EPH241

Comparison of Comorbidities Between Adults and Children with Glycogen Storage Disease Type Ia (GSDIa)

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INTRODUCTION

- Glycogen storage disease type Ia (GSDIa) is a rare inherited disorder resulting in acute hypoglycemia due to impaired release of glucose from glycogen[1,2]
- Despite dietary management practices to prevent hypoglycemia in patients with GSDIa, serious complications still occur in children and throughout adulthood[2]
- Real-world data on complications associated with GSDIa are limited

OBJECTIVE

- This retrospective cohort study compared the prevalence of complications in adults and children with GSDIa

METHODS

- Using ICD-10 diagnosis codes, the PharMetrics Plus® database was searched for patients in the US with ≥2 GSDI claims (E74.01) from January 2016 through February 2020, with ≥12 months continuous enrollment beginning prior to March 2019 (for one year of follow-up before COVID-19), no data quality issues, and no inflammatory bowel disease diagnoses (indicative of GSDIb)
- Non-GSDIa comparators were identified using 10:1 exact matching (age, sex, payer type, and enrollment start/end date)
- ICD-10 diagnosis codes in any billing position for inpatient and outpatient claims were identified for complications selected based on literature and clinical expert suggestions
- Prevalence of complications was assessed across all ages and among pediatric and adult groups and was summarized descriptively
- Patient counts of 1−10 were not reported for privacy reasons

RESULTS

Patient Demographics

- In total, 557 patients with GSDIa and 5570 matched comparators were identified (Table 1)
  - Demographics were balanced between matched cohorts
  - Across the GSDIa and comparator cohorts, 67% were adults and 63% were male; most were from the South (37−40%) or Northeast (17%−33%) regions and most were self-insured (44%−49%) or had commercial insurance (44%−45%)
  - The GSDIa cohort included 372 adults (median age, 41 years) and 185 children (median age, 7 years)

Table 1. Patient Demographics

	Pediatric		Adults		Total	
	GSDIa (n=185)	Comparator (n=1850)	GSDIa (n=372)	Comparator (n=3720)	GSDIa (n=557)	Comparator (n=5570)
Mean (SD) Age, Years	7.4 (5.5)	7.4 (5.5)	41.1 (15.2)	41.1 (15.2)	29.9 (20.4)	29.9 (20.4)
Children, n (%)	185 (100)	1850 (100)	0	0	185 (33.2)	1850 (33.2)
Adults, n (%)	0	0	372 (100)	3720 (100)	372 (66.8)	3720 (66.8)
Sex, n (%)						
Female	42 (22.7)	420 (22.7)	163 (43.8)	1630 (43.8)	205 (36.8)	2050 (36.8)
Male	143 (77.3)	1430 (77.3)	209 (56.2)	2090 (56.2)	352 (63.2)	3520 (63.2)
Geographic Region, n (%)						
South	56 (30.3)	699 (37.8)	150 (40.3)	1522 (40.9)	206 (37.0)	2221 (39.9)
Midwest	36 (19.5)	517 (28.0)	76 (20.4)	933 (25.1)	112 (20.1)	1450 (26.0)
Northeast	73 (39.5)	321 (17.4)	109 (29.3)	631 (17.0)	182 (32.7)	952 (17.1)
West	19 (10.3)	295 (16.0)	36 (9.7)	617 (16.6)	55 (9.9)	912 (16.4)
Unknown	1 (0.5)	18 (1.0)	1 (0.3)	17 (0.5)	2 (0.4)	35 (0.6)
Payer Type, n (%)						
Commercial	72 (38.9)	848 (45.8)	176 (47.3)	1897 (51.0)	248 (44.5)	2745 (49.3)
Self-insured	99 (53.5)	862 (46.6)	171 (46.0)	1573 (42.3)	270 (48.5)	2435 (43.7)
Medicaid	12 (6.5)	120 (6.5)	12 (3.2)	120 (3.2)	24 (4.3)	240 (4.3)
Medicare	0	0	13 (3.5)	130 (3.5)	13 (2.3)	130 (2.3)
Unknown	2 (1.1)	20 (1.1)	0	0	2 (0.4)	20 (0.4)
Year of First Enrollment, n (%)						
2016	127 (68.7)	1270 (68.7)	275 (73.9)	2750 (73.9)	402 (72.2)	4020 (72.2)
2017	29 (15.7)	290 (15.7)	49 (13.2)	490 (13.2)	78 (14.0)	780 (14.0)
2018	25 (13.5)	250 (13.5)	36 (9.7)	360 (9.7)	61 (11.0)	610 (11.0)
2019	4 (2.2)	40 (2.2)	12 (3.2)	120 (3.2)	16 (2.9)	160 (2.9)

SD, standard deviation.

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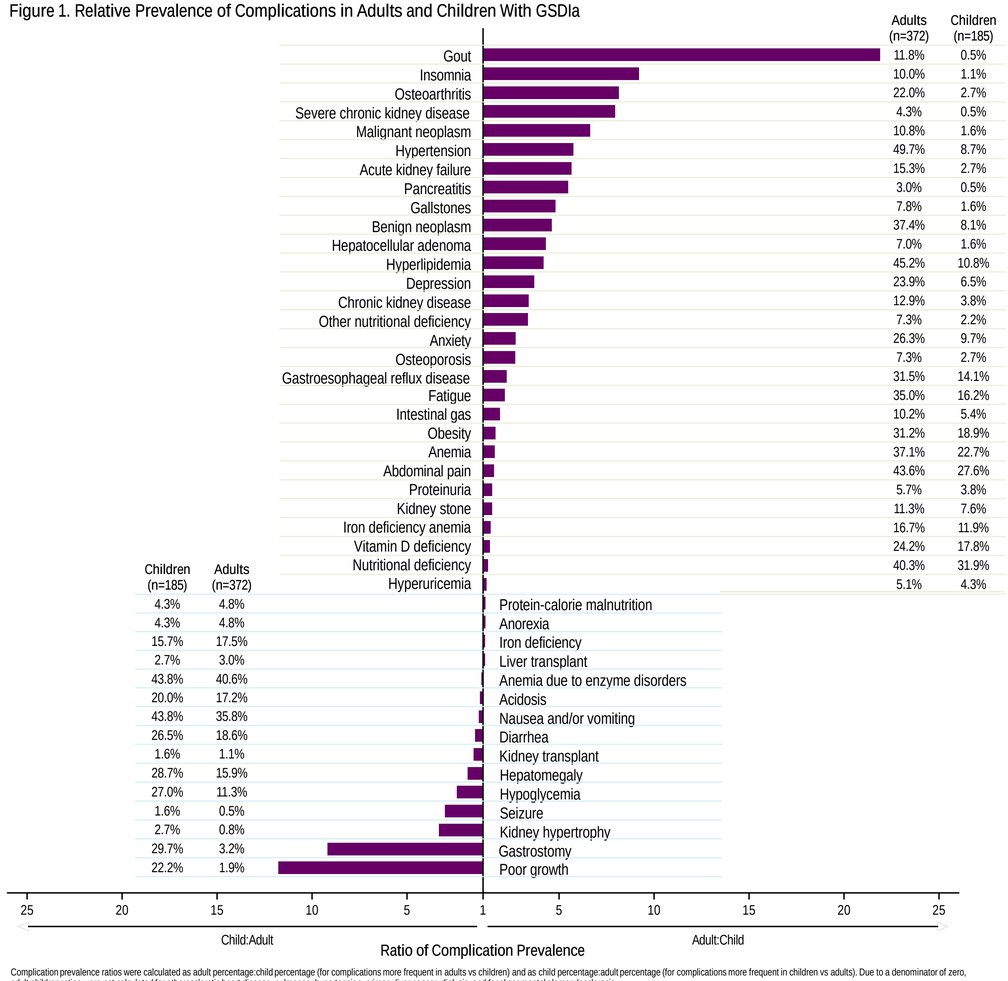
RESULTS (continued)

Complications in Adults vs Children with GSDIa

- Complications that occurred in adults with GSDIa but not in children with GSDIa were atherosclerotic heart disease (8.6%), pulmonary hypertension (3.0%), primary liver cancer (1.9%), dialysis (0.8%), and focal segmental glomerulosclerosis (0.3%)
- The most frequent complications in adults versus children (reported as the adult versus children ratio of prevalence percentages) included gout, insomnia, osteoarthritis, severe chronic kidney disease, malignant neoplasm, hypertension, acute kidney failure, pancreatitis, gallstones, benign neoplasm, hepatocellular adenoma, and hyperlipidemia (Figure 1)
- In contrast, complications occurring more often in children were poor growth, gastrostomy, kidney hypertrophy, seizure, hypoglycemia, hepatomegaly, kidney transplant, diarrhea, nausea and/or vomiting, acidosis, and anemia due to enzyme disorders (Figure 1)
- Complications occurring more often in adults than in children tended to reflect the chronic and progressive nature of GSDIa, whereas children tended to have more acute manifestations

Figure 1. Relative Prevalence of Complications in Adults and Children With GSDIa

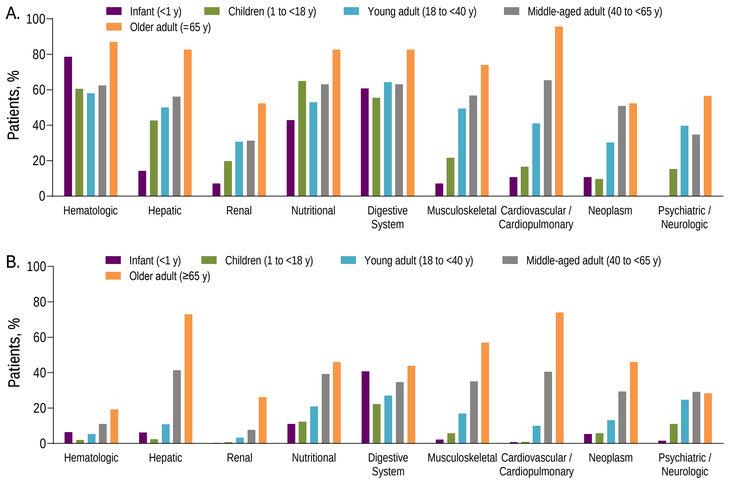


Complication prevalence ratios were calculated as adult percentage:child percentage (for complications more frequent in adults vs children) and as child percentage:adult percentage (for complications more frequent in children vs adults). Due to a denominator of zero, adult:children ratios were not calculated for atherosclerotic heart disease, pulmonary hypertension, primary liver cancer, dialysis, and focal segmental glomerulosclerosis.

Complication Frequency by Age

- In general, the frequency of each type of complication increased with age in patients with GSDIa and in comparators, with the highest prevalence occurring in those aged ≥65 years (Figure 2)

Figure 2. Frequency of Complication Types by Patient Age in Patients With GSDIa (A) and Comparators (B)



LIMITATIONS

- Medical claims may have been driven by reimbursement concerns that may not accurately reflect the medical condition. Additionally, the retrospective design limited information to encounters where an insurance claim was generated and may not have captured less serious complications
- Given that patient counts of 1 to 10 were not reported for privacy reasons, the analysis captured complication ICD-10 codes occurring in any billing position to be less restrictive, especially for rare but serious complications. Furthermore, it is possible that the prevalence of some conditions may be modestly overestimated as a result of bill practices
- Dietary management is not captured in claims data; therefore, this analysis could not evaluate the safety or efficacy of dietary management strategies among patients with GSDIa

CONCLUSIONS

- GSDIa is associated with numerous, potentially serious complications
- Compared with children, adults with GSDIa had a greater prevalence of chronic complications, potentially indicating the progressive nature of disease
- Children with GSDIa had more acute complications related to suboptimal metabolic control

REFERENCES

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