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FDA APPROVES WORLD'S MOST EXPENSIVE DRUG, WITH A \$2.125 MILLION PRICE TAG

On May 24, the Food and Drug Administration (FDA) approved Zolgensma®, the first and only gene therapy product available for the treatment of spinal muscular atrophy (SMA). AveXis, a Novartis company, set a \$2.125 million price, the highest of any drug approved to date. SMA is the leading genetic cause of infant mortality in the US, occurring in about 1:10,000 newborns. If left untreated, the most serious form of SMA often results in ventilator dependence or death by age two. Zolgensma is the second FDA approved treatment for SMA, following Spinraza®, which was introduced in late 2016.

Zolgensma's high cost, coupled with its unique mechanism of action and one time only dosing schedule, is raising important questions for state Medicaid programs and contracted Managed Care Organizations (MCOs) as the pharmaceutical industry moves toward the development of more high cost gene therapies.

POLICY AND OPERATIONAL CONSIDERATIONS

Multiple options exist for the incorporation of high cost drugs such as Zolgensma® into a managed care delivery system.

Some states and MCOs may be presented with logistical challenges if claims systems are unable to accommodate seven figure drug prices without significant IT investment and system changes.

Some states may elect to continue including drugs like Zolgensma in managed care capitation rates. Other states may consider employing risk mitigation techniques for this and other high cost drugs, such as the implementation of a risk corridor or a budget neutral risk pool, development of a stop loss program or the creation of a kick payment for plans.

Unlike other recent high profile treatments for diseases such as hepatitis C or cystic fibrosis, which often occur in clusters, treatment for Zolgensma is likely to be distributed across communities and across plans.

Zolgensma is expected to be available through multiple specialty pharmacies. States and MCOs will need to create policy to determine if the drug will be billed and paid for through the pharmacy benefit, the medical benefit, or both. States and MCOs will also need to develop policy regarding how or if reimbursement for the cost of the drug will be addressed if it is administered as part of a neonatal intensive care unit (NICU) or other hospital stay.

States should consider the precedent set by any policy action taken for Zolgensma as it is expected to be the first of many gene therapies introduced to the market over the next several years.

INDICATION AND USE

Zolgensma is a gene therapy indicated for the treatment of pediatric patients less than two years of age with SMA with specific mutations in the survival motor neuron 1 (SMN1) gene. It is not to be used in patients with advanced SMA (complete limb paralysis or permanent ventilator dependence). The vast majority of infants diagnosed with SMA will be eligible for Zolgensma treatment.

The SMN protein is insufficient in SMA due to the mutation of the SMN1 gene, resulting in progressive muscle weakness and atrophy. As a gene therapy, Zolgensma is designed to replace a copy of the gene encoding the human SMN protein with a functional version.

Zolgensma administration requires a single, 60 minute intravenous (IV) infusion. Long-term patient follow up is minimal at this point due to the limited amount of time the product has been available for testing.

However, early evidence suggests that Zolgensma has shown a durable effect of nearly four years in patients with infantile onset SMA, significantly extending patients' expected survival time.

PLACE IN THERAPY

Historically, treatment for SMA has primarily been focused on supportive therapy such as nutrition, respiratory assistance and treatment or prevention of complications related to muscle weakness. If left untreated, the most severe form of SMA typically results in permanent ventilation or death by age two.

On December 23, 2016, the first SMA disease modifying therapy, Spinraza, was approved for both the adult and pediatric SMA populations. With Spinraza treatment, some patients were able to sit unassisted, stand or walk at milestones when they would have otherwise been unexpected to do so. Furthermore, patients were able to survive beyond ages expected for their condition. Because Spinraza's effects are temporary, treatment with the drug must continue in order to prevent disease progression over time.

Zolgensma is the newest and second disease modifying treatment option available. Zolgensma presents the potential for a once and done treatment for this lethal disease, and is expected to replace ongoing Spinraza use for some SMA patients and become the treatment of choice. Unlike Spinraza, which requires a spinal tap for each administration and can only be administered at approved sites, Zolgensma can be administered via a one time infusion. Zolgensma was not studied in conjunction with Spinraza, and is not intended to be used in combination, although it's possible that some patients may use both products.

COST

Zolgensma's list price, based on wholesale acquisition cost (WAC), is \$2,125,000 for a one time IV infusion. Zolgensma is provided as a kit customized to the patient's weight. The kit price is uniform regardless of dose, so the price is the same for all patients.

Zolgensma is currently the most expensive drug ever FDA approved, at \$2,125,000 for the one time dose. AveXis has publicly offered a pay over time model through Accredo, an Express Scripts owned specialty pharmacy. Accredo will give payers the option to pay for Zolgensma on an installment plan over multiple years. The annual cost will then depend on several factors, including the payer's down payment amount, the duration of the pay over time arrangement and contract terms between Accredo and the payer.

At this time, it is not expected that Medicaid agencies or Medicaid MCOs will enter into pay over time arrangements with Accredo due to challenges around the churn of enrollment eligibility within and between MCOs and Medicaid programs.

Despite the high cost of Zolgensma, it has the potential to significantly reduce disability and save significant Medicaid costs over time for patients who would have previously survived only with significant supportive care, including mechanical ventilation.

Zolgensma is an important therapeutic advancement in the treatment of SMA because it addresses the genetic cause of SMA through a one time, potentially curative gene therapy. If the results are long lasting, this therapy may prove to be cost effective for some patients. However, the long term effectiveness remains unknown at this time.

Zolgensma represents a challenge and opportunity case for state Medicaid programs and other payers to begin to address the operational, financial and logistical challenges associated with a new wave of very high cost drugs with strong potential to become life changing or lifesaving treatments.

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