High-Cost Pharmaceuticals & Therapies: New Approvals and the Pipeline to Watch through 2021

August 2020

What's Here? What's Coming?

Anticipation has been high for the last half of 2020 with many new disease management options expected to be approved or on the verge of entering the market. The therapies that everyone's been awaiting have sparked interest, in particular, due to their high costs and potential for battling rare diseases.

Tecartus™, which was approved in July 2020 as a one-time infusion, is the third CAR T-cell therapy approved for non-Hodgkin Lymphoma and addresses a different subtype of this disease called Mantel Cell Lymphoma.[1] Tecartus™ is anticipated to cost the facility $373,000 for the one-time infusion, but ancillary charges may bring the actual total closer to $1,000,000 on the plan year, similar to other CAR T-cell therapies on the market. Therapies such as this can impact financial outcomes for self-funded employers, health plans and provider organizations. To help guard their financial health, HM Insurance Group (HM) delivers Stop Loss and Managed Care Reinsurance protection that emphasizes smart cost containment strategies.

The following provides details about newly approved and upcoming therapies that could have an influence on claim costs.

Recently Approved and Pipeline Therapies			
Drug	**Disease**	**Details/Approval**	**Price***
Uplizna™ (Inebilizumab-cdon)	Neuromyelitis optica spectrum disorder (NMOSD) in adult patients	FDA approved in June 2020	$200,000 - $280,000 PPY indefinitely[2]
Tecartus™ (Brexucabtagene Autoleucel, KTE-X19)	Mantle Cell Lymphoma • Occurs in 1 out of 200,000 people in the US and is more common in men (3:1)[3]	• CAR T-cell therapy • FDA approved in July 2020	Cost to the medical facility is estimated at $373,000 for a one-time infusion
Roctavian™ (valoctocogene roxaparvovec, Valrox, BMN 270)	Severe Hemophilia type A without inhibitors	• Gene therapy • FDA issued a Complete Response Letter (CRL) in August 2020, indicating that this product will be denied based on current information; therefore, it could be at least 12 months before a gene therapy for this disease arrives in the market	Estimated $2 million to $3 million for a one-time dose
Evrysdi™ (risdiplam) *Additional details are available on page 3.*	Spinal Muscular Atrophy (SMA) • As many as 10,000 – 25,000 children and adults in the US are affected[4]	FDA approved in August 2020	Estimated $340,000 PPY under the Pharmacy Benefits
Enspryng™ (Satralizumab)	Neuromyelitis optica spectrum disorder (NMOSD)	• Developed to compete with Soliris® and Uplizna™ • FDA approved in August 2020	Estimated $220,000 for the first year and $190,000 PPY indefinitely

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Recently Approved and Pipeline Therapies			
Drug	**Disease**	**Details/Approval**	**Price***
Monjuvi™ (Tafasitamab)	Monoclonal antibody for Relapse/ Refractory Diffuse Large B-cell Lymphoma (DLBCL) • DLBCL is the most common subtype of non-Hodgkin Lymphoma (NHL). There's more than 18,000 new cases yearly[5]	• Developed to compete with CAR T-cell therapies Yescarta® and Kymriah® • FDA approved in July 2020	Estimated cost to the medical facility is $198,000 in the first year, then $156,000 PPY thereafter indefinitely
Breyanzi™ (Lisocabtagene maraleucel; Liso-cel)	Relapse/Refractory Large B-cell Lymphoma	• CAR T-cell therapy • Expected to be FDA approved November 16, 2020	Estimated cost to the medical facility is $373,000 for a one-time infusion
Kymriah® (tisagenlecleucel)	Follicular Lymphoma (FL)	• CAR T-cell therapy • Third indication for Kymriah® • Approval likely in 2021 via FDA RMAT process	Cost likely to remain at $373,000 for the one-time therapy
Idecabtagene vicleucel ("ide-cel;" bb2121)	Relapsed/refractory Multiple Myeloma (B-cell cancer cells)[6]	• CAR T-cell therapy • On delay from FDA for further details	Cost estimate for a one-time infusion is expected in December 2020
Lumasiran	Primary Hyperoxaluria • Gene mutation resulting in oxalate accumulation in organs • Estimated 1 in 58,000 people worldwide are affected[7]	Expected to be FDA approved December 3, 2020	Estimated at $350,000 to $575,000 PPY
Berotralstat	Hereditary Angioedema	• For attack prevention • Oral therapy • Developed to compete with Cinryze®, Haegarda® and Takhzyro®[8] • Expected to be FDA approved December 3, 2020	Cost to Rx Benefits not known at this time
Viltepso™ (Viltolarsen)	Duchenne Muscular Dystrophy	• Works at the cellular level • Price competitive to Vyondys 53 • FDA approved in August 2020	Cost to the medical facility is approximately $750,000 PPY for a 66 lb. child

**Prices are estimates and subject to change; the prices listed do not include any ancillary charges or additional costs that may be incurred when therapy is in use.*

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Treatment Options Continue to Grow for Spinal Muscular Atrophy (SMA)

Three extremely high-cost treatment options are now available for the treatment of Spinal Muscular Atrophy (SMA): Spinraza®, Zolgensma® and the latest, Evrysdi™. All were designed to slow the progression of this disease. Currently, these therapies are being studied in combination use for patients with SMA type 1.[9] While no adverse effects have been noted related specifically to the drug therapy, more research is needed to prove that this combination is safe and effective.[3] Knowing that this possible combination therapy may be used helps to optimize billing strategies so that costs can be anticipated and contained.

Evrysdi™ (Risdiplam), which was recently approved by the FDA as a new oral therapy for SMA, touts a less invasive formulation than Spinraza® and Zolgensma®, and it doesn't require hospitalization for administration. In the JEWELFISH trial, Risdiplam was studied in patients who had Spinraza or other SMA therapies concurrently within a specific time frame.[10] These patients had a sustained increase in SMN protein (a marker for drug efficacy) meaning that it may offer the additional benefit of Spinraza.[11] Long-term data will need to be gathered following the JEWELFISH trial to confirm risks and benefits. Evrysdi™ is reported to cost an additional $340,000 on top of the costs for Spinraza® and Zolgensma® per patient per year, keeping in mind that Zolgensma® is currently a one-time only gene therapy.

About HM Insurance Group

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For more information, please contact **HMPharmacyServices@hmig.com**

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