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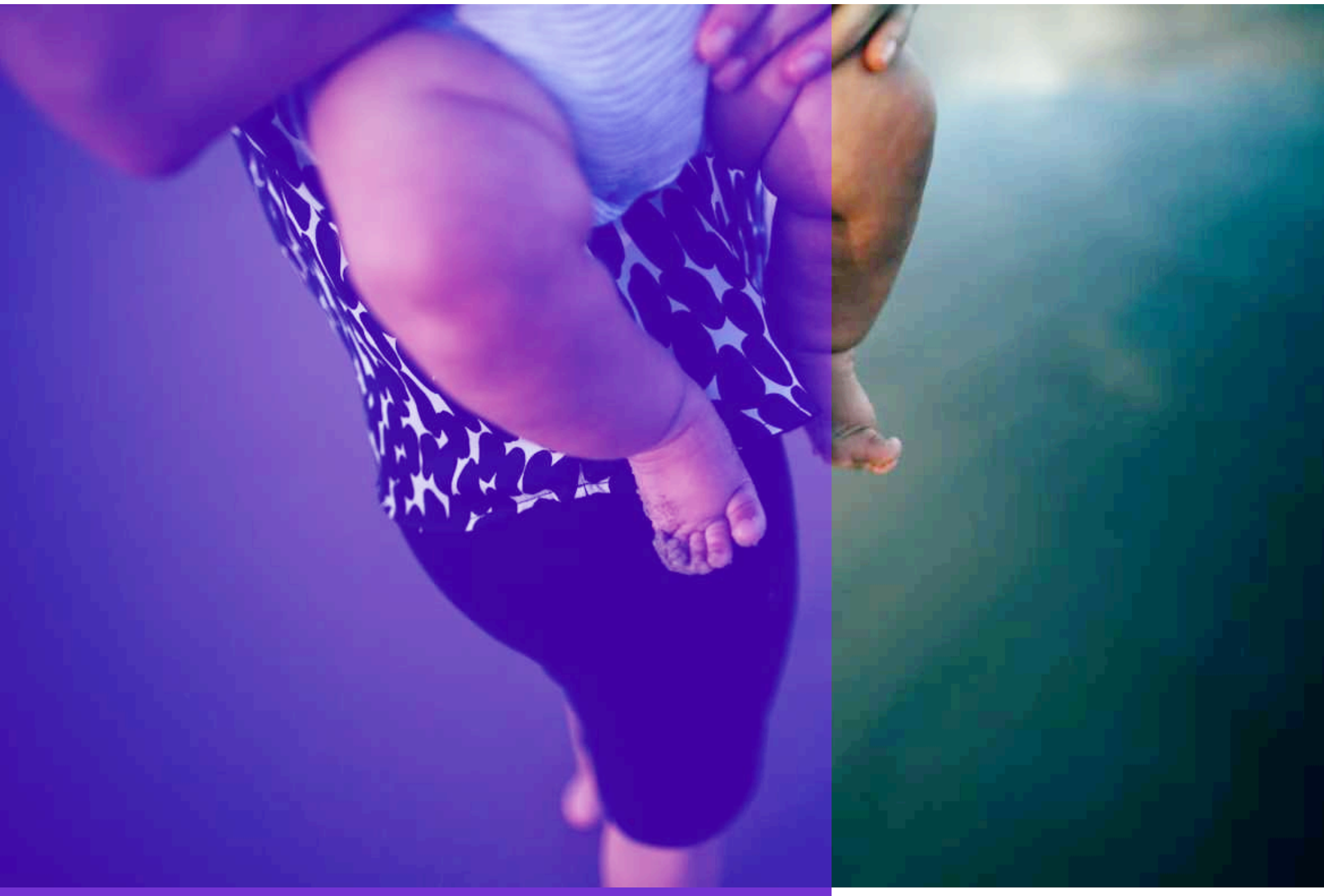

Key Opinion Leader's Expectations for Novel Hyperphagia Therapies in Prader-Willi Syndrome

Executive insights
September 2025

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CONTEXT

The therapeutic landscape in Prader-Willi Syndrome (PWS) is expected to shift toward targeted modulation of neuroendocrine pathways, with recent-to-market and emerging agents like Vykar XR, ARD-101, setmelanotide, and ACP-101 addressing satiety signaling and hypothalamic regulation through distinct mechanisms. The introduction of novel drugs in the United States and Europe is poised to stimulate market expansion and enhance competition in the PWS treatment landscape. To capitalize on this potential, drug marketers and developers in PWS must tailor clinical and commercial approaches to unlock global potential.

INTRODUCTION

Although PWS is considered a “rare” disease, it is one of the most commonly encountered conditions in clinical genetics and is the most common genetic cause of obesity. The hallmark condition of PWS, that is, hyperphagia, is the leading cause of mortality in the PWS population and creates a significant burden for patients and caregivers. Treatments that can effectively manage hyperphagia are one of the key unmet needs in PWS.

The US FDA approval of Soleno Therapeutics’ Vykat XR (a potassium channel activator) in 2025 marked a breakthrough, showing promise in reducing hyperphagia and improving body composition. Interviewed U.S. physicians expressed a high willingness to prescribe this drug to the eligible patients to improve their quality of life. However, its efficacy varies, and long-term safety data are still emerging.

DRUG DEVELOPMENT ACTIVITY

The clinical pipeline in PWS reflects broad mechanistic diversity, with programs modulating oxytocin signaling, hypothalamic hunger circuits, endocannabinoid and histamine pathways, gut- peptide release, and central neurotransmission. Unlike traditional appetite-suppressing treatments (often with limited efficacy and undesirable side effects in PWS), these programs collectively represent a paradigm shift toward biologically tailored interventions for hyperphagia in PWS.

Several pipeline candidates in PWS have shown encouraging safety and efficacy signals across caregiver-reported endpoints such as hyperphagia, irritability, and compulsivity—markers linked to real-world burden and justifying premium pricing and orphan strategies.

KOL INSIGHTS

For a drug targeting hyperphagia in PWS, clinical trial success is typically judged by statistically and clinically meaningful reductions in hyperphagia-related behaviors, often measured using validated tools like the Hyperphagia Questionnaire for Clinical Trials (HQ-CT). However, it becomes important to understand the expectations of key opinion leaders (KOLs) regarding the primary and secondary efficacy-related endpoints and the safety of a novel hyperphagia treatment.

In the second and third quarters of 2025, we conducted interviews with ten key opinion leaders (KOLs) in the United States and the EU5 for our ‘Epi & Markets’ offering related to Prader-Willi Syndrome (PWS). Here, we summarize the key findings regarding the KOLs’ expectations for novel therapies aimed at treating hyperphagia in PWS.

Primary Efficacy Endpoint Expectations

Metric	Acceptable Improvement	Notes
HQ-CT Score Reduction	≥30% from baseline	Seen as clinically meaningful by physicians and caregivers
Placebo-Adjusted Difference	≥2–3 points	Based on prior trials like DCCR Phase 3
Responder Rate	≥40–50% achieving predefined reduction	Indicates real-world benefit in a significant subset

Secondary Endpoints That Would Strengthen Commercial Success:

Caregiver Global Impression of Change (CGI-C): Positive ratings in ≥50% of treated patients	Weight stabilization or reduction: Especially in pediatric cohorts	Reduction in food-seeking behavior: Documented via behavioral logs or caregiver reports	Improved emotional regulation: Often co-occurs with reduced hyperphagia

Safety Endpoints

Metric	Acceptable Improvement	Notes
Adverse Event (AE) Rate	<15–20% for Grade 2+ events	Mild-to-moderate side effects are tolerable if efficacy is strong
Serious Adverse Events (SAEs)	<5% incidence, ideally unrelated to drug	Must be clearly distinguishable from PWS- related baseline risks
Discontinuation Due to AEs	<10%	Higher rates may raise concerns about tolerability
Weight Gain / Metabolic Impact	Neutral or beneficial effect preferred	Drugs should not exacerbate obesity or insulin resistance
Behavioral Aggravation	No increase in aggression, mood instability	Especially important due to emotional dysregulation in PWS

CONCLUSION

Hyperphagia remains an unmet need in PWS due to limited access to approved therapies globally, variable response to medications, lack of long-term data, and continued reliance on restrictive environments. Addressing hyperphagia is critical for physical health, emotional well-being, and independence. Future therapies must offer sustained appetite control, improved metabolic outcomes, and better quality of life for both patients and caregivers. In addition, clinical safety is just as critical as efficacy—especially given the vulnerability of this population to metabolic, behavioral, and neurological complications.

REFERENCES

1. Epi & Markets content: Disease Landscape, Market Sizing and Forecast (2024-2034) in Prader-Willi Syndrome (published in August 2025; to be updated in October 2025)
2. EpiSage content: Detailed epidemiology Insights in Prader- Willi Syndrome (2020-2040) (updated in August 2025)

AUTHOR

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For more insights like these, please contact us for our following 'Market Assessment' report offerings in PWS:

Epi & Markets:

This report provides a comprehensive analysis of the current and evolving disease landscape, including market sizing and forecasts for the United States and EU5 from 2024 to 2034. Supported by expert interviews, epidemiological data, and real-time drug pipeline intelligence, this report equips you with the strategic insights necessary to succeed in the PWS space.

Key Questions Answered:

- What's the size of the prevalent and diagnosed populations, and where are they located?
- What's the current treatment landscape for a disease? What unmet needs and opportunities exist?
- What is the current and forecasted market size? What is the expected market impact of Vykar XR in PWS?
- How will the key pipeline agents shape the future of this therapy market?
- What are the requirements for commercial success?
- What are the market access forces that will drive and influence treatment selection?

Salient features:

Epidemiology-based 10-year forecasts, segmented by drug class, drugs, and country

Customizable and user-friendly market forecast model

Patient funnels, clearly indicating target patient pools

Expert opinions from 15 in-depth interviews to enable decision-making

Utilize industry-leading data sources

Comprehensive market dynamics

Updated quarterly based on regulatory, clinical, and market events

EpiSage:

This report provides a comprehensive analysis of epidemiology for the G8+ markets from 2020 to 2040. Supported by peer-reviewed literature, data from disease registries, real-world insights, primary market research, and validated bottom-up methodology, this report equips you with the much-needed epidemiology insights in PWS to support portfolio, clinical trial, launch, and other critical planning decisions.

Key Questions Answered:

- How can we quickly gauge the potential addressable patients for my asset?
- Of all people with PWS, how many people in each country have been formally diagnosed?
- What are the various drug-treatable patient segments?
- What is the in-depth view of PWS patients with breakout data on age, sex, race/ethnicity, etc?
- How will the prevalent and other populations evolve in the future? What factors will affect the epidemiology over the forecast period?

Salient features:

Global, country, and region-level coverage

Customizable and user-friendly epidemiology forecast model

Patient funnels, clearly indicating target patient pools

Utilize relevant latest data sources

Interactive epidemiology dashboard

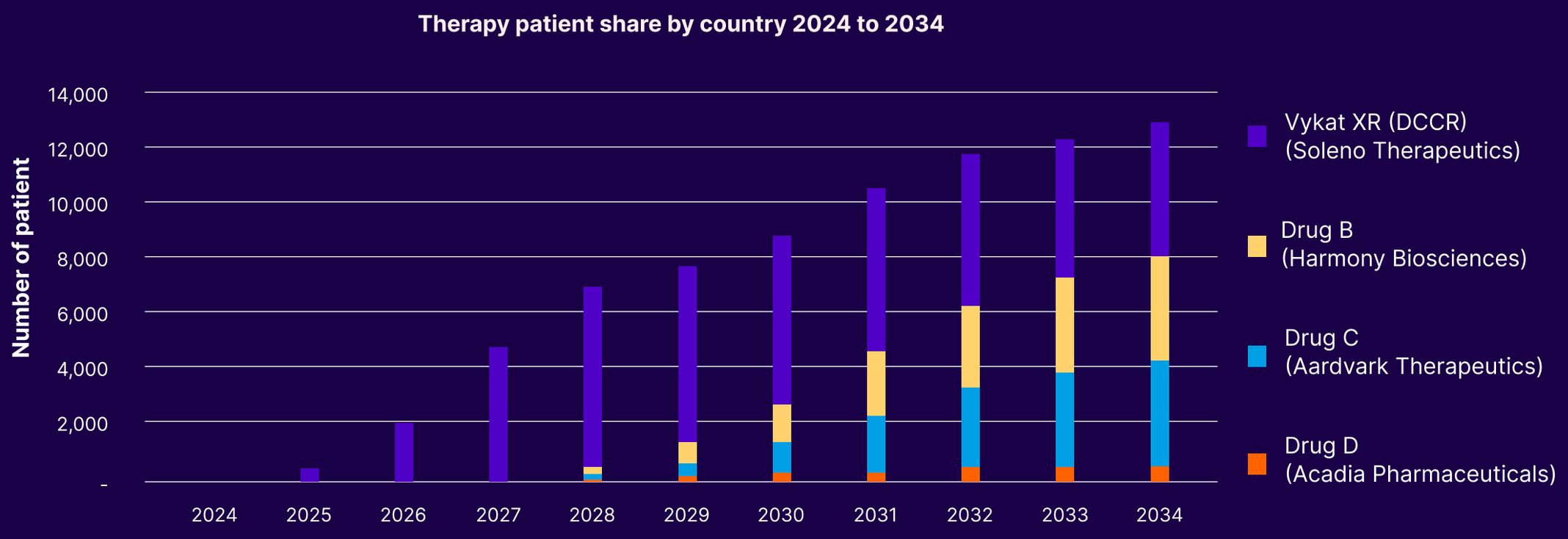
Transparent and validated methodology and assumptions, with proprietary models

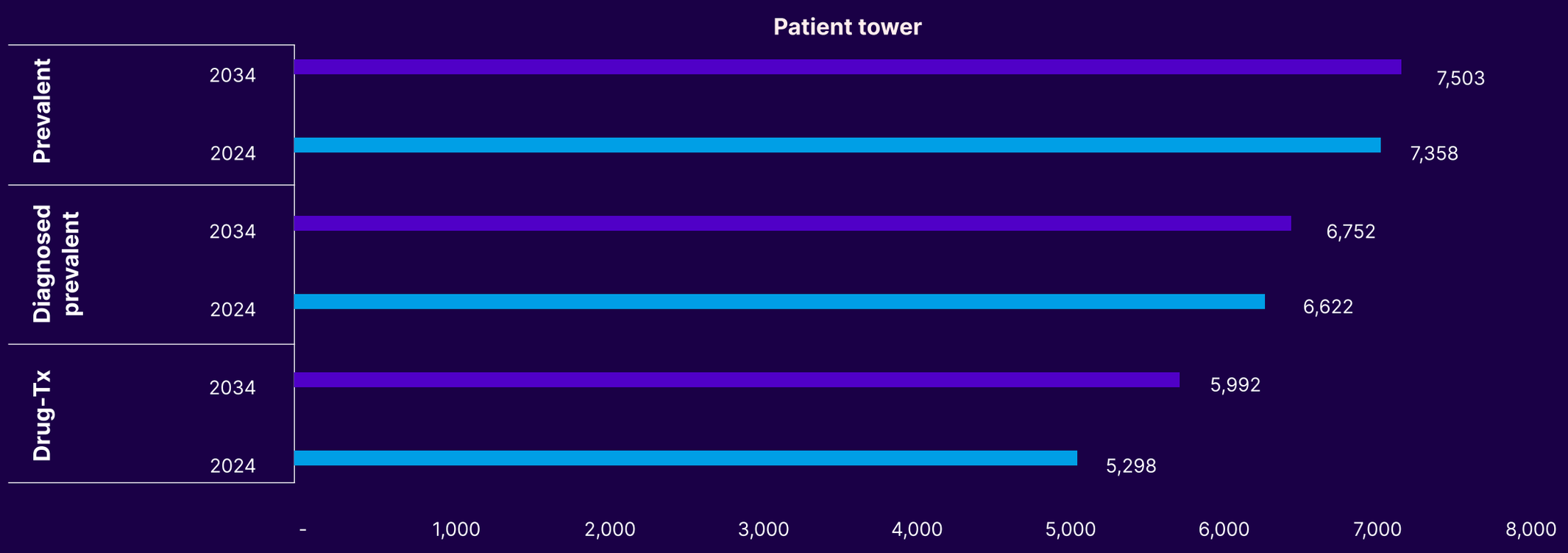
Updated in real-time based on new datasets and market events

About TINA Strategia

TINA stands for Therapy Insights & Analytics. We provide strategic insights and advanced analytics to life sciences and healthcare companies, helping them make informed decisions. Our Market Assessment reports, including ‘Epi & Markets’ and ‘EpiSage,’ are highly valued by our clients for their depth, accuracy, and relevance, among other offerings.

Get the much-needed market insights and epidemiology-based forecast models for PWS.





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Market Assessment | Competitive Intelligence | Primary Market Research | Analytics & Consulting

For any queries over this content and more information on our offerings in Prader-Willi Syndrome Disorder, please contact: healthcare.support@tinastrategia.com