The future is now: Are payers ready for gene therapies?

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Gene therapies for rare diseases may offer a life-changing treatment option for some patients, but can payers keep up with the cost?

Modifying genetics to treat diseases may seem futuristic, but the reality is that the clinical development of gene therapies is making significant progress. As of December 2016, there are 802 gene therapy clinical trials worldwide with 66 in Phase III.[1] Gene therapy may be especially promising for patients diagnosed with rare diseases because an estimated 80% of rare diseases are genetic in nature.[2]

A disease is considered to be rare if there are fewer than 200,000 Americans diagnosed (approximately 0.06% of the U.S. population).[3] In aggregate, there are over 7,000 rare diseases, which affect nearly one in 10 Americans.[4] Historically, many of these diseases have lacked adequate treatments. For patients diagnosed with rare genetic diseases, gene therapies may offer a life-changing treatment option, and in some cases, a cure.

Health insurance providers and other payers may not be aware of the number of gene therapy treatments that could enter the market in the next decade. Treating patients with gene therapies could avoid years of medical and drug expenses for both patients and payers. However, because of the anticipated value that gene therapies may provide patients, the cost of development, and the low number of patients diagnosed with rare diseases, these treatments are expected to be costly.

Many gene therapies are administered at a single point in time with an injection or other procedure. If the therapy is effective, the resulting benefits (and any offsetting cost) would be realized in the years following treatment. Because of the up-front expense, gene therapies may be disruptive to the healthcare system using the current payment models available in the United States. Most payment models in the U.S. health insurance market are based on a single year of expenses, measured by per member per month (PMPM) costs. The launch of one or two gene therapies for rare diseases may be manageable for a payer without overhauling current payment structures, but expenses could quickly overwhelm annual budgets as more enter the market.

Figure 1 displays illustrative incremental PMPM costs for gene therapy assuming different treatment rates and a one-time cost ranging from $50,000 to $2 million.

FIGURE 1: PMPM COSTS FOR ONE-TIME GENE THERAPY TREATMENT

	SAMPLE COSTS OF GENE THERAPY			
TREATMENT RATE	$50,000	$500,000	$1,000,000	$2,000,000
0.01%	$0.42	$4.17	$8.33	$16.67
0.02%	$0.83	$8.33	$16.67	$33.33
0.03%	$1.25	$12.50	$25.00	$50.00
0.04%	$1.67	$16.67	$33.33	$66.67
0.05%	$2.08	$20.83	$41.67	$83.33
0.06%	$2.50	$25.00	$50.00	$100.00
0.07%	$2.92	$29.17	$58.33	$116.67
0.08%	$3.33	$33.33	$66.67	$133.33
0.09%	$3.75	$37.50	$75.00	$150.00
0.10%	$4.17	$41.67	$83.33	$166.67

For example, assume gene therapies, which are priced at $1 million each, are available for diseases affecting 0.10% of your population. Realistically, in any given year, the treatment rate would be lower than the prevalence rate because some patients would not be indicated for treatment or may not elect to receive treatment. In our example, if 30% of eligible members are treated, this would result in an increase of $25 PMPM. For comparison, the Express Scripts 2015 Drug Trend Report estimated the innovative Hepatitis C drugs to cost $3.20 PMPM in the commercial channel.[5]

Gene therapies may be costly for payers using the current payment models in the U.S. healthcare system. Other financing, pooling techniques, and payment models must be discussed and vetted to make it possible for payers to cover the cost of gene therapy, and thereby allow patients to access these therapies.

1 Alliance for Regenerative Medicine. Clinical Trials and Products. Retrieved January 20, 2017, from http://alliancerm.org/page/clinical-trials-products.

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3 U.S. Food and Drug Administration (July 18, 2013). Orphan Drug Act. Retrieved January 20, 2017, from http://www.fda.gov/RegulatoryInformation/Legislation/SignificantAmendmentstotheFDCAct/OrphanDrugAct/default.htm.

4 Office of Rare Disease Research (August 11, 2016). FAQs About Rare Diseases. National Institutes of Health. Retrieved January 20, 2017, from https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases.

5 Express Scripts (2016). 2015 Drug Trend Report. Retrieved January 20, 2017, from https://lab.express-scripts.com/lab/drug-trend-report.

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