Recombinant Human Growth Hormone Therapy Clinical Outcomes at an Integrated Health System Specialty Pharmacy

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BACKGROUND

Since its initial FDA approval, recombinant human growth hormone (hGH) has gained multiple indications for expanded use, including growth failure secondary to chronic kidney disease, idiopathic short stature, small for gestational age at birth, and Turner Syndrome.[1] Benefits of hGH include not only increased linear growth but also positive impacts on protein and glucose metabolism, bone density, and quality of life.[2] Optimal hGH outcomes depend on timely initiation, however cost and insurance authorization can be barriers to access that delay treatment.[3] Integrated health system specialty pharmacies (HSSPs) associated with pediatric hospitals ensure coordination of care and timely access to hGH, however real-world outcomes for these programs are limited. The objective was to assess the impact of a HSSP program on operational and clinical outcomes for patients on hGH.

METHODS

Design and inclusion criteria: Retrospective, observational analysis of pediatric patients filling hGH prescriptions at the Phoenix Children's specialty pharmacy between July 1, 2023 and November 30, 2025

Inclusion Criteria: Phoenix Children's patients treatment-naïve to hGH, on therapy for up to one year, and enrolled in clinical pharmacy services

Primary Outcome: Time to medication access, defined as the number of days from the date the prescription was written to the first fill

Secondary Outcomes: Percentage of prescriptions requiring prior authorization (PA), PA appeal rate, and change in growth velocity from baseline (prior to hGH initiation) up to 1 year after initiation of therapy for patients on therapy for ≥ 8 months

Analysis: Patients were identified from the HSSP patient management platform. Demographics and growth velocity were obtained through the electronic medical record. Insurance information, fill dates, and medication data were obtained from the HSSP patient management platform.

RESULTS

A total of 76 patients meeting the inclusion criteria were identified; **Table 1** summarizes patient characteristics. The median time to access was 1 day (IQR, 0.75 to 2). The average change in growth velocity from baseline up to 1 year after hGH initiation was 3.7 cm/year for patients included in the analysis (**Figure 2**).Overall, 96% (n=73) of patients required a PA for their initial prescription, and of those, 22% (n=16) required an appeal (**Figure 3**).

Table 1: Patient Characteristics

Characteristic	N=76
Median Age (years)	10
Sex (n, %)	
M	44 (58)
F	32 (42)
Medication (n, %)	
Norditropin	58 (76)
Genotropin	6 (8)
Omnitrope	5 (7)
Sogroya	4 (5)
NGELA	2 (3)
Skytrofa	1 (1)
Diagnosis (n, %)	
Hypopituitarism	43 (56.5)
Short Stature	25 (33)
Turner Syndrome	3 (4)
Other	5 (6.5)
Time on Therapy (days)*	168

*Average

Figure 1: Time to Medication Access

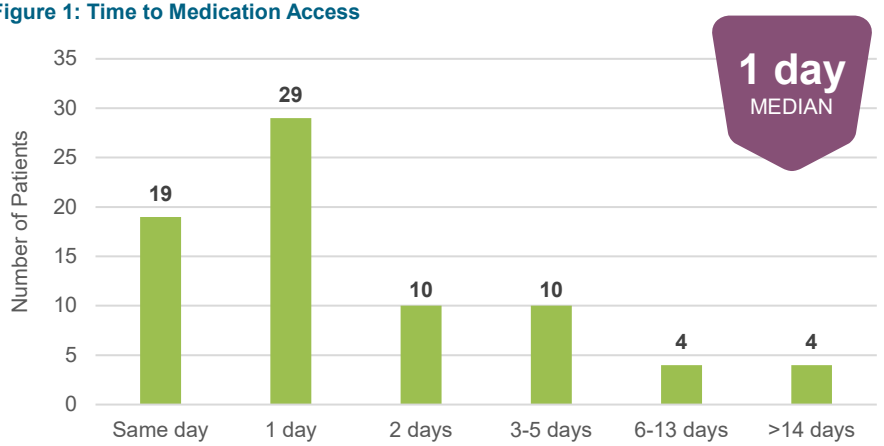


Figure 2: Change in Growth Velocity (n=44)

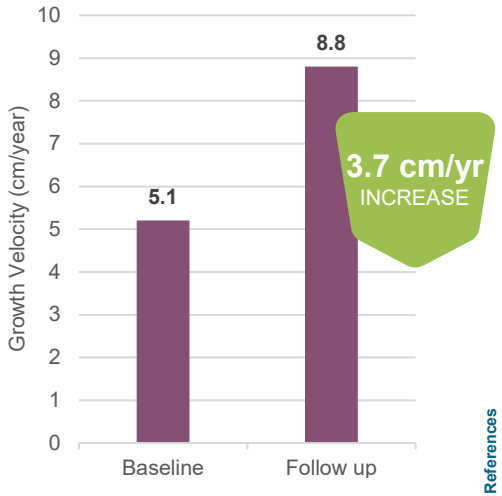
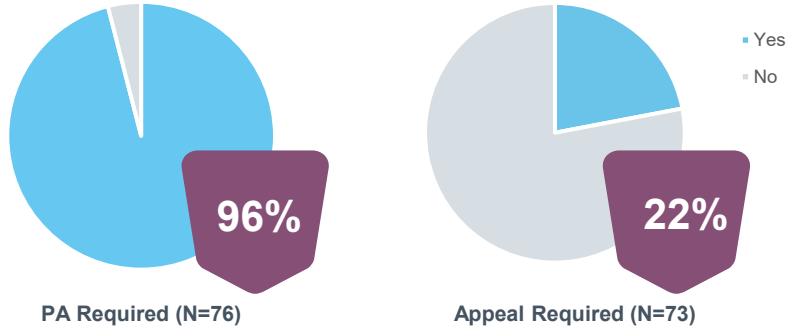


Figure 3: PA and Appeal Requirement



CONCLUSION

This analysis demonstrated positive operational and clinical outcomes among patients receiving hGH and were managed through HSSPs. By coordinating care and minimizing barriers to medication access, HSSPs facilitated timely treatment initiation, with a median time to therapy access of 1 day, comparable to previously reported findings.[3] Additionally, the mean change in growth velocity through 1 year of therapy was comparable to previously published reports,[4] indicating that patients managed through HSSPs experienced growth outcomes consistent with those observed in other hGH-treated populations. Further research is warranted to better understand barriers to hGH access and to evaluate outcomes across different diagnostic populations.

References

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4. Finkelstein B, et al. Effect of Growth Hormone Therapy on Height in Children With Idiopathic Short Stature *Arch Pediatr Adolesc Med* 2002;156:230-240.