EVERSANA™

HOW DO WE PAY FOR A CURE?

How to Put a Price on Life-changing Treatments

The global cell and gene therapy market is expected to reach $13.8 billion by 2026, expanding at a compound annual growth rate of 12.4%.

— *Global Cell & Gene Therapy Manufacturing Services Market by Type - Forecast to 2026 Report*

With over 2,000 ongoing clinical trials in regenerative medicine, patients across the world will soon have even greater access to a large number of life-changing therapies for a variety of diseases. In its 2022 state of the industry report, the Alliance for Regenerative Medicine (ARM) expects at least seven cell and gene therapy approvals in Europe by 2023. A handful of curative therapies have already begun to come to market, offering hope for many patients with diseases such as spinal muscular atrophy (SMA); aggressive blood cancer; or Leber's congenital amaurosis, a rare inherited eye condition. These frontrunners have signaled the evolution of healthcare. However, the innovative nature of these products means they have challenged global healthcare systems.

Curative therapies, specifically **cell and gene therapies**, bear high upfront costs, ranging anywhere from several thousands to over 1 million euros or dollars. Health technology assessment (HTA) agencies set the valuation of these drugs against willingness-to-pay thresholds. Although long-term data is limited for these novel therapies, the fact that **they promise a cure, which can offset future spending on treating a disease over time**, is pertinent when examining the drug's impact on both patients and healthcare budgets. As such, it is crucial to figure out not only *how* to pay for a cure but also *who* is going to pay for it.

FIGURE 1 *- PRICING CURES: FACTORS TO CONSIDER*

EVERSANA™

How to Competitively Price a Complex, Life-changing Product

Specifically, when setting the price for a cell and gene therapy, pharmaceutical manufacturers must also consider:

- The risk of recurrence/relapse.
- Differences in efficacy among cures.
- Comparators, including full life-cycle costs of a disease and the burden of health economic costs on budgets.

Ultimately, this is the question:

How can manufacturers meet critical stakeholder needs while ensuring both patient affordability and a return on their investment?

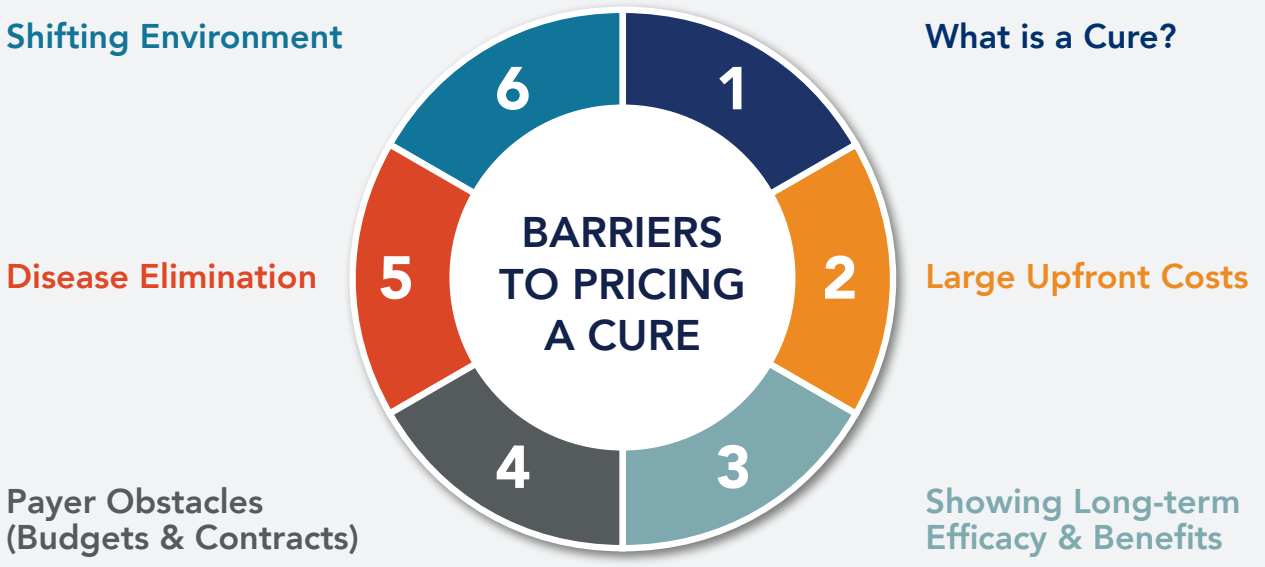
Limiting Risk for Payers

Think about this: How would a payer value a cure with 90% efficacy and an outcome guarantee versus a therapy with 100% efficacy and no outcome guarantee?

Ideally, curative therapies would result in a 100% disease-free outcome, with no chance of disease recurrence and no need for follow-up therapy. While a recent study has confirmed CAR T-cell therapy delivers on its promise of curing certain blood cancers, there is still the chance that the most effective curative therapies might not work with such efficacy, potentially leading to relapse or disease recurrence and requiring follow-up therapy.

Current outcomes-based payment schemes for gene and curative therapies show that national health systems in Europe are opting to pay for these drugs over time — the payments, of course, contingent on the drug's efficacy. By entering such contracts with drug manufacturers, **health systems ensure patient access despite the uncertainties and high costs of these curative treatments**.

***FIGURE 2** - PRICING CURES: FACTORS TO CONSIDER*



As more gene and curative products enter the market, manufacturers must quell uncertainties over the lack of long-term efficacy data as well as cost concerns by designing **innovative payment models** that will be attractive to health systems facing increased budgetary pressures. This includes outcomes- and performance-based schemes, which have gained momentum across Europe. However, manufacturers must be cautious, as such models could leave them vulnerable to "known unknowns," including whether a cure can be sustained throughout a patient's lifetime, given the lack of long-term data. **Nevertheless, these models serve as the first step in rethinking how the industry and payers can work together to mitigate risk and finance cures while ensuring patient access**.

Types of Innovative Payment Models

Increasingly, payers are becoming resistant to making upfront, one-time payments at the time of treatment, which delays access and reimbursement. Given these considerations, both payers and manufacturers are exploring innovative contracts in which healthcare systems pay (or not) over time through models that use rebates, outcomes guarantees and other mechanisms. A few examples of these models include the following:

- **Amortization models,** which involve payment over time. In fact, many payers prefer to engage in long-term contracts to mitigate their risk.
- **Outcomes-based payment schemes:** Under this agreement, health funds will reclaim part of the treatment cost if a patient dies.
- **Risk-sharing agreement:** Half of the cost is paid upfront, and the remainder is paid 18 months after treatment, but only if the therapy is effective.
- **The "payment at results" model,** under which the health fund pays the manufacturer at 6 months and 12 months.
- **Subscription or flat pricing,** which involves flat annual payments (with or without amortization for unlimited use) for an unrestricted supply of a curative medicine.

While more payers are opting for outcomes-based arrangements that shift some of the risk to the manufacturers, the models do have at least one major drawback: They require tracking outcomes over several years, which can place a financial and administrative burden on healthcare systems.

To ensure the success of these arrangements, stakeholders can coordinate in several ways. Specifically, they can:

- Develop patient hubs and support programs to improve adherence to therapy.
- Create robust patient registries that track how patients respond to therapy.
- Collaborate on longer-term outcomes studies to track a drug's efficacy.

With payers willing to enter innovative payment models, such as outcomes-based contracts, manufacturers have a lot to keep in mind when setting prices for their gene and curative therapies. **By devising a collaborative strategy with payers, they can mitigate the impact of uncertainty, ensure an optimal launch and help patients globally receive the curative treatments they need to live longer, healthier lives.**