**Market Insight Gene and Immuno-Therapy**

Current Landscape: 6/10/2019

There are four gene therapies currently approved by the FDA. This landscape is constantly changing and therefore this will be a fluid document with changes made as more therapies are approved or if the indications and cost change.

Two of the recently approved treatments are for blood cancers. The other two therapies are for rare conditions related to blindness and spinal muscular atrophy.

**CAR-T:**

**Kymriah** for pediatric acute lymphoblastic leukemia (ALL), a rare blood cancer and for adults with the most common form of non-Hodgkin lymphoma (NHL). The one-time drug cost for Kymriah is anticipated to be $475,000 however there will be additional costs for the infusion facility, the stay after the infusion, and any possible complications.

**Yescarta** for adults with large B-cell lymphoma that has failed conventional treatment. The anticipated one-time drug cost for Yescarta is approximately $373,000 again, with additional facility, follow up, and complication expenses.

There are select approved centers that are authorized to perform the procedures for Kymriah and Yescarta CAR-T Gene Therapy.

**Luxturna** for inherited pediatric (under 12 months of age) retinal disease (i.e., Leber congenital amaurosis and retinitis pigmentosa). This drug is delivered as a one-time injection into the eye. The cost of the drug is $850,000 or $425 per eye plus the cost of the facility, follow up, and any complications. LUXTURNA is only administered at Ocular Gene Therapy Treatment Centers (Only 10 centers across US).

**Zolgensma** is the drug therapy for a rare conditional of SMA (Spinal Muscular Atrophy). There are four types of SMA and Zolgensma is only approved for one type. The anticipated cost is over $2,000,000. These are patients that were likely taking Spinraza previously which has an ongoing cost of $750 first year and $375 per year thereafter. If the Zolgensma treatment is successful, then theoretically Spinraza is no longer needed; however, if the treatment is unsuccessful, both Zolgensma and Spinraza may be utilized.

Because of the high cost of Zolgensma, the manufacturer, payors, and providers are trying to formulate a financial plan that would allow for payment over time ($425K over 5 years) and for some level of rebate if the procedure is not effective. This will create other questions for payment and reimbursement should the member change health plans. There are currently 29 sites that are approved to provide the gene therapy.
http://www.curesma.org/zolgensma-administration-sites.html

**Pipeline:**

The pipeline currently has over 800 clinical trials underway for gene therapies. The FDA has fast-tracked a path for gene therapies for patients with Hemophilia B (rare) and Hemophilia A (more common). The cost is estimated at >$1M. If the therapy is not effective, Factor products may still be utilized.

Please contact Jakki Lynch, Director of Cost Containment directly at (415) 360-5197 or by email jlynch@sequoiaris.com to discuss our specialty pharmacy cost management strategies