Prime Therapeutics finds gene therapy treatment for rare spinal muscular atrophy (SMA) disease may significantly impact costs

Integrated medical and pharmacy real-world data is essential to forecast gene therapy potential financial impact

EAGAN, Minn. – March 26, 2019 – Pharmacy benefit manager (PBM) Prime Therapeutics LLC (Prime) forecasts the financial impact of a new gene therapy treatment potentially coming this year for spinal muscular atrophy (SMA). The new treatment for SMA Type 1 may significantly increase payers’ medical drug spend in 2019 and beyond.

SMA is a genetic disease that affects motor nerve cells in the spinal cord, inhibiting the ability to move and breathe. The disease has a high mortality rate and is a leading, genetic cause of death for infants in the U.S.

In December 2016, the Food and Drug Administration (FDA) approved Spinraza® (nusinersen) as the first therapy indicated to treat SMA in pediatric and adult patients. Spinraza launched in January 2017 with an annual first year cost of $700,000. Another treatment for SMA Type 1, Zolgensma® (onasemnogene abeparvovec), is under FDA review and is expected to enter the market later this year. If approved, this one-time gene therapy could cost $4 million to $5 million per treated member.¹

Real-world integrated medical and pharmacy claims data can be used to inform health plans about increased spend.

Prime analyzed pharmacy and medical claims data for its 15 million commercially insured members from January 2017 to August 2018 to identify members being treated for SMA with Spinraza. To evaluate if members stayed on Spinraza, Prime researchers looked at members who were continuously enrolled nine months from their index (earliest) Spinraza claim, and compared claims to prescribing information. To forecast new gene therapy use, Prime used medical claim diagnosis codes to identify members with SMA Type 1 and assumed 50 percent of identified members would receive gene therapy at $4.5 million.¹

Despite being used by less than 1 per 100,000 commercial members, Spinraza contributed to the increasing cost trend from 2017 to 2018. Spinraza total paid was $0.15 per member per month (PMPM) in 2017 and increased to $0.23 PMPM in the first nine months of 2018. Depending on medical claim diagnosis code and age requirements, the new gene therapy is forecasted to contribute $0.21 PMPM to $1.46 PMPM net new spend one year following the gene therapy launch.

“For the currently available SMA treatment, Spinraza, our analysis showed 4 of 10 members received two or more Spinraza doses less than expected based on prescribing information. Therefore, the potential to negotiate an outcome-based contract with the manufacturer may exist to recoup costs
due to lack of members staying on the drug, thus impeding its effectiveness,” said Catherine Starner, PharmD, health outcomes consultant senior principal, Prime Therapeutics. “In addition, the new gene therapy for SMA Type 1, Zolgensma, could significantly increase costs for our health plan clients, especially if the drug receives approval to treat to all SMA types.”

Prime researchers will present this silver ribbon-winning study at the Academy of Managed Care Pharmacy’s (AMCP) Managed Care & Specialty Pharmacy 31st Annual Meeting March 25-28 in San Diego.


About Prime Therapeutics
Prime Therapeutics LLC (Prime) helps people get the medicine they need to feel better and live well. Prime manages pharmacy benefits for health plans, employers, and government programs including Medicare and Medicaid. The company processes claims and offers clinical services for people with complex medical conditions. Prime serves more than 28 million people. It is collectively owned by 18 Blue Cross and Blue Shield Plans, subsidiaries or affiliates of those plans. For more information, visit www.primetherapeutics.com or follow @Prime_PBM on Twitter.

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