	171 (12)
Unknown	37 (<1)	31 (3)	*(<1)

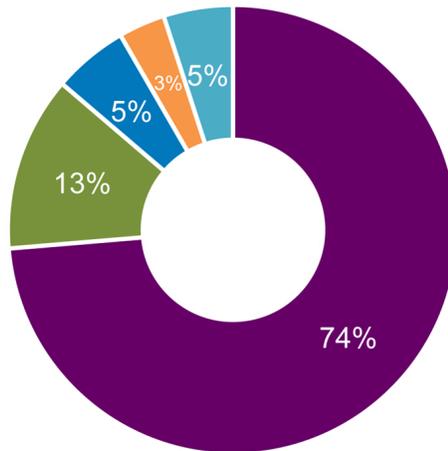
*Patient counts of 1–10 were not reported for privacy reasons.

OVERALL HOSPITALIZATIONS

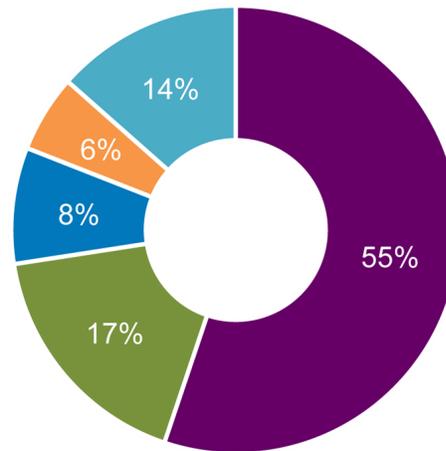
Overall Number of Hospitalizations per Patient



Pediatrics (<18)

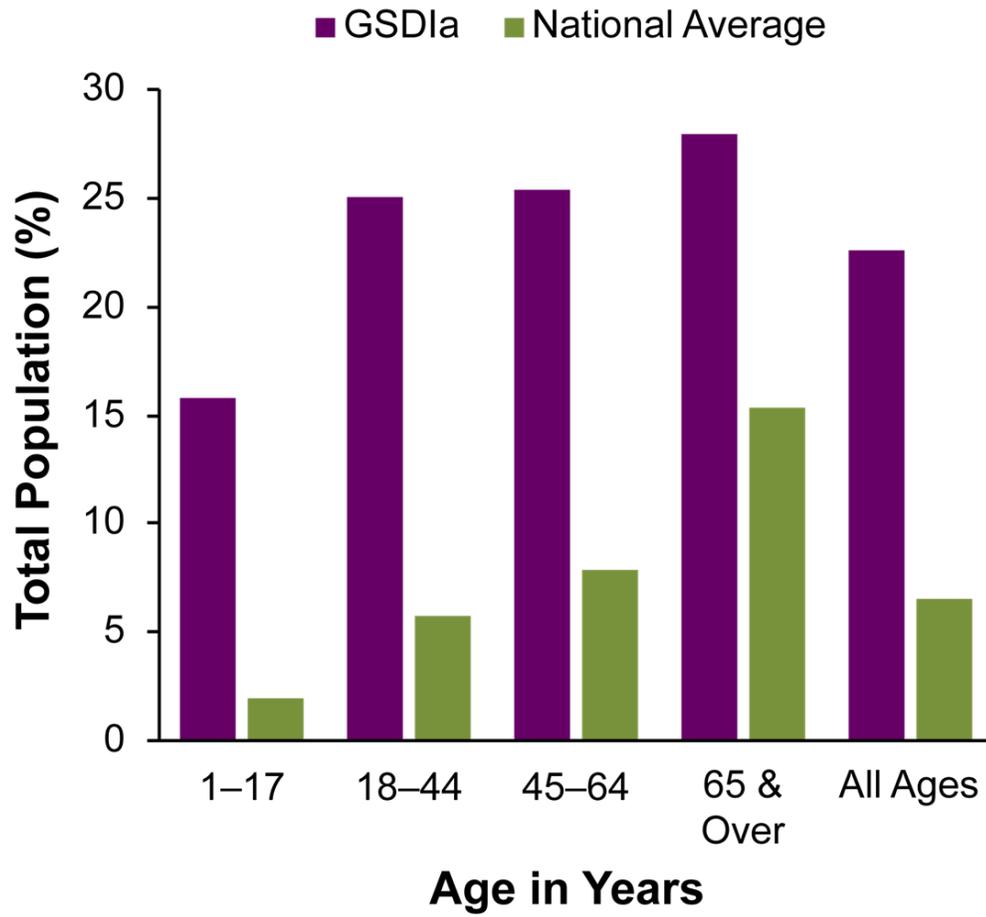


Adults (≥18)



- Overall, 36% of patients were hospitalized during the ~3-year enrollment
- Adult patients experienced more inpatient events than pediatric patients
- Multiple hospitalizations were common
- Mean LOS was 9.7 days

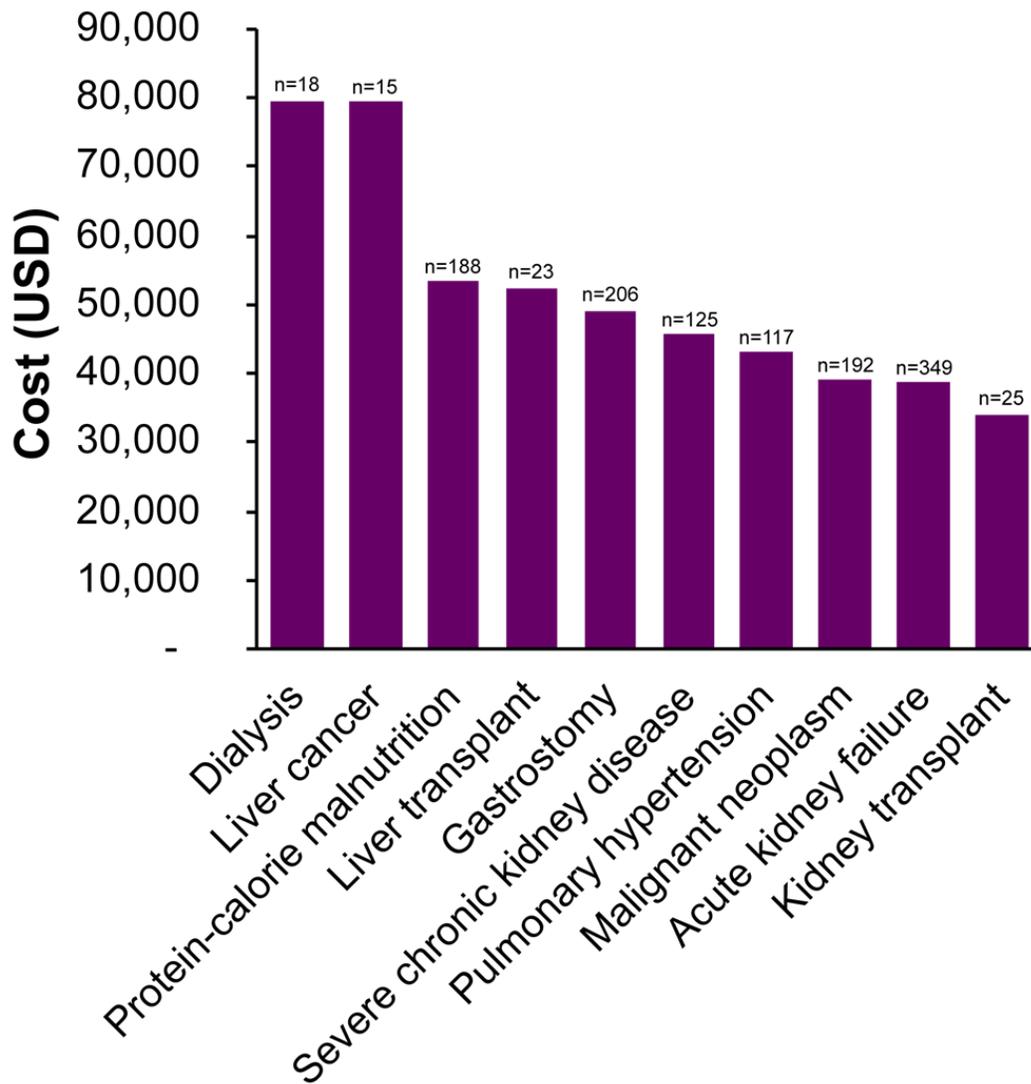
Proportion of Patients with ≥1 Inpatient Event



- In each age group, patients with GSDIa were more likely to be hospitalized compared with the 2017 CDC national average

OVERALL COSTS

Overall Annual Cost Per Patient



- GSDIa was associated with high-cost complications often related to kidney disease and cancer

METHODS

- PearlDiver Mariner claims database was searched for patients (any age) with
 - GSDI (ICD-10-D-E7401)
 - No diagnoses related to inflammatory bowel disease (indicative of GSDIb)
 - ≥ 12 months continuous insurance enrollment from October 2015 through December 2019
- Proportion of patients with an inpatient admission (within one year after first ICD-10 diagnosis code) were captured and compared with corresponding national average hospitalization data from the CDC
- Complications were defined by grouping related ICD-10 diagnosis codes
- Number of claims, events, costs, and length of stay (LOS) information was collected for claims with an ICD-10 code in the primary or secondary billing position
 - Outpatient events were defined as unique calendar days with a non-bundled claim with reimbursement
 - Inpatient events were defined by unique hospital admissions
 - Mean LOS was calculated based on inpatient procedures
 - Inpatient costs and LOS were compared with Healthcare Cost and Utilization Project (HCUP) national descriptive statistics
- Overall costs were captured for each complication and included all claims (any diagnosis, any billing position) from time of first complication ICD-10 diagnosis code to end of enrollment
 - Costs were annualized based on span of cost collection (from time of first complication diagnosis to end of enrollment)

INPATIENT CLAIMS AND OUTPATIENT CLAIMS

Inpatient Cost and Event Data

Comorbidity	Patients (n)	Claims (n)	Reimbursed Events (n)	Mean Cost per Event (USD)			Mean LOS per Event (Days)		
				GSDIa Patients	HCUP Comparator	Difference	GSDIa Patients	HCUP Comparator	Difference (%)
GSDIa	328	400	110	\$21,977	\$14,580	\$7,397	5.3	5.97	89
Chronic kidney disease	68	184	62	\$33,375	\$14,513	\$18,863	6.4	5.85	110
Acute kidney failure	68	91	35	\$46,766	\$9,526	\$37,240	10.9	4.92	222
Hypertension	51	87	31	\$38,982	\$12,437	\$26,545	6.4	5.25	121
Malignant neoplasm	34	70	30	\$39,384	\$20,471	\$18,913	6.2	5.95	104
Seizure	32	54	19	\$29,443	\$10,755	\$18,688	4.6	3.69	124
Anemia	45	52	22	\$22,505	\$6,572	\$15,933	4.4	2.87	154
Protein-calorie malnutrition	31	43	11	\$43,303	\$15,305	\$27,998	11.7	7.82	150
Osteoarthritis	22	32	12	\$25,680	\$15,970	\$9,710	2.4	2.16	110
Kidney and/or liver transplant	14	25	7	\$37,343	\$21,320	\$16,023	8.7	6.41	135

- Most common inpatient events were related to GSDIa or renal complications
- Cost per event for patients with GSDIa is substantially higher than national average data (from HCUP) for inpatient events
 - Most costly events were for acute kidney failure (AKF) and protein-calorie malnutrition (PCM) and aligned with highest LOS
- Patients with GSDIa experience extended stays versus the average US population
 - Both AKF and PCM had mean LOS >10 days

Outpatient Cost and Event Data

Complication	Patients (n)	Claims (n)	Reimbursed Events (n)	Mean Cost per Event (USD)
Hypertension	722	6460	5024	\$442
Chronic kidney disease	249	5908	4425	\$961
GSDIa	1271	5152	3311	\$708
Anemia	701	4545	3437	\$743
Gastrostomy	158	3925	2530	\$1,454
Malignant neoplasm	155	3777	2403	\$1,347
Abdominal pain	762	3218	2174	\$534
Seizure	254	2484	1623	\$614
Nausea and/or vomiting	640	2124	1440	\$656
Hypoglycemia	161	1943	1426	\$822

- Most common outpatient events were hypertension, CKD, and GSDIa related events
- High outpatient resource utilization was likely multifactorial
 - GSDIa requires intensive monitoring
 - Prevalent chronic comorbidities

CONCLUSIONS

- GSDIa and its associated complications require substantial resource utilization resulting in high healthcare costs
- High outpatient utilization is likely a consequence of the chronic progressive nature of the disease and high prevalence of complications
- Patients were more likely to experience extended hospitalizations—most commonly for renal or hepatic complications
 - Inpatient events for PCM were particularly costly—PCM in this population appears to be associated with renal failure and cancer
- Abdominal pain, nausea/vomiting, and gastrostomy are complications which have been reported with cornstarch supplementation; however, the extent of cornstarch related complications in this study are unknown
- Despite current dietary management recommendations for GSDIa, many patients had claims for hypoglycemia

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