

Analysis of Healthcare Resource Utilization and Costs in Patients with Glycogen Storage Disease Type Ia (GSDIa)

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INTRODUCTION

- Glycogen Storage Disease Type Ia (GSDIa) is a serious, life-threatening, rare inherited disorder of carbohydrate metabolism that results from glucose-6-phosphatase deficiency, leading to glycogen accumulation in organs and tissues^{1,2}
- Patients with GSDIa experience fasting intolerance and require a continuous source of exogenous glucose to maintain glucose homeostasis³
- Despite best practices with dietary carbohydrate supplementation, patients experience serious complications and require frequent care
- This retrospective cohort study quantified healthcare resource utilization (HRU) and management costs associated with GSDIa

METHODS

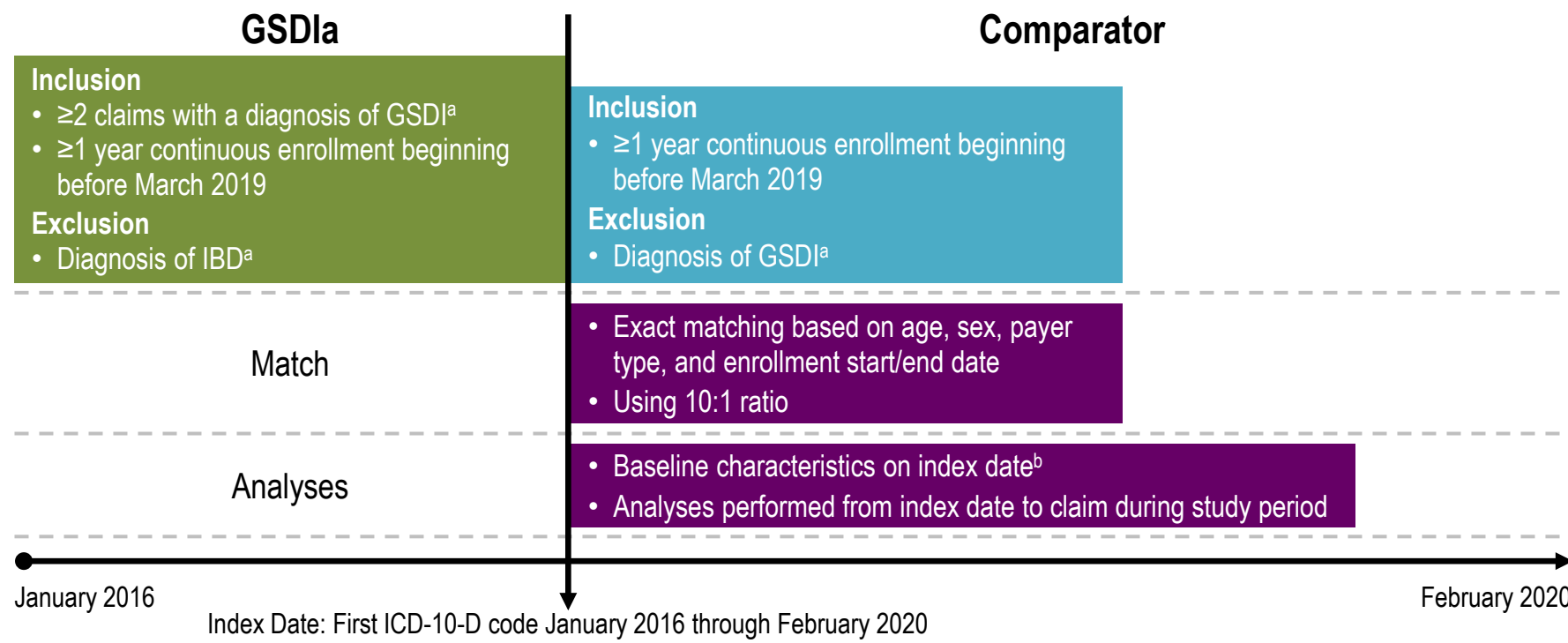
Data Source

- The IQVIA PharMetrics® Plus is a longitudinal database of adjudicated medical and pharmacy claims
- The deidentified integrated data include all paid medical and pharmacy (retail and mail order) claims for >150 million members from >70 United States commercial health plans
- The database contains inpatient and outpatient claims, diagnoses, and procedures based on International Classification of Diseases, Tenth Revision, Clinical Modification (ICD-10-CM), Healthcare Common Procedure Coding System (HCPCS), and Current Procedural Terminology (CPT) codes
- Because PharMetrics® is deidentified and compliant with the Health Insurance Portability and Accountability Act, institutional review board approval was not needed

Study Population

- The IQVIA PharMetrics® Plus database was searched for patients with:
 - ≥2 GSDI claims (ICD-10-E74.01) from January 2016–February 2020 with ≥12 months continuous insurance enrollment beginning February 2019 (one year follow-up before COVID-19)
 - no inflammatory bowel disease diagnoses (that may indicate GSDIb)
- Non-GSDIa comparators were identified using a ratio of 10:1 – exact age, sex, payer type, and enrollment start/end date matching
- HRU and costs were stratified by care setting (**Figure 1**)

Figure 1. Study Design



GSDI, glycogen storage disease type I; IBD, inflammatory bowel disease.
^aInternational Classification of Diseases, Tenth Revision. Inpatient or outpatient care setting, any diagnosis position. ^bBaseline characteristics included age, sex, region, and insurance plan type.

Statistical Analysis

- Descriptive statistics were used to summarize study outcomes
- Categorical variables were summarized as the number and percentage of patients
- Continuous variables were summarized using mean and standard deviation (SD)
- Patient counts of 1 to 10 were not reported for privacy reasons

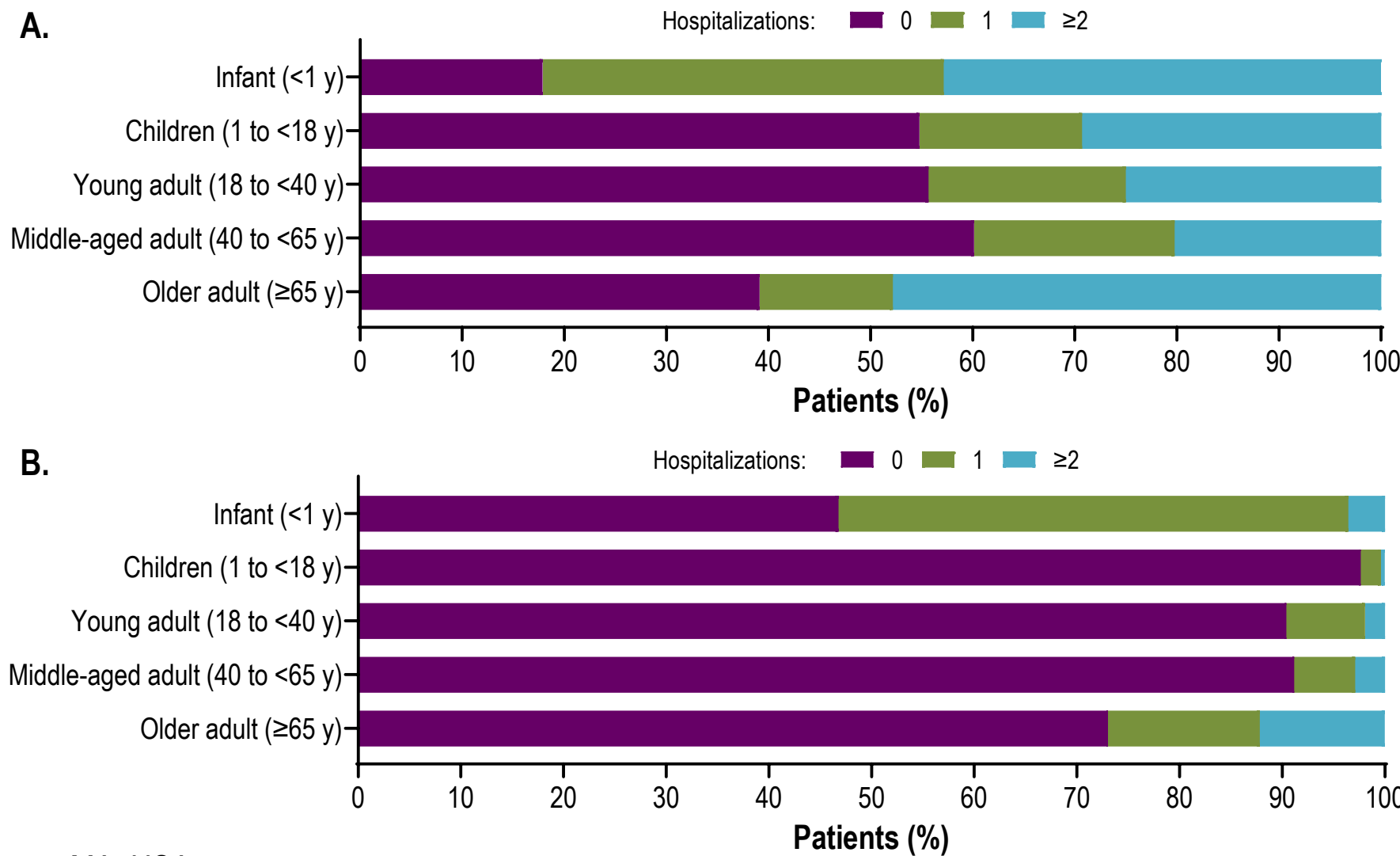
RESULTS

Table 1. Healthcare Resource Utilization

	Pediatric		Adult		Total	
	GSDIa (n=185)	Comparator (n=1850)	GSDIa (n=372)	Comparator (n=3720)	GSDIa (n=557)	Comparator (n=5570)
Inpatient						
Patients with ≥1 hospitalization, n (%)	94 (51)	186 (10)	161 (43)	384 (10)	255 (46)	570 (10)
Mean (SD) hospitalizations PPPY, n	0.6 (1.2)	0.1 (0.2)	0.5 (1.1)	0.1 (0.3)	0.5 (1.1)	0.1 (0.3)
Mean (SD) length of stay per hospitalization PPPY, days	2.7 (6.6)	0.2 (1.5)	3.2 (9.2)	0.4 (6.2)	3.1 (8.4)	0.4 (5.2)
Mean (SD) cost per hospitalization	\$29,510 (\$41,930)	\$16,120 (\$42,230)	\$33,910 (\$78,280)	\$18,250 (\$27,240)	\$32,200 (\$66,550)	\$17,690 (\$31,880)
Emergency Department Visits						
Patients with a visit, n (%)	129 (70)	557 (30)	258 (69)	1256 (34)	387 (70)	1813 (33)
Mean (SD) emergency department visits PPPY, n	1.2 (1.8)	0.2 (0.5)	1.3 (2.3)	0.3 (0.7)	1.3 (2.1)	0.3 (0.6)
Mean (SD) cost per visit	\$7400 (\$21,430)	\$1430 (\$9370)	\$7230 (\$39,560)	\$2610 (\$11,370)	\$7290 (\$34,680)	\$2290 (\$10,870)
Outpatient Hospital Visits						
Patients with a visit, n (%)	163 (88)	849 (46)	338 (91)	2230 (60)	501 (90)	3079 (55)
Mean (SD) outpatient hospital visits PPPY, n	5.8 (9.2)	0.6 (1.5)	5.9 (7.7)	1.6 (4.2)	5.9 (8.2)	1.2 (3.6)
Mean (SD) cost per visit	\$1030 (\$2150)	\$630 (\$2220)	\$1030 (\$4890)	\$790 (\$2710)	\$1030 (\$4170)	\$770 (\$2630)
Physician Office Visits						
Patients with a visit, n (%)	179 (97)	1786 (97)	359 (97)	3493 (94)	538 (97)	5279 (95)
Mean (SD) physician office visits PPPY, n	10.8 (16).2	5.4 (6.4)	10.4 (10.7)	6.3 (8.3)	10.5 (12.8)	6.0 (7.8)
Mean (SD) cost per visit	\$170 (\$230)	\$140 (\$180)	\$150 (\$340)	\$110 (\$200)	\$160 (\$310)	\$120 (\$200)
Other Outpatient Visits						
Patients with a visit, n (%)	159 (86)	929 (50)	326 (88)	2417 (65)	485 (87)	3346 (60)
Mean (SD) other outpatient visits PPPY, n	15.0 (42.2)	1.0 (4.6)	6.7 (16.3)	1.8 (6.9)	9.5 (28.0)	1.5 (6.2)
Mean (SD) cost per visit	\$410 (\$1550)	\$200 (\$650)	\$300 (\$2870)	\$270 (\$1360)	\$350 (\$2280)	\$250 (\$1250)
Pharmacy Visits						
Patients with a pharmacy visit, n (%)	146 (79)	1198 (65)	331 (89)	2850 (77)	477 (86)	4048 (73)
Mean (SD) pharmacy visits PPPY, n	11.7 (39.4)	2.1 (4.0)	12.2 (17.6)	6.4 (10.9)	12.1 (26.8)	5.0 (9.4)
Mean (SD) cost per visit	\$460 (\$1240)	\$100 (\$370)	\$450 (\$2020)	\$180 (\$1110)	\$450 (\$1780)	\$170 (\$1040)

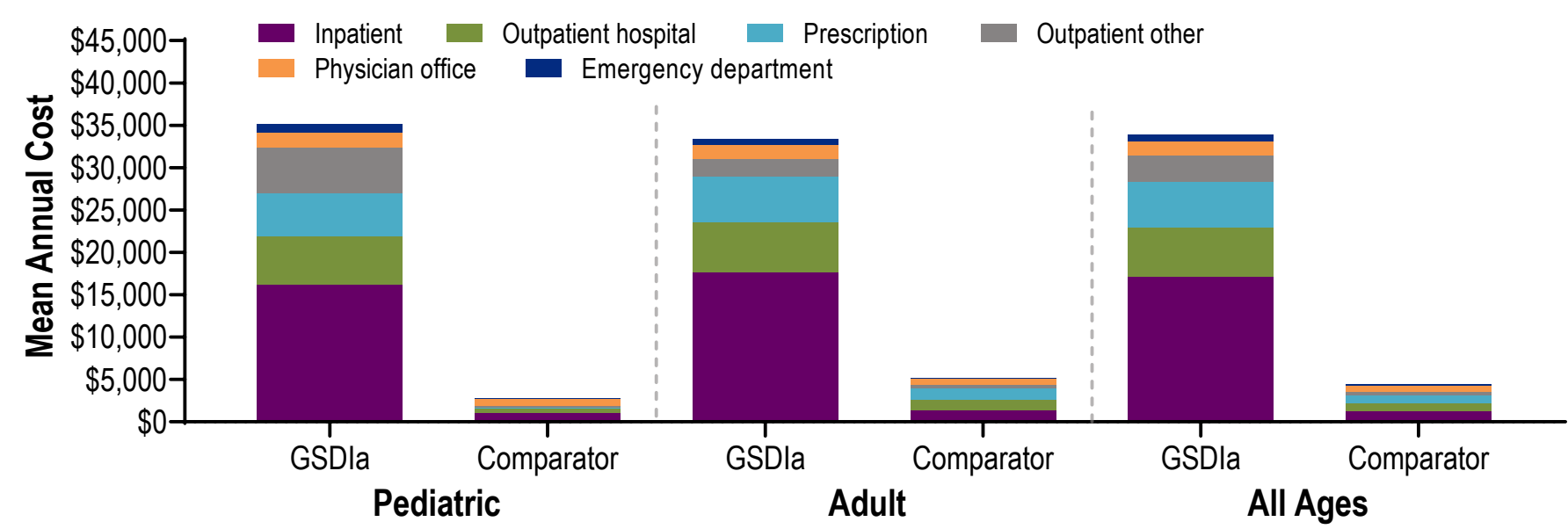
- In total, 557 patients with GSDIa and 5570 comparators were included (**Table 1**)
- Patients with GSDIa versus comparators had:
 - A greater number of hospitalizations: ≥1 (45.8% vs 10.2%) or ≥2 hospitalizations (26.6% vs 2.3%; [**Figure 2**]) over the observation period
 - Higher mean (95% CI) annual hospitalizations: 0.5 (0.4–0.6) vs 0.1 (0.1–0.1) per patient per year (PPPY)
 - Longer mean length-of-stay: 3.1 (2.4–3.8) vs 0.4 (0.2–0.5) days

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



- Patients with GSDIa versus comparators had more annual visits (95% CI) PPPY in the
 - Emergency department: 1.3 (1.1–1.4) vs 0.3 (0.2–0.3)
 - Outpatient setting: 5.9 (5.2–6.6) vs 1.2 (1.1–1.3)
 - Physician office: 10.5 (9.5–11.6) vs 6.0 (5.8–6.2)
 - Other outpatient setting: 9.5 (7.1–11.8) vs 1.5 (1.4–1.7)
 - Pharmacy: 12.1 (9.8–14.3) vs 5.0 (4.7–5.2)

Figure 3. Mean Annual Healthcare Costs Per Patient by Setting of Care



- Patients with GSDIa vs comparators had higher mean (95% CI):
 - Annual overall total healthcare costs of \$33,910 (\$27,630–\$40,200) vs \$4410 (\$3910–\$4910)
 - Costs by setting:
 - Inpatient \$17,100 (\$12,510–\$21,690) vs \$1210 (\$920–\$1490)
 - Outpatient \$5860 (\$4710–\$7000) vs \$1010 (\$840–\$1170)
 - Pharmacy \$5320 (\$3800–\$6830) vs \$910 (\$720–\$1090)
 - Other outpatient \$3160 (\$2220–\$4110) vs \$400 (\$310–\$480)
 - Physician \$1680 (\$1440–\$1910) vs \$750 (\$710–\$780)
 - Emergency department \$800 (\$650–\$940) vs \$150 (\$140–\$160)

LIMITATIONS

- The results of this analysis may be subject to measurement errors, and 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
- Dietary management is not captured in administrative claims data
- Quality of life and mortality were not captured in this study

CONCLUSIONS

- GSDIa is a chronic and progressive disease associated with numerous complications that may be serious and result in extensive resource utilization and high costs vs comparators
 - Average annual overall total healthcare costs are nearly eight-fold higher for patients with GSDIa vs comparators
- GSDIa is associated with a greater number of hospitalizations vs comparators, with nearly twice the average cost per hospitalization for patients with GSDIa
- The results of this analysis demonstrate a large unmet medical need for patients with GSDIa, despite the availability of dietary supplementation practices
- Future studies may assess mortality and quality of life among patients with GSDIa

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DISCLOSURES AND ACKNOWLEDGMENTS

This study was funded by Ultragenyx Pharmaceutical Inc. Andrew Grimm and Leah B. Sansbury are employees and shareholders of Ultragenyx Pharmaceutical Inc. Eliza Kruger and Justin Nedzesky were employees and shareholders of Ultragenyx Pharmaceutical Inc. at the time the study was completed. Yun Guo and Siyi He are employees of Tianjin Happy Life Technology Co., Ltd, Beijing, China. Tianjin Happy Life Technology Co. was paid by Ultragenyx Pharmaceutical Inc. to perform this analysis. Jamie Ziobro of Ultragenyx Pharmaceutical Inc. provided medical writing support in the preparation of this presentation.