MARWOOD GROUP

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INNOVATIVE PAYOR CONTRACTING FOR DURABLE GENE THERAPIES; STAKEHOLDER CONSIDERATIONS

In recent years, gene therapy technology has evolved from offering modest effects in pilot trials to producing measurable and durable benefits in the clinic. While treatment for chronic diseases has previously focused largely on maintenance of palliation through routine dosing, one can envision a day when limited-dose curative therapies, or "durable therapies," will become a mainstay of treatment for many chronic diseases. In fact, according to PhRMA, the number of cell and gene therapies held in the US pipeline alone has increased 25% in the last year to 362 Phase I-III clinical trial candidates. Durability of these treatments encompassing significant multiyear benefits will raise issues surrounding pricing of the limited or even single dose.

STAKEHOLDER CONSIDERATIONS

Payor Considerations

Payors face challenges to adopting innovative contracting arrangements that vary based on the number of lives covered, the financial strength of their balance sheets, and the regulations that govern their operations. These predominantly include:

- Actuarial Risk
- Patient Portability
- Structural Challenges to Multi-Year, Multi-State Arrangements (Medicaid)

Manufacturer Considerations

The varying financial capacity, administrative capabilities, and risk appetite of pharmaceutical developers may influence which innovative contracting scheme in which they choose to engage. Manufacturers should keep in mind:

- Revenue Timing
- Administrative Challenges
- Rigid Federal Reimbursement Structures
  - Medical Best Price (MBP) Reporting Rules
  - Federal and State Anti-Kickback Laws

Provider Considerations

Limited-dose durable gene therapies pose potential issues for providers, such as new accreditation requirements for administering the therapies and financial risks from inadequate reimbursement for ancillary medical services. Providers require innovative solutions that engage:

- Centers of Excellence
- Specialty Pharmacy

Patient Considerations

Patient choice is influenced by direct healthcare out-of-pocket costs, including co-payments, coinsurance, deductibles, and high annual cost sharing limits. The Marwood Group posits that their biggest concerns include:

- Out-of-Pocket Costs
- Limitations to Patient Support Programs, Particularly Among Medicare Benefit Recipients

Limited-dose durable gene therapies present an immense challenge when it comes to payor contracting. A number of innovative contracting mechanisms from traditional pharmaceutical pricing are consequently being explored, combined, and adapted to bridge this divide, while other mechanisms are being developed *de novo*. The Marwood Group recently published *INNOVATIVE PAYOR CONTRACTING CONSIDERATIONS FOR DURABLE GENE THERAPIES*. This in-depth white paper covers payor, manufacturer, provider, and patient pain points in the implementation of innovative contracting schemes, as well as the current and emerging contracting mechanisms being adapted and developed to address the unique challenges inherent to limited-dose durable gene therapies.

CONCLUSION

Limited-dose durable gene therapies will become the mainstay of treatment for many diseases. A growing array of value-based contracts are emerging to bridge the gap between cost and value. Due diligence of these therapies and potential ramifications of price, population size, and contracting structures will require not only strategic analysis, but future-focused regulatory awareness as well. As a leading healthcare-focused advisory firm, the Marwood Group advises biopharma, diagnostics, and device companies and healthcare investors in conducting market diligence, developing market access strategies, and managing product life cycles, leveraging our insight into Federal and state policy, financial markets, and the intra-institutional dynamics of the health care sector.

About the Author

Mark Slomiany PhD MBA MPA is a Senior Vice President of Advisory at The Marwood Group and a former faculty member of the Department of Cardiothoracic Surgery at New York University Langone Health, as well as former research associate at the Mossavar-Rahmani Center for Business and Government at the Harvard Kennedy School of Government.

Marwood Group is a healthcare advisory firm offering strategic consulting services, with expertise in the U.S., E.U., and Asian markets. Marwood advises investors and life sciences companies on growth strategies and new business models, product lifecycle management, commercialization, market access and pricing strategies, and legislative and regulatory concerns.

Contact Information: For more information on the content in this publication or to learn more about Marwood Group Advisory's capabilities, we encourage you to contact us:

Nayan Ghosh, Vice President, Life Sciences
Office: 212-532-3651
nghosh@marwoodgroup.com

Lee Alvarez, Senior Managing Director
Office: 212-532-3651
lalvarez@marwoodgroup.com