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Acadia pulls plug on Prader-Willi drug after trial flop

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Acadia Pharmaceuticals is scrapping development of intranasal carbetocin after the experimental Prader-Willi syndrome (PWS) drug failed a Phase III trial, sending company shares down nearly 12% on Wednesday.

The decision scuttles Acadia's effort to bring a second treatment option to patients struggling with the rare genetic disorder's hallmark symptom of insatiable hunger. Soleno Therapeutics' Vykat XR (diazoxide choline) became the first drug **approved** by the FDA specifically for hyperphagia in PWS this past March.

Intranasal carbetocin, also known as ACP-101, is a synthetic analogue of oxytocin, a neuropeptide involved in regulating eating behaviour that is deficient in PWS patients.

The 12-week **COMPASS PWS** trial tested a 3.2-mg three-times-daily dose in 175 children and adults ages 5 through 30 years old, measuring changes from baseline in the Hyperphagia Questionnaire for Clinical Trials (HQ-CT) as the main outcome. Acadia reported no statistically significant improvement over placebo on the primary measure, while there was also no separation from placebo on any secondary endpoints.

'Muted' expectations

The outcome dashes hopes raised by an earlier Phase III study, **CARE PWS**, which had hinted at benefit with the same low dose, but not a higher one. Ahead of Wednesday's readout, analysts at Citizens JMP said investor expectations were "muted" for COMPASS PWS given the inverse dose response seen in the previous Phase III trial. Acadia has suggested that the lack of efficacy at the high dose may have been due to activity on vasopressin receptors, which offsets the beneficial activity driven through oxytocin receptors.

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Still, the analysts noted that learnings from that earlier study were used to optimise COMPASS PWS, including focusing solely on the lower dose and extending the duration of treatment from 8 to 12 weeks, in hopes of replicating the efficacy signal. They were also projecting a "highly conservative" \$250,000/year price at launch, compared to the roughly \$500,000 expected average annual cost of Vykat XR.

"Despite this disappointment, Acadia is well-positioned to deliver long-term, sustainable growth



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average annual cost of Vykat XR.

"Despite this disappointment, Acadia is well-positioned to deliver long-term, sustainable growth supported by two approved products projected to generate over \$1 billion in net sales in 2025, and a robust pipeline," remarked CEO Catherine Owen Adams. "Looking ahead, we anticipate seven Phase II or III study starts through 2026 and four data readouts by the end of 2027."

Acadia's two marketed products are Nuplazid (pimavanserin) for Parkinson's disease psychosis and the Rett syndrome therapy Daybue (trofinetide).

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