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Gene Therapy Reimbursement Models – A Stakeholder Assessment

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Introduction: The first one-time curative gene therapy was approved in December 2017 with a list price of $850,000, and there is a historic increase in gene therapy clinical trials with 89 having reached phase III as of May 2018. Gene therapy's high upfront cost will challenge the traditional reimbursement models which are well suited for common, chronic conditions.

Objective: This stakeholder assessment surveys groups to identify existing opinions and generate insights regarding gene therapies and their reimbursement models.

Methods: A 12 question survey was sent to 200 stakeholders with the list generated from author's contacts. Stakeholder groups targeted were providers, payers, biotech/pharma, finance, consulting, government, patients &/or disease advocacy groups, scientists, and think tanks/academia. Findings from the survey are intended to be directional and hypothesis-generating instead of statistically representative.

Results: 26 individuals responded to the survey, but based upon self-reported familiarity with gene therapies five respondents were excluded.

The average favorability of gene therapy model adoption in the next 5 years (n=21) was as follows: outcomes based payments (7.33), annuity payments (5.52), upfront payments (4.62), modified upfront payments (5.61).

The average favorability of gene therapy model adoption in the next 15 years (n=21) was as follows: outcomes based payments (7.76), annuity payments (5.57), upfront payments (4.29), modified upfront payments (5.52).

Conclusion: Stakeholders surveyed responded with the highest favorability for outcomes based contracts and expressed general positive responses regarding efficacy/safety profiles. The main limitation for this survey is the sample size (n=21) and stakeholder make up with only 2 payers/insurers. Next steps include collecting more payer responses.

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