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# COST OF CARE ANALYSIS FOR US PATIENTS WITH PRADER-WILLI SYNDROME (PWS)

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## Summary

- Prader-Willi syndrome (PWS) direct medical costs were evaluated relative to a non-PWS matched cohort across select age groups for comparison
- This analysis yielded results that on average, PWS patients **costs are approximately 10 times higher than non-PWS patients**
- Among all PWS patients, those who take growth hormone experience higher costs than those that do not take growth hormone, due primarily to the associated prescription costs

## Introduction and Objective

**Introduction:** PWS is a rare genetic disorder resulting in endocrine dysregulation with clinical hallmarks of short-stature, developmental delay, cognitive-behavioral problems, and hyperphagia[1,2]; resulting obesity can lead to other complications.[1] Life-long medical care is required but the relative costs associated with these services is not well-defined in currently available literature

**Objective:** This study analyzed direct medical costs of PWS patients and compared these to non-PWS patients; cost variation between those receiving growth hormone (GH) and those not was also evaluated

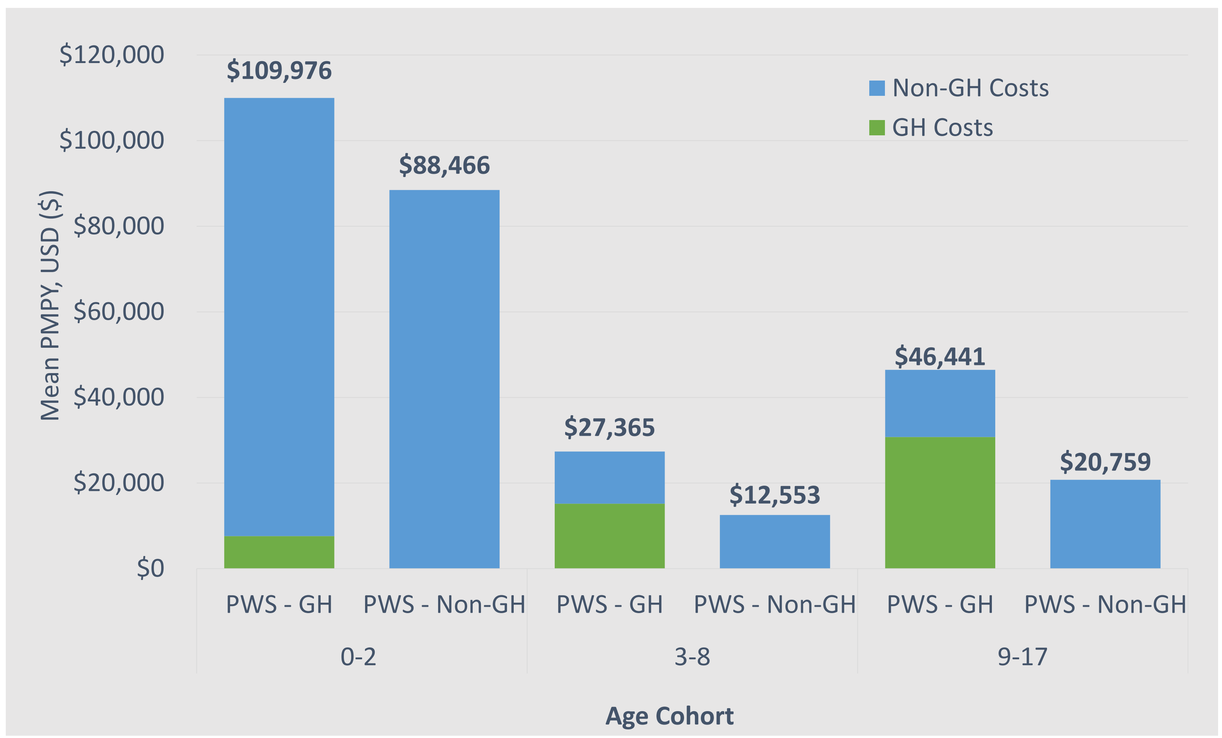
## Methods

- **Design**: Retrospective comparative analysis of medical and pharmacy claims data for commercially-insured beneficiaries with PWS
- **Dataset**: IQVIA™ Health Plan Claims Data for beneficiaries between 1/2006 – 11/2018
- **Cohort eligibility**: All patients were required to have been continuously enrolled for ≥ 12 months post-enrollment
  - PWS: required to have ≥2 ICD-9 diagnosis claims for PWS (759.81); observation period begins from date of enrollment
  - Note: No new PWS patients added after 10/1/2015 due to change in International Classification of Disease (ICD) coding; existing patients were still analyzed
- **Analysis**: Within a given 12-month period, eligible patients' medical and pharmacy claims were identified and allocated into mutually exclusive utilization and spending categories via standard billing code conventions. In additional to total per member per year (PMPY) expenditures, spending categories of interest included: inpatient and outpatient services and prescription drugs; overall PMPY spending was also examined between patients receiving GH and those not receiving GH
  - Dollar values were adjusted for inflation to 2018 dollars using the Personal Consumption Expenditure health component price index
- **Matching:** Controls were matched based on age, sex, length of continuous enrollment (+/- 3 months), and payer type (5 controls : 1 case ratio)

## Results

- 1,621 PWS and 8,105 non-PWS patients were eligible for analysis, results are reported as the mean PMPY costs

**Figure 3. Mean Overall PWS PMPY Cost of GH and Non-GH users**




## Discussion

- Across age groups, direct medical costs of PWS patients were 10 times higher on average than non-PWS patients; differences peaked in infancy and adolescence ranging from 12.1 to 14.7 times higher for PWS patients <18 years of age compared to non-PWS
- The primary cost driver was inpatient care (~48% of PWS costs), followed by outpatient-based and prescription costs (~21% and ~31% of costs, respectively)
- The mean annualized costs per PWS patient per year ranged from $7,960 - $97,673, while costs per non-PWS patient per year ranged from $1,589 - $8,724 across age groups
- Costs were higher among PWS patients taking GH (driven primarily by the cost of the GH itself); PMPY GH cost varies by age group (likely due to dose escalation)
- In older cohorts (i.e., 9-17 years of age) PMPY costs (excluding the cost of GH) are lower for patients receiving GH
- **Study Limitations:**
  - ICD-10 code transition (2015) prevented analysis of most recently incident PWS patients
  - The weighting of the data toward the first 12 months of enrollment biases data toward costs of a younger patient cohort (for both PWS and non-PWS patients)
  - Analysis of GH use does not account for differences in adherence and/or long-term use, only point-in-time exposure

**Figure 1. Total mean PMPY Cost Between Matched PWS vs Non-PWS**

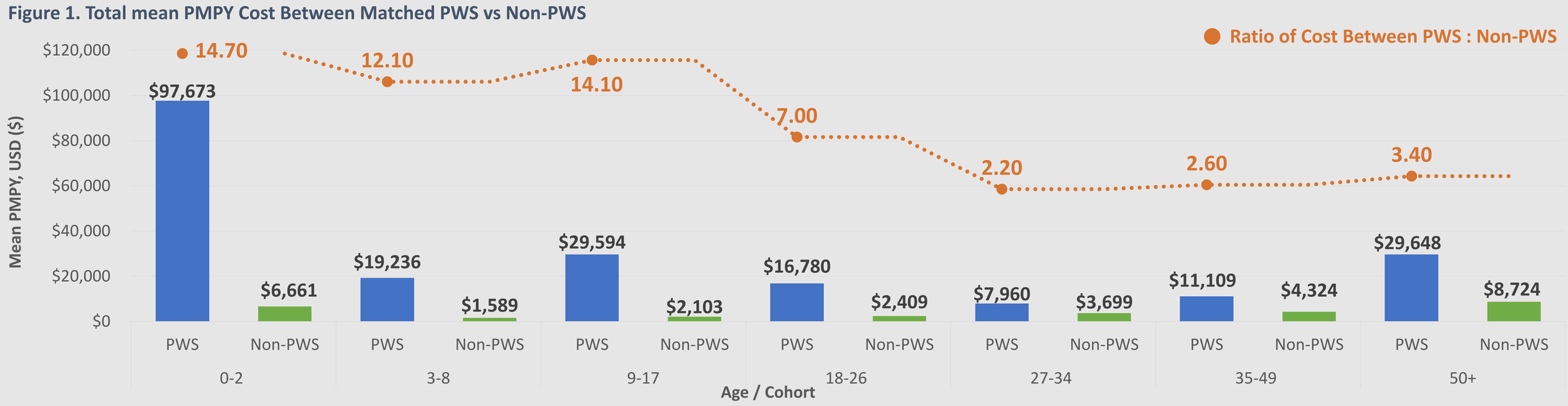



**Table 1. Matched PWS vs Non-PWS Costs**

| Age-group (years): | 0-2 | | 3-8 | | 9-17 | | 18-26 | | 27-34 | | 35-49 | | Over 50 | | All ages | |
|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|
| **Cohort (PWS vs non-PWS)** | **PWS** | **non-PWS** | **PWS** | **non-PWS** | **PWS** | **non-PWS** | **PWS** | **non-PWS** | **PWS** | **non-PWS** | **PWS** | **non-PWS** | **PWS** | **non-PWS** | **PWS** | **non-PWS** |
| Patient count (n) | 278 | 1,390 | 379 | 1,895 | 468 | 2,340 | 197 | 985 | 76 | 380 | 95 | 475 | 128 | 640 | 1,621 | 8,105 |
| Median | $36,116 | $2,385 | $14,989 | $521 | $8,850 | $468 | $5,541 | $640 | $2,903 | $1,034 | $5,303 | $1,232 | $8,142 | $2,983 | $11,311 | $818 |
| **Mean PMPY by care setting** | | | | | | | | | | | | | | | | |
| Inpatient | $69,920 | $3,545 | $1,337 | $212 | $9,935 | $501 | $5,053 | $396 | $2,519 | $1,152 | $4,903 | $667 | $9,936 | $2,353 | $16,976 | $1,129 |
| Outpatient/ Physician office | $21,945 | $2,921 | $9,927 | $1,159 | $7,802 | $1,266 | $7,320 | $1,596 | $3,202 | $1,782 | $4,442 | $2,552 | $16,125 | $4,441 | $10,910 | $1,915 |
| Prescription drug | $5,768 | $190 | $7,938 | $208 | $11,811 | $330 | $4,394 | $397 | $2,231 | $764 | $1,744 | $1,098 | $3,386 | $1,738 | $7,263 | $462 |

## Conclusions and Implications

- PWS patients require **life-long care**, reflected in higher medical costs than non-PWS
- Because infancy and adolescence appear to be **critical periods**, preventative care for PWS sequelae (e.g., obesity complications in adolescents) should be a priority
- Quantitation of **direct medical costs** may stoke awareness of PWS and heighten calls to continue research into **effective disease management**, particularly for patients <18 years old
- Future research should seek to elucidate the **underlying conditions** underpinning these **higher relative costs** and evaluate other economic cost drivers for this population (e.g., group home costs not included in this study)

REFERENCES **1.** Elena G, Bruna C, Benedetta M, Stefania DC, Giuseppe C. Prader-Willi Syndrome: Clinical Aspects. J Obes. 2012;2012:1-13. doi:10.1155/2012/473941 **2.** Butler MG, Kimonis V, Dykens E, et al. Prader–Willi syndrome and early-onset morbid obesity NIH rare disease consortium: A review of natural history study. Am J Med Genet Part A. 2018;176(2):368-375. doi:10.1002/ajmg.a.38582

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