Economic Burden of Growth Hormone Deficiency in a US Pediatric Population

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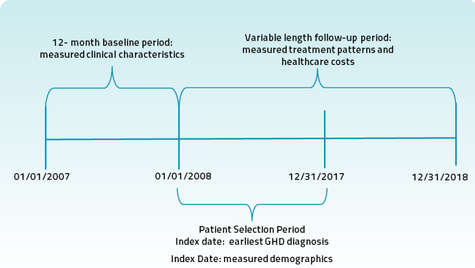
BACKGROUND

Pediatric GHD is a disorder of short stature that is currently treated with daily injections of somatropin.[1] In addition to short stature, GHD is associated with other comorbidities such as impaired musculoskeletal development, cardiovascular disease, and decreased quality of life.[2]

Our objective was to analyze somatropin utilization, adherence, and healthcare costs among children with GHD who had either Medicaid or commercial health insurance.

This study fills the current gap in knowledge of real-world evidence on the clinical and economic burden of pediatric GHD among patients in the U.S.

Figure 1: Patient selection



METHODS

Retrospective observational cohort design within the IBM® MarketScan® Commercial Claims and Encounters Database and the MarketScan Medicaid Multi-State Database.

Children (age <18 years) diagnosed with GHD between Jan. 1, 2008 and Dec. 31, 2017 were matched (1:3) to controls without GHD (or other short stature-related disorder) on age, gender, plan type, region, and race (Medicaid only). Index date was set to the date of the first GHD diagnosis during the selection window for GHD patients and set randomly for controls.

Patients were followed from the 12 months prior to the index date until the end of continuous database enrollment or December 31, 2018.

Comorbidities and medications were measured during the 12 months pre-index. Treatment patterns and healthcare costs were measured during the variable-length follow-up period.

Multivariable modeling compared costs between GHD patients and controls and between somatropin treated and untreated GHD patients.*

RESULTS

A total of 14,070 commercial patients and 6,820 Medicaid with GHD met study inclusion criteria and 68.4% of commercial patients and 63.2% of Medicaid patients were treated with somatropin. Among Medicaid patients, the treatment rate was highest among white males and lowest among black females.

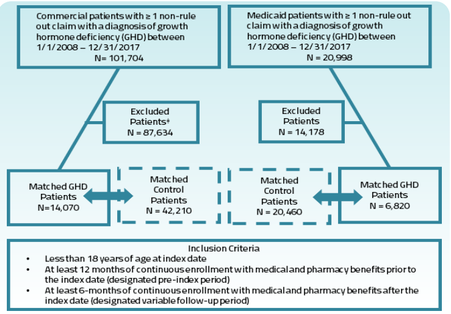
Adherence (proportion of days covered) was low: 32.3% of commercial patients and 18.4% of Medicaid patients were "adherent" (PDC ≥ 0.8). A quarter (24.3%) of commercial patients and nearly half (49.1%) of treated Medicaid patients discontinued somatropin therapy before age 13.

Unadjusted annual all-cause healthcare costs were 16.7 times greater (Δ$37,860) for commercial GHD patients and 10.8 times greater (Δ$33,920) for Medicaid GHD patients compared to controls, including somatropin costs.

Adjusted* all-cause non-somatropin costs were 5.5 times higher (Δ$12,305) for commercial patients and 5.7 times higher* (Δ$19,309) for Medicaid patients than matched non-GHD controls. Adjusted‡ all-cause non-somatropin costs were 31% lower (Δ$7,650) for treated commercial patients and 41% lower (Δ$14,416) for treated Medicaid patients than for untreated patients. Annual all-cause costs were positively associated with GHD related conditions including endocrine-related conditions, pituitary tumors, cardiovascular disease, hepatic conditions, metabolic conditions, anxiety, depression, and sleep disorders.

RESULTS

Figure 2: Final sample and cohorts



	GHD Cohort		Control Patients	
	N/ Mean	%/SD	N/ Mean	%/SD
Commercial	**N =**	**14,070**	**N =**	**42,210**
Age (Mean, SD)	11.1	3.7	11.1	3.7
Male (N, %)	10,006	71.1%	30,018	71.1%
Geographic region (N, %)				
Northeast	3,196	22.7%	9,588	22.7%
North Central	2,994	21.3%	8,982	21.3%
South	5,759	40.9%	17,148	40.6%
West	1,988	14.1%	5,964	14.1%
Unknown	133	1.0%	528	1.3%
EPO/PPO Insurance type (N, %)	8,545	60.7%	25,635	60.7%
Duration of follow-up (Days; Mean, SD)	1,339	986	1,293	959
Charlson Comorbidity Index (Mean, SD)	0.3	0.8	0.1	0.3
Endocrine-related conditions	3,132	22.3%	94	0.2%
Cardiovascular disease	561	4.0%	378	0.9%
Metabolic conditions	775	5.5%	800	1.9%
Mental health conditions	3,191	22.7%	4,040	9.6%
Medicaid	**N =**	**6,820**	**N =**	**20,460**
Age (Mean, SD)	9.5	4.5	9.5	4.5
Male (N, %)	4,497	65.9%	13,491	65.9%
Race/Ethnicity (N,%)				
White	4,212	61.8%	12,636	61.8%
Black	937	13.7%	2,811	13.7%
Hispanic	415	6.1%	1,245	6.1%
Other/Unknown	1,256	18.4%	3,768	18.4%
Comprehensive/POS Insurance type (N, %)	3,197	46.9%	9,591	46.9%
Duration of follow-up (Days; Mean, SD)	1,446	938	1,187	773
Charlson Comorbidity Index (Mean, SD)	0.5	1.0	0.1	0.4
Endocrine-Related conditions	1,511	22.2%	69	0.3%
Cardiovascular disease	482	7.1%	240	1.2%
Metabolic conditions	702	10.3%	1,003	4.9%
Mental health conditions	2,503	36.7%	3,610	17.6%

N=number; SD=standard deviation

Figure 3: Adjusted * annual all-cause healthcare costs for patients with and without GHD (excluding somatropin costs)

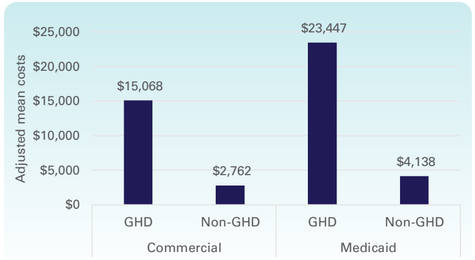


Figure 4: Unadjusted annual all-cause healthcare costs, by service category, for patients with and without GHD setting (including somatropin costs)

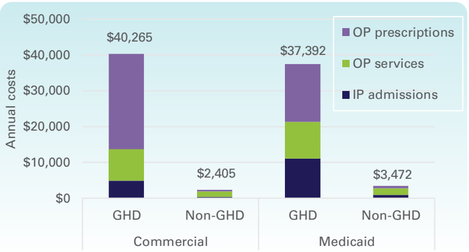


Figure 5: Adjusted ‡ annual all-cause healthcare costs by treated vs. untreated GHD patients (excluding somatropin costs)

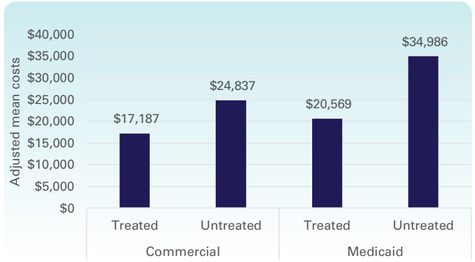
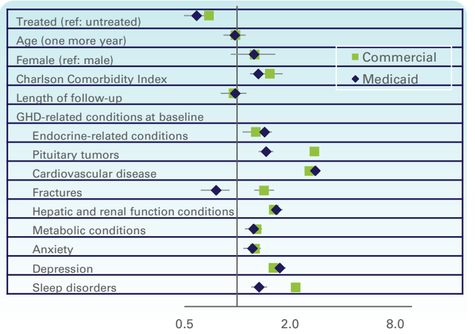


Figure 6: Generalized linear model cost ratios of annual all-cause healthcare costs (excluding somatropin costs), treated vs. untreated GHD patients ‡



SUMMARY

In a retrospective cohort study of claims data, healthcare costs were almost 6 times higher for pediatric patients with GHD compared to controls, controlling for key demographics and clinical characteristics,* in both commercial and Medicaid populations. **GHD-related healthcare costs were 31% and 41% lower among pediatric patients with GHD treated with somatropin compared to untreated pediatric patients with GHD in commercial and Medicaid populations, respectively.**

CONCLUSIONS

Pediatric GHD presents a significant healthcare burden, and many patients remain untreated or undertreated

Untreated GHD was associated with higher non-somatropin healthcare costs than treated GHD. Strategies to improve adherence may reduce the healthcare burden faced by these patients.

*All-cause and GHD-related healthcare cost model comparing GHD patients to controlled patients controlled for age, sex, geographic region (Commercial only), healthcare plans, CCI, index year, length of follow-up, and race/ethnicity (Medicaid model only).
‡ All-cause and GHD-related health cost model comparing treated to non-treated patients among GHD patients controlled for age, sex, geographic region (Commercial only), healthcare plans, CCI, index year, length of follow-up, race/ethnicity (Medicaid model only), and GHD-related conditions measured during the baseline.

References: 1. Stochholm K, Gravholt CH, Laursen T et al. Incidence of somatropin deficiency–a nationwide study. Eur J Endocrinol 2006;155:61-71; 2. Brod M, Alolga SL, Beck JF, Wilkinson L, Højbjerre L, Rasmussen MH. Understanding burden of illness for child growth hormone deficiency. Qual Life Res 2017;26:1673-1686. **Disclosure:** Alden Smith and Pisit Pitukcheewanont are employed by Ascendis Pharma Inc. Janna Manjelievskaia, Lorena Lopez-Gonzalez and Cynthia Drake Morrow are employed by IBM Watson Health which received funding from Ascendis Pharma to conduct this study. Paul Kaplowitz is a paid consultant of Ascendis Pharma.

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