Gene Therapy Reimbursement Models - A Stakeholder Assessment

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Introduction

The first one-time curative gene therapy Spark's vantigene neuroaporevax (Luxturna) was approved in December 2017 for RPE65-mediated inherited retinal disease with a $850,000 list price.¹ There is a historic increase in gene therapy clinical trials with 89 having reached phase III as of May 2018.² In 2017 there were 285 gene therapy candidates in clinical development compared to 143 small molecules and 129 monoclonal antibodies.³ 20% by number & 29% by market capitalization of precommercial public biotech companies in November 2017 were gene therapy focused.⁴

Gene therapies will challenge traditional reimbursement models which are suited for paying for treatments related to common, chronic conditions. As gene therapies reach market the healthcare system will be pressured by the high upfront cost of treatments.⁵ Healthcare stakeholders including payers, providers, and pharmaceutical firms have begun experimenting with reimbursement models to address development costs as well as access, list price, and efficacy/safety concerns. These reimbursement models include outcomes based contracts, annuity payments, and upfront payments.⁶ These models are not mutually exclusive and can be used in combination with other financial or insurance vehicles such as reinsurance and carveouts.⁷

In January of 2018 Spark announced an outcomes based contracting model which shares risk with commercial payers and/or specialty pharmacies. This model leaves negotiation for payments to treatment centers up to commercial insurers. The model offers rebates to commercial insurers based on short term efficacy and long term durability. Spark is also currently working with the CMS on an installment payment program due to concerns with current government price-reporting requirements.⁸ The goal of this stakeholder assessment is to identify existing opinions and generate insights regarding gene therapies and their reimbursement models.

Methods

A twelve question survey was designed to collect demographic information and stakeholder feedback regarding gene therapies and reimbursement models. Key findings from the survey are intended to be directional and hypothesis-generating instead of statistically representative. Survey questions were designed to address findings raised from the results of a June 2018 Health Affairs Blog survey of commercial payers.⁹

- Question 1 was a stakeholder group self-identification where participants were able to select more than one option. See figure one for list of stakeholder categories.
- Question 2, 3, and 4 used a likert scale 1-10 to ask about familiarity with gene therapies and efficacy and safety concerns
- Questions 5 and 6 used a likert scale to ascertain favorability of four reimbursement models (outcomes based contracts, annuity payments, upfront payments, and modified upfront payments). Question 5 specified the time frame as the next 5 years and question 6 specified the time frame as 15 years. These questions were designed to identify potential trends in model favorability over time.
- Question 7 presents an annuity model example and different payment duration options.
- Questions 8-12 were open free response questions designed to collect stakeholder insights regarding current list prices, patient access & affordability, and global barriers.

A list of 200 stakeholders was generated from authors contacts and via Philbyio, a healthcare industry networking organization. Care was taken to include representatives from each of the following stakeholder groups described in the introduction. 26 individuals completed the survey. Based upon self reported familiarity with gene therapy below 5, four individuals were removed from subgroup analysis. These removed individuals self-identified as finance (n=1), provider and patient or patient/disease advocacy group (n=1), and two patient or patient/disease advocacy group (n=2). One provider was also removed due to familiarity indicated in free response answers.

Results

The self identification of the survey participants can be found in figure 2. Since question one allowed for self identification in multiple the 26 respondents represent 31 separate self identification groups. For later sub analysis, the stakeholder group utilized was taken as the respondents current role. Government stakeholders did respond to survey requests however as federal employees are unable to comment on reimbursement models.

The averages including all 26 respondents for gene therapy familiarity, efficacy concern, and safety concern are 6.3, 6.6, and 5.5 respectively. Once respondents unfamiliar with gene therapies were removed (n=21) the gene therapy familiarity, efficacy concern, and safety concern are 7.6, 6.3, and 5.6 respectively. Stakeholders surveyed on average believe that gene therapy efficacy benefits outweigh safety concerns. Note that a high efficacy score is uncorrelated with long term efficacy and a low safety score indicated concern for long term safety.

The average favorability of gene therapy model adoption in the next 5 years excluding low familiarity responders (n=21) is as follows: outcomes based payments (7.53), annuity payments (5.92), upfront payments (4.62), modified upfront payments (5.64).

The average favorability of gene therapy model adoption in the next 15 years excluding low familiarity responders (n=21) is as follows: outcomes based payments (7.76), annuity payments (5.97), upfront payments (4.40), modified upfront payments (5.52).

Results Continued

The 21 responders analyzed for response based upon subgroup. Note again that results are not statistically representative but rather hypothesis generating.

- No note modified upfront payment modified by risk adjustment or favored.

![Figure 3](image)

The most favored duration for an annuity payment was 10 years (42.3%) which may reflect beliefs about ability of payers to bear costs of treatments. It also may reflect beliefs in gene therapy durability of response.

Conclusion

Stakeholders surveyed responded with the highest favorability for outcomes based contacts and expressed extreme positive/reproductive safety profiles. The main limitation for this survey is the sample size (n=21) and make up of respondents; therefore results are hypothesis generating and not statistically representative of stakeholder groups. Next steps include collecting more responses.

Gene therapy reimbursement models will face many difficulties as more candidates gain FDA approval. The US healthcare system will face challenges adapting payment and reimbursement models to reduce market barriers, address high R&D costs, and encourage patient access. Gene therapies represent just one pain point as the healthcare system shifts from reimbursing based on volume to reimbursing based on value and patient outcomes.

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Citations