

# *A HEOR-centric Strategy to Gene Therapy Development*



Gene therapies have proven to be treatments for monogenic conditions, however, often are accompanied by a substantial price tag that payers are forced to accommodate. Complex and variable requirements exist based on the country and the respective health technology assessment (HTA) process, presenting a challenging approval landscape that innovative therapy companies must navigate.



Recently a gene therapy company was confronted with a request to demonstrate substantial outcomes data demonstrating the benefit of the therapy compared to SOC. This required the company to consider not only the cost of SOC, but the quality of life implications associated with blood transfusions.

Negotiations were not successful, as payers preferred the treatment be reimbursed at a 60% price cut for the therapy. While many may argue that this was not reasonable, a more careful analysis reveals lessons learned for other gene therapy companies entering into reimbursement discussions.

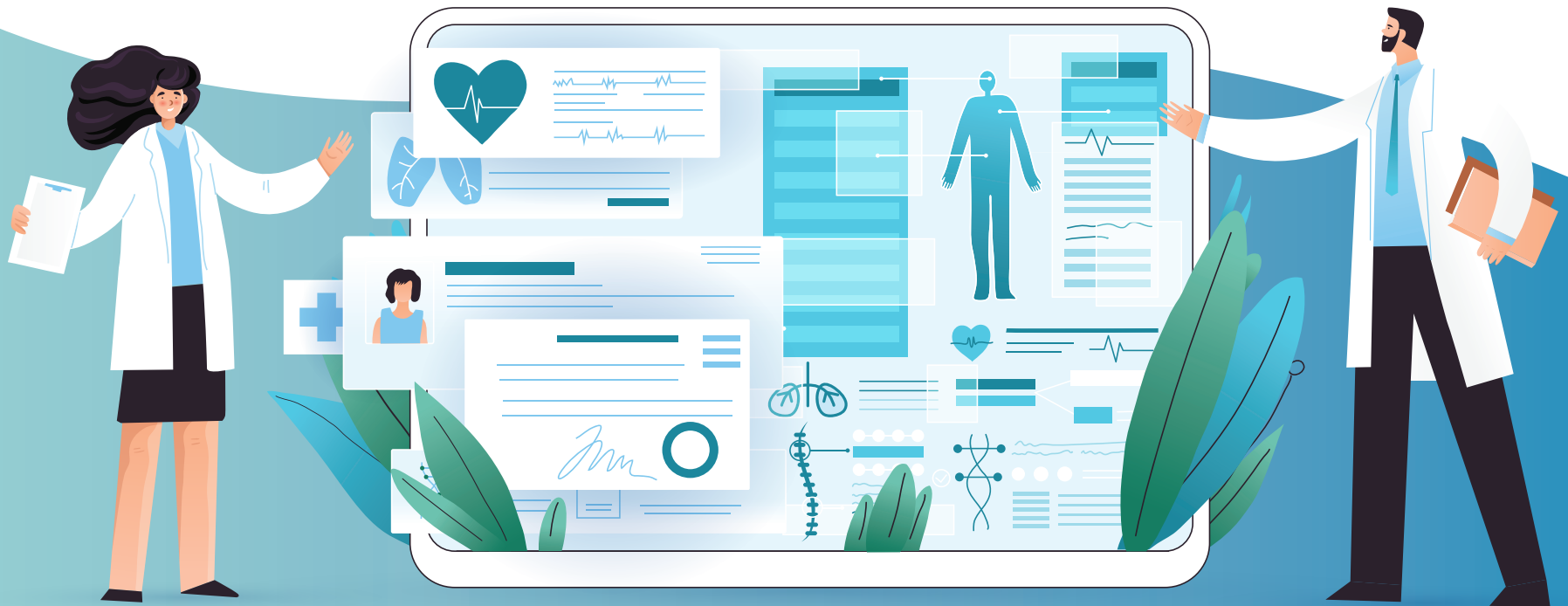


**Cost Effectiveness by Subpopulation:** Analysis by our HEOR team reveals that only patients who receive 7 or more blood transfusions incur a cost of care that actually exceeds the proposed price for this gene therapy. In our opinion, the company should have identified the presence of subpopulations within their patient population, and determined which groups of patients and respective cost of care aligned with the original price tag.

- Based on this finding, if a biotech's goal is to treat the broader population, then the proposed price for therapy should more accurately reflect the cost of care across this blended population.
- Alternatively, companies can consider entering into value based reimbursement agreements with independent payers, however, this adds additional complexity to the approval process, impacting timelines, resource requirements, and potentially delaying access to therapy for patients in need.



This scenario is not an isolated example of the reimbursement challenges faced by gene therapy companies. For a retinal dystrophy treatment, many markets provide access to the therapy, however, at a heavily discounted price compared to the original list price. Additionally, a treatment for SMA is heavily discounted in many markets, and in the Netherlands fails to meet the cost effectiveness criteria specific to the durability of the treatment (Broekhoff et al. 2021). Another consideration could be that the approved label for the one-time SMA product is broader than the original trial populations, leading to uncertainty with payers.



**U.S. Payer & Regulatory Impacts:** While these recent instances have occurred outside the U.S., regulators and payers are constantly assessing the cost effectiveness of new therapies. This requires gene therapy developers to clearly define the treatment populations and subpopulations, and also consider mechanisms to capture the appropriate outcomes data to demonstrate effectiveness compared to SOC.



**HEOR should be Top of Mind:** Gene therapy companies should carefully consider the evidence guidelines presented by regulatory entities, and also by payers, which tend to enforce more rigorous thresholds for efficacy data across clinical trials and comparator natural history datasets. Further consideration should also be applied to the evidence generation strategy to minimize the disconnect between regulatory approval and clinical trial populations, therefor leading to greater payer confidence.

To appropriately address the cost effectiveness and value-based outcome requirements, SSI recommends incorporating HEOR early on in the development of a therapy:

- Including considering the value across therapy stakeholders
- Collecting and analyzing real world evidence across broader disease populations
- Ensuring translatability of endpoint selection
- Demonstrating the durability of treatment effect





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