Matthew Majewski, vice president at Charles River Associates’ Life Sciences Practice, considers the need for new pricing models in an Expert View column.

As pharma and life sciences companies continue to make progress in the development of many potentially curative therapies including cell and gene treatments, stakeholder groups including manufacturers, patient advocates and payers are working to identify the new pricing models that will be necessary to support patient access. Most experts agree that current reimbursement models will need to be reconfigured to accommodate these therapies, and that the clock is ticking.

High-cost curative therapies introduce many new considerations related to pricing and reimbursement. Many will have narrow treatment windows and small patient populations that could restrict the opportunity for manufacturers to maximize commercial success. There may be significant costs for hospital procedures and protocols associated with administration of these therapies. In addition, the introduction of many new high-cost therapies in a condensed period could lead to a major spike in overall healthcare costs.

The payers’ view

CRA recently reached out to a range of medical and pharmacy directors in the payer community for their perspectives on the impact of high-cost curative therapies. These discussions identified several rapidly emerging concerns:

- The impact of these therapies on healthcare costs tend to be “front loaded,” with the highest impact felt in the first year following launch. Costs will typically diminish over time as appropriate patients complete treatment
- Manufacturers may be unable to provide the level of long-term safety and efficacy data needed to position payers to assess the cost effectiveness of a curative therapy over many years
- The potential budget impact will likely require entirely new and previously untried payment models

The emerging pricing models for curative therapies

Despite these challenges, payers and other industry insiders have outlined several innovative pricing models for curative therapies that are now being widely considered. These include approaches involving annuity payments, payments based on pre-determined outcomes, and expanded risk pools.
Annuity payment models

The most popular model under consideration involves a series of payments to manufacturers over a set time period. In this way, the cost of a therapy would be spread out in installments over many months or years, reducing the burden of high up-front payments. On the downside, with this model payers might continue to be responsible for costs for patients even after they transfer to another insurance plan -- essentially representing a transfer of benefit to a competitor. Annuity payments are also likely prohibited by state and federal budget rules. This approach would also require new and potentially costly and cumbersome levels of record keeping and reporting for government and manufacturers alike.

Payments based on outcomes

Among the few curative therapies introduced thus far, Kymriah (tisagenlecleucel) and Luxturna (voretigene neparvovec) were launched using outcomes-based models where treatment must meet pre-determined clinical targets at pre-defined time periods to earn reimbursement. If the endpoints are not met, the product is not reimbursed by these payers. This can help reduce the risk of payment for unsuccessful treatments and can help address the lack of available long-term data -- though payers may still be burdened with related hospital and other administration costs. The effort to get all stakeholders to agree on what constitutes a successful outcome and to centralize outcomes data management and reporting could also be major challenges.

Expanding risk-pools

Under this model, commercial payers would join with other stakeholders including government in risk pools designed to help keep premiums and cost sharing at more manageable levels. But payers anticipate this model will face challenges in achieving the necessary levels of broad alignment among US payers to make this model commercially viable. With a small number of approved products and limited efficacy data for most curative therapies, reliable cost estimates may also be difficult to develop.

The next steps

While opinions about pricing for curative therapies continue to diverge, payers generally agree that there will be no simple solution. The right approaches could include elements from each of the models outlined above and require entirely new levels of collaboration among manufacturers, payers, government and other stakeholders. The pace of innovation means that the need for one or multiple workable pricing models could quickly become a critical issue in healthcare soon.