Delivering Proactive Pharmaceutical Cost Management Opportunities: **Zolgensma®**

Gene therapies and multimillion-dollar procedures are increasingly part of the health care cost risk – and increasingly highlighted in national news features that create a stir of uncertainty. The impact won't be lessening any time soon. In fact, in January 2019, the Food and Drug Administration (FDA) announced that it anticipates approving 10 to 20 gene therapies per year by 2025.

Gene therapies are ultimately designed to either create a solution for rare diseases that have no known treatment beyond symptom management or increase the precision of the treatment to prevent the chronic high-cost spend of regimens that have shown little positive outcome overall. However, these "once in a lifetime" opportunities, often touted in the news as potential cures, can come with multimillion-dollar price tags, adverse effects and the potential need for additional therapies and further management, depending on patient response.

Big News and Big Costs

Because of its orphan drug designation, the gene therapy Zolgensma® (onasemnogene abeparvovec-xioi) was FDA-approved May 24, 2019, under the BLA Pathway with its Breakthrough Therapy, Fast Track and Priority Review designations, for the treatment of Spinal Muscular Atrophy (SMA) in patients less than two years of age.

SMA is a debilitating, life-threatening condition characterized by progressive muscle weakness and loss of movement due to muscle atrophy. It is caused by the body's inability to produce enough of a protein called survival motor neuron (SMN). Traditionally there have been few options for treatment beyond symptom management of the disease. Then, in 2016, nusinersen (Spinraza®) was FDA-approved to treat SMA by introducing a molecule that works to increase the body's ability to produce the SMN protein.

Three years later, Zolgensma® was approved to deliver a replacement of the defective or missing primary SMN-1 gene, giving the patient the ability to produce the needed SMN protein.

Currently, the once in a lifetime cost for Zolgensma® only (direct from the manufacturer) is approximately $2,125,000.*

After the receipt of Zolgensma®, the other potential cost-driver is the management of subsequent and severe liver injury.

How May These Costs Be Contained?

The strongest way to help contain the costs associated with multimillion-dollar therapies is to anticipate FDA approval, identify the potential population risk and then be proactive in determining a strategy.

Consideration 1: Contracting Directly with Pharma
The manufacturer offers options such as provider "buy and bill" or billing the health plan directly. Both pathways have strengths, and knowing which strategy is best for a specific client's situation is key. A good Stop Loss carrier will have resources that empower clients with a baseline understanding of what has been observed under both pathways so that all strategies may be considered.

Consideration 2: Being Alert to Notices
There hasn't been an administrator identified that does not require some type of prior authorization or pre-certification for Zolgensma®. Once there is notification of the authorization request, pricing negotiations and strategies are usually implemented simultaneously. If negotiations are not done at the time of notice, there is the potential for billed charges that could include a standard mark-up of approximately three times the cost.

Consideration 3: Payment Strategy/Method
Locking in the price strategy and payment method for the one-time use Zolgensma® is very important. Depending on the group's Specific deductible, locking in the price may be more for the Stop Loss carrier and the client's carrier relationship. Educated Stop Loss carriers will stand beside clients during the process to offer insight and experience. After the direct cost has been contained, facility administration fees and other ancillary or indirect costs may be addressed as well.

Consideration 4: Site of Care
Gene therapies are currently only administered at facilities or hospitals that have been trained and certified by the manufacturer and deemed Centers of Excellence (COEs). Since the COEs have the same credentialing, there is opportunity to evaluate administration fees and other ancillary fees associated with the procedure at each facility. Some may offer fee bundles. If a self-funded group offers an option for domestic medical tourism, patients and clients have an opportunity to select what could be considered the right place, at the right time, at the right cost. Smart Stop Loss carriers actively monitor facilities to determine those that are cost-friendly and those that are billing significantly above average.

There are additional options, in some cases, to finance the cost of the therapy. However, initial review of this approach has determined that it is not likely a desirable choice at this time.

Our Approach

HM Insurance Group (HM) makes cost containment knowledge and practices a top priority in working with our partners and clients. We are available from notice to claim, working to drive cost efficiencies and bringing awareness to vendor services and other opportunities to reduce claim costs. This enables us to help our clients achieve savings on first-dollar claims in addition to helping achieve reductions in billed charges on claims exceeding our clients' deductible levels (Stop Loss claims). We have a case-by-case range of involvement. It may be as simple as a conversation in which we make recommendations for cost containment practices that we believe should be implemented, or it could extend to negotiating the cost of pharmaceuticals on a client's behalf.

To date, HM Insurance Group has supported several Stop Loss and Managed Care Reinsurance clients in the identification of risk for the use of Zolgensma®. HM also provides information to assist in client negotiations specific to the cost of therapy. The ability to negotiate, however, varies case-by-case, as do the results of such negotiation.

About HM Insurance Group

HM Insurance Group (HM) works to protect businesses from the potential financial risk associated with catastrophic health care costs. The company provides reinsurance solutions that address risk situations confronting employers, providers and payers. A recognized leader in Employer Stop Loss, HM also offers Managed Care Reinsurance nationally.

HM Life Insurance Company and HM Life Insurance Company of New York are rated "A" (Excellent) by A.M. Best Company, one of the country's oldest and most respected rating agencies. Through its insurance companies, HM Insurance Group holds insurance licenses in 50 states and the District of Columbia and maintains sales offices across the country.

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*Cost listed is for the gene therapy only at the initial time of FDA approval; it does not include any mark-up by the provider or facility or any additional related costs, nor does it reflect any treatment expansions for the therapy that may have been approved following the initial release or the costs related to that expanded use.