U.S. PAYER RESEARCH
ALTERNATIVE FINANCING MECHANISMS FOR GENE THERAPY AND TRANSFORMATIVE THERAPIES

ONLINE PAYER SURVEY

Introduction Screen

We would like your feedback on a new approach to pay for therapies that have high upfront costs but theoretically could result in a lifetime of avoided medical or other drug cost burden, together with extended and higher quality of life for patients.

*We will be asking about **gene therapy** specifically.*

Under the approach, payments to drug manufacturers would be spread over a few payments when certain milestones are met ("milestone payments"), or over a set of regular annual payments for a number of years ("long-term payment agreements"), rather than being fee-for-service payment(s) associated with administration of the therapy, or associated diagnostic tests and medical interventions.

I will ask you some questions to understand your expectations for how they would work, the conditions under which your organization would consider them, and your organization's likely response to such offers.

Please assume unless price is stated that the therapies are cost-effective at the net price to be charged your organization (we are interested in how factors other than price would influence your organization's response to such approaches).

Motivating Concerns and Awareness

1. How much a concern for your organization is managing the financial risk and impact of gene therapies? *(on a scale of 1 to 5, with 1 meaning "not at all a concern" and 5 meaning "a very high concern.")*

2. Are there specific disease areas and new therapies of this type that you are particularly concerned about? *(Yes / No; if yes, please list)*

3. Assuming the therapy is otherwise something you would provide access to, please select by line of business which factor would be **most important** in triggering your organization's serious consideration of one or more alternative approaches to manage the financial risk and impact of such therapies?

   Would a high upfront treatment cost per treated patient, or a high total PMPM impact (taking into account the number of treated patients), be the most important trigger for your organization's serious consideration of such proposals?

   (By line of business: high upfront treatment cost per patient is much more important; high upfront treatment cost per patient is somewhat more important; both factors are about equal; high total PMPM impact is somewhat more important; high total PMPM impact is much more important; neither factor would trigger our serious consideration of an alternative payment model)

4. Please indicate by line of business the dollar amount of treatment costs per patient or total PMPM impact that would trigger your organization seriously considering an alternative financing approach to mitigate its budget impact. *(By line of business, whole number)*

Approaches to Managing Costs for Gene Therapies

5. Which of the following approaches do you think your organization will use to manage the financial risk and impact of new gene therapies, by line of business? **Please check all that apply**.

   By line of business: Therapies will be excluded from coverage; Stop-loss with reinsurance; Carve-out benefit managed separately (e.g., similar to the way the mental health benefit may be managed by a separate entity); Separate benefit structure (i.e., a gene therapy benefit that is sold separately from the traditional medical and pharmacy benefit); Payment agreement that smooths payments over 1 to 2 years or less; Payment agreement that smooths payments over 3 to 5 years; Payment agreement that smooths payments over more than 5 years; Other (Please specify).

6. You indicated in the prior question that your organization will use approaches that smooth treatment payments over time to manage the financial risk and impact of gene therapy. **How would you expect this approach to implemented**? *(Choose one)*

   - Formal, written contractual agreements with manufacturers
   - Manufacturers bill in installments using procedure or J-codes
   - Either approach works
   - Both approaches are problematic

7. All other factors equal, would the following **factors affect your organization's willingness** to enter into these sort of arrangements? *(Yes / No to each)*

   - Whether the disease is a genetic versus non-genetic disease
   - Whether the therapy involves one-time administration of therapy versus multiple times
   - Whether the therapy directly reduces your budget (by reducing, e.g., covered hospitalizations, ER visits, physician visits, pharmacy costs)
   - Whether the therapy is a cure versus patients still requiring ongoing maintenance therapy and treatment
   - Whether patients are children versus adults versus seniors
   - Other factors [Please specify _____]

8. Would you expect any **performance-based requirements for payment**? (i.e., your payments would only be made as long as the therapy was working, or the patient was achieving specified clinical performance metrics)

   - Yes
   - No
   - Depends on the specific circumstances

9. What types of **metrics** would you expect to use for the **performance-based requirement** for payment?

- Clinical metrics only (achievement of demonstrable clinical response to therapy, using appropriate diagnostic tests or clinical marker(s))
- Financial metrics only (achievement of threshold improvements in total cost of care, or other financial metric)
- Both
- Either; depends on the specific circumstances

10. Are there circumstances under which you would expect the stream of payments made to the manufacturer to **terminate prior to the original agreed-upon date**? *Check all that apply.*

    - When patients die
    - When patients stop responding to therapy (according to previously agreed-upon metrics)
    - When patients transfer their insurance coverage from my company to another commercial payer
    - When patients transition from commercial coverage to some type of government insurance (to Medicaid, or to Medicare when the patient turns 65)?
    - Other [Please specify _____]

11. Which of these elements would you say are important (with 1 being not at all important, and 5 being very important) to your organization in **deciding whether or not to participate in such programs**? [please rate each element on a scale of 1 to 5]

    - Term (the number of years the payouts are stretched over)
    - Performance-based requirements for payment being included
    - Termination of my payment obligation with death of the patient
    - Termination of my payment obligation with transfer of patient from my coverage to another commercial insurer (if not, my payment obligation would "follow the patient" to any future insurer)
    - Termination of my payment obligation with transfer of patient from my coverage to a government insurer (Medicare or Medicaid)
    - Other [Please specify _____]

12. Would any of these factors be "**deal-breakers**" for your organization when considering whether to participate in an alternative payment program? *(Yes / No to each)*

    - Term (the number of years the payouts are stretched over)
    - Performance-based requirements for payment being included
    - Termination of my payment obligation with death of the patient
    - Termination of my payment obligation with transfer of patient from my coverage to another commercial insurer (if not, my payment obligation would "follow the patient" to any future insurer)
    - Termination of my payment obligation with transfer of patient from my insurance coverage to a government insurer (Medicare or Medicaid)
    - Other [Please specify _____]

Scenario 1: Gene therapy for very rare childhood genetic disorder

Now I would like you to assume that the following product profile represents the ***first gene therapy*** approved by the FDA that your organization is considering covering. Please read the following profile carefully and respond to the questions that follow:

Disease Description	• A very rare childhood genetic disorder that affects young children • Children with this disorder on average currently die at age 5
Prevalence	• Fewer than 1 in 50,000 children
Current Treatment Options	• No effective treatments are currently available • Current standard of care averages $100,000 per year and consists of regular maintenance infusion therapy and treatment of side effects and complications
New Therapy Description	• New gene therapy will be administered once • Therapy involves several steps, including an initial hospitalization to extract cells from the patient, modification of those cells in a lab, and then reinfusion of the genetically-modified cells in the patient on an outpatient basis
New Therapy Efficacy	• Therapy is curative for patients that respond (i.e., they live a normal life going forward) • Short-term clinical trials with small populations suggest 80% of patients respond • No long-term data are available
Therapy Cost	• The cost of the new one-time gene therapy, including the inpatient hospital stay, drug infusion, and all preparatory and follow-up care is $1,000,000

13. In the **absence of a special alternative payment program** negotiated with the drug manufacturer, what would you expect the **coverage and management of the new gene therapy**, as described, to be, for each of your lines of business?

    By line of business: Covered with restrictions only as specified in the FDA-approved label; Covered with more restrictions than the FDA-approved label; Not reimbursed; Other (please specify).

14. If covered with more restrictions than the FDA-approved label, do you anticipate imposing an explicit **waiting list**? [Y / N]

15. Which of the following approaches do you think your organization will use to manage the financial risk and impact of this new therapy, by line of business? *Check all that apply*.

    By line of business: Therapies will be excluded from coverage; Stop-loss with reinsurance; Carve-out benefit managed separately (e.g., similar to the way the mental health benefit may be managed by a separate entity); Separate benefit structure (i.e., a gene therapy benefit that is sold separately from the traditional medical and pharmacy benefit); Payment agreement that smooths payments over 1 to 2 years or less; Payment agreement that smooths payments over 3 to 5 years; Payment agreement that smooths payments over more than 5 years; Other (Please specify).

16. For approaches that smooth payments over time, would you expect payment to be dependent upon **performance-based requirements**? (i.e., the payments would only be made as long as the therapy was working, or patient was achieving specified clinical performance metrics) *(Yes / No)*

17. What types of **metrics** would you expect to use for the **performance-based requirement** for payment?

    - Clinical only (achievement of demonstrable clinical response to therapy, using appropriate diagnostic tests or clinical marker(s))
    - Financial only (achievement of threshold improvements in total cost of care, or other financial metric)
    - Both

18. Please assume that the previous product profile represents one of 20 gene therapies in the market place for various diseases and patient types (it is no longer the first gene therapy, but one of many). Would your approach to managing the financial risk and impact change? [Yes / No]

    By line of business: Therapies will be excluded from coverage; Stop-loss with reinsurance; Carve-out benefit managed separately (e.g., similar to the way the mental health benefit may be managed by a separate entity); Separate benefit structure (i.e., a gene therapy benefit that is sold separately from the traditional medical and pharmacy benefit); Payment agreement that smooths payments over 1 to 2 years or less; Payment agreement that smooths payments over 3 to 5 years; Payment agreement that smooths payments over more than 5 years; Other (Please specify).

Scenario 2: Gene therapy for adults with genetically-linked cancer

Now I would like you to assume that a **gene therapy** has been approved by the FDA for the treatment of **adults with a genetically-linked cancer.** Assume that **this is the first and only gene therapy on the market that your organization is considering covering.** Please read the following profile carefully and respond to the questions that follow:

Disease Description	• Adults with a genetically-linked cancer • Adults with this disorder on average die 10 years after diagnosis
Prevalence	• 1 in 500 adults
Current Treatment Options	• There are other effective therapies for this cancer, but they have significant side effects and the response rate is only 25% • The five-year survival rate (across responders and non-responders) is approximately 10% • The current standard-of-care consists of radiation, surgery and chemotherapy regimen, together with treatment of side effects and complications
New Therapy Description	• New gene therapy will be administered once • Therapy involves several steps, including an initial hospitalization to extract cells from the patient, modification of those cells in a lab, and then reinfusion of the genetically modified cells in the patient on an outpatient basis
New Therapy Efficacy	• Therapy is curative for patients that respond (i.e., they live a normal life going forward) • Short-term clinical trials with small populations suggest 50% of patients respond • No long-term data are available
Therapy Cost	• The cost of the new one-time gene therapy, including the inpatient hospital stay, drug infusion, and all preparatory and follow-up care is $1,000,000

19. In the absence of a special alternative payment program negotiated with the drug manufacturer, what would you expect the **coverage and management of the new gene therapy**, as described, to be, for each of your lines of business?

    By line of business: Covered with restrictions only as specified in the FDA-approved label; Covered with more restrictions than the FDA-approved label; Not reimbursed; Other (please specify).

20. If covered with more restrictions than the FDA approved label, do you anticipate imposing an explicit **waiting list**? [Y / N]

21. Which of the following approaches do you think your organization will use to manage the financial risk and impact of this new therapy, by line of business?

    By line of business: Therapies will be excluded from coverage; Stop-loss with reinsurance; Carve-out benefit managed separately (e.g., similar to the way the mental health benefit may be managed by a separate entity); Separate benefit structure (i.e., a gene therapy benefit that is sold separately from the traditional medical and pharmacy benefit); Payment agreement that smooths payments over 1 to 2 years or less; Payment agreement that smooths payments over 3 to 5 years; Payment agreement that smooths payments over more than 5 years; Other (Please specify).

22. For approaches that smooth payments over time, would you expect payment to be dependent upon **performance-based requirements**? (i.e., the payments would only be made as long as the therapy was working, or patient was achieving specified clinical performance metrics) [Y / N]

23. What types of **metrics** would you expect to use for the **performance-based requirement** for payment?

    - Clinical only (achievement of demonstrable clinical response to therapy, using appropriate diagnostic tests or clinical marker(s))
    - Financial only (achievement of threshold improvements in total cost of care, or other financial metric)
    - Both

Barriers and Benefits

24. What would you say would be the **biggest barriers** to your organization accepting alternative payment proposals from drug manufacturers such as milestone-based payments or long-term payment agreements for gene therapies? [Please rate each barrier on a scale of 1 to 5, where 1 is not a barrier at all, and 5 is a deal-breaker]

    - Financial burden of covering the therapy
    - Program administration complexity
    - Information burden (to track patients, provide relevant data)
    - Insurance regulatory barriers (e.g., minimum reserve requirements)
    - Pricing and price reporting regulations (e.g., best-price requirement)
    - Uncertainty in cost accounting for multi-year agreements
    - Paying for patients who are no longer actively insured by me

- Paying for patients who are no longer responding to therapy
- Inability to off-load long-term risk
- Having to accept agreements for "incoming" patients negotiated previously by other payers
- Other [Please specify _____]

25. What would you say are the **biggest benefits** to your organization of accepting such a proposal? [Please rate each benefit on a scale of 1 to 5, where 1 is not a benefit at all, and 5 is a major benefit]

    - Reducing upfront budget impact of the new therapy by smoothing payments over time
    - Aligning the timing of the therapy costs with its benefits (costs would be paid over time, as benefits were realized)
    - Reducing uncertainty by locking in what the costs will be
    - Only paying for therapy that works (Including performance-based requirements for initial or continued payment)
    - Other [Please specify _____]

26. In your opinion, are "long-term payment agreements" that follow the patient from one insurer to another **feasible without legislation** requiring all payers to honor them? *[Y/N]*

27. Would you be **willing to accept the terms negotiated by an earlier payer** even if you think you could have reimbursed a lower amount, so long as other payers would accept the terms you had negotiated? *[Y/N]*

28. Would your willingness to accept these terms negotiated by other insurers **vary based on the dollar amoun**t? For instance, would you be less willing to accept them for a $1 million therapy than a $250,000 therapy*? [Y/N]*

29. In your opinion, does there need to be an explicit **written contract** between the manufacturer and the payer for this sort of approach to work? *[Y/N]*

30. Is there a **maximum total PMPM cost** your organization would be willing to accept in such arrangements negotiated by other insurers? *[Y/N]*

Performance-Based Contract Comparison

31. Would you consider these potential arrangements to be **more complicated or less complicated** than the performance-based payer contracts your organization has administered in the past? *(More complicated / About the same / Less complicated)*

32. Would your organization be **more or less willing to participate** in these potential arrangements than in performance-based payer contracts? *(More willing / About the same / Less willing)*

Wrap-up

33. Overall, what is the likelihood of your organization entering **into at least one such arrangement for gene therapy** within the next three years in any of your lines of business? *(Highly unlikely, Somewhat unlikely, Somewhat likely, Highly likely)*