



Economic Evidence on Potentially Curative Gene Therapy Products: A Systematic Review

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Gene therapy is an emerging class of treatment based on altering gene expression to treat or cure severe medical conditions. Curative gene therapies offer the potential for lifelong benefits from a single treatment, which may result in these therapies being more cost-effective over the long term relative to current therapies, even at significantly higher costs. Given the high cost, uncertainty about long-term efficacy, and increases in eligible patient populations as new treatments are approved, concerns have emerged around the feasibility and financial impacts of gene therapies for patients and healthcare systems. The objective of this study was to conduct a systematic review of existing cost-effectiveness studies on curative gene therapies, and identify potential challenges that economic evaluations face in this area.

All gene therapies were deemed effective relative to their comparators; however, due to high costs, most were not deemed cost-effective. The range in cost effectiveness ratios was substantial, both between and within specific gene therapy products, ranging from dominant to over 2 million USD per Quality-Adjusted Life Year (QALY), far exceeding accepted thresholds for cost-effectiveness. Some of the model parameters with the greatest impact on cost-effectiveness included assumptions about the efficacy and duration of the therapy, alternative treatment used as comparator, and the inclusion of indirect costs.