

# **Payer Research: Alternative Financing Mechanisms for Gene Therapies**

## **Summary of Key Findings**

**Prepared for: National Pharmaceutical Council**

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# Project Objectives & Market Research Approach

## Project Objectives

- **Explore reactions to alternative payment models for transformative therapies with high upfront therapy costs, benefits over time, to different payers:**
  - Several “clinical milestone payments”
  - More extended “long-term payment agreements”
  - With or without performance guarantees
- **Focus on gene therapies**
  - A current issue for payers and manufacturers, as therapies are approved
  - Other transformative therapies may also share some features (i.e., high upfront therapy costs, patient switching over time)
- **Investigate circumstances under which alternative financing mechanisms are most acceptable and most viable**
  - Reactions and preferred approaches
  - “Deal-breakers”

## Market Research Approach

- **Online survey of 21 leading national and regional managed care organizations (MCOs) to assess:**
  - Level of concern about financial risk and impact of gene therapies
  - Trigger for willingness to consider alternative payment models (i.e., upfront treatment cost vs. high PMPM impact)
  - Factors affecting interest in alternative payment models (e.g., genetic vs non-genetic disease)
  - Expected management approaches, by line of business
  - Response to potential deal features (importance to decision to participate, “deal-breakers”)
    - **Term (period of time over which payments are made)**
    - **Performance-based requirements and metrics**
    - **Termination provisions**
  - Response to two gene therapy scenarios
    - **Scenario One: Extremely rare pediatric genetic disorder (Fewer than 1 in 50,000)**
    - **Scenario Two: Less rare adult genetically-linked cancer (1 in 500)**
  - Barriers to and benefits of alternative payment models
  - Likelihood of participating in alternative payment model within next three years
- **MCO participants represent approximately 123 million Commercial, Medicare Advantage, Managed Medicaid lives**
- **Caveat: Findings are directional and hypothesis-generating, and should not be considered statistically representative of all U.S. commercial payers**

# Key Findings

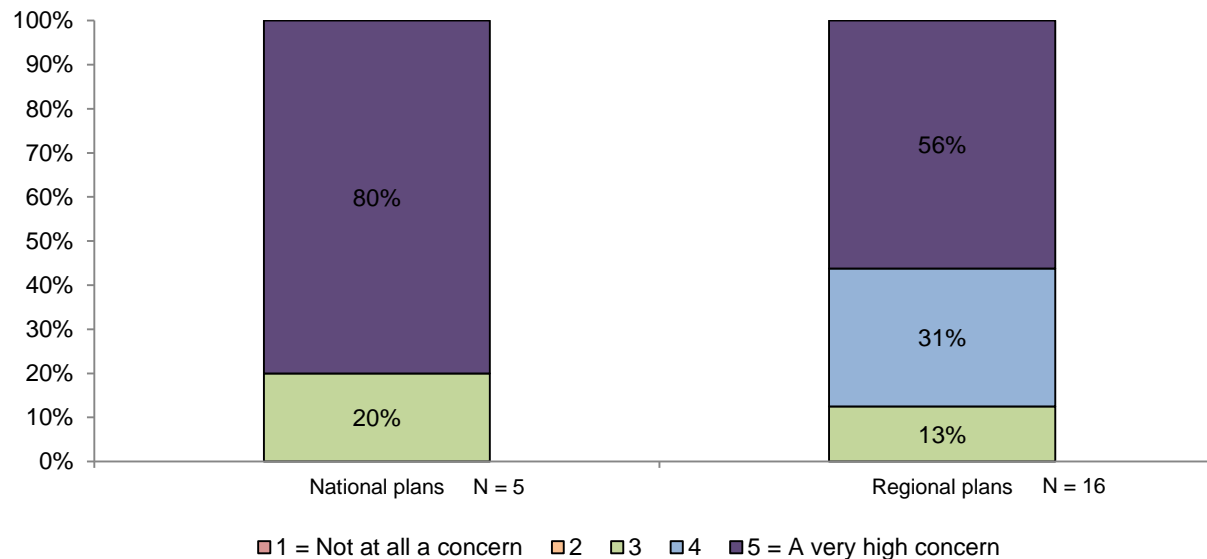
## Summary

- **Key Finding #1:** Financial risk and impact of gene therapies is an important concern for payers
- **Key Finding #2:** Payers would consider alternative payment models to manage plan risk under the right set of circumstances
- **Key Finding #3:** Payers expect to use a combination of new and existing approaches to manage the financial risk of gene therapy
- **Key Finding #4:** Given uncertainty about magnitude and duration of clinical effects, payers likely will require performance guarantees with alternative payment approaches
- **Key Finding #5:** Greatest patient access challenges may be encountered at smaller employer plans and managed Medicaid plans
- **Key Finding #6:** Payers' preferred approaches may not be entirely effective in addressing their concerns
- **Key Finding #7:** Many payers were uncomfortable with "patient portability" proposals

## Key Finding #1: Financial risk and impact of gene therapies is an important concern for payers

- Over half of respondents had **very high levels of concern about financial risk and impact of gene therapies** (4 of 5 national plans and 9 of 16 regional plans rated it a 5 of 5)
- Specific areas of concern** identified unaided included: oncology, hemophilia, spinal muscular atrophy, sickle cell disease, as well as “common chronic diseases” and “any disease that has a high incidence and hence high potential costs across a population”

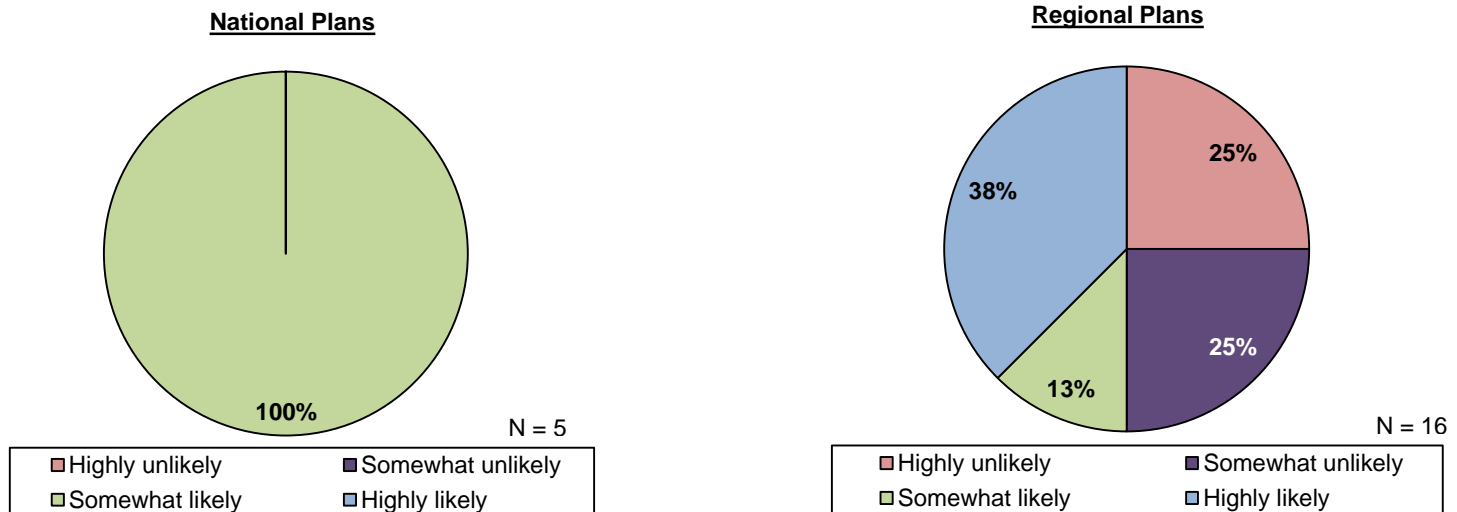
*Q: How much a concern for your organization is managing the financial risk and impact of gene therapies on a scale of one to five? (where one is “not at all a concern” and five is “a very high concern”)*



## Key Finding #2: Payers would consider alternative payment models to manage plan risk under the right set of circumstances

- All of the national plans and half of the regional plans said they were somewhat or highly likely to enter into at least one alternative payment arrangement for gene therapy within the next three years in any of their lines of business (LOBs).**

Q. 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?





## Key Finding #2: Payers would consider alternative payment models to manage plan risk under the right set of circumstances (cont'd)

- **High upfront treatment impact** was cited as **most important trigger** for Commercial Fully Insured, TPA – Larger ERs, and TPA – Small ERs, **together with “both factors are about equal”** for Commercial Fully Insured, Medicare Advantage, managed Medicaid.
- High total PMPM impact was not cited as highest-rated factor for any LOB.

*Q: 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*

Line of Business	High Upfront Treatment Cost Per Patient (Top Two Box*)	Both Factors Are About Equal	High Total PMPM Impact (Top Two Box*)	Neither Factor Would Trigger Our Serious Consideration
Commercial Fully Insured	<b>43%</b>	<b>43%</b>	10%	5%
TPA – Larger ERs	<b>47%</b>	41%	6%	6%
TPA – Small ERs	<b>50%</b>	38%	13%	--
Medicare Advantage	33%	<b>50%</b>	11%	6%
Managed Medicaid	33%	<b>40%</b>	20%	7%

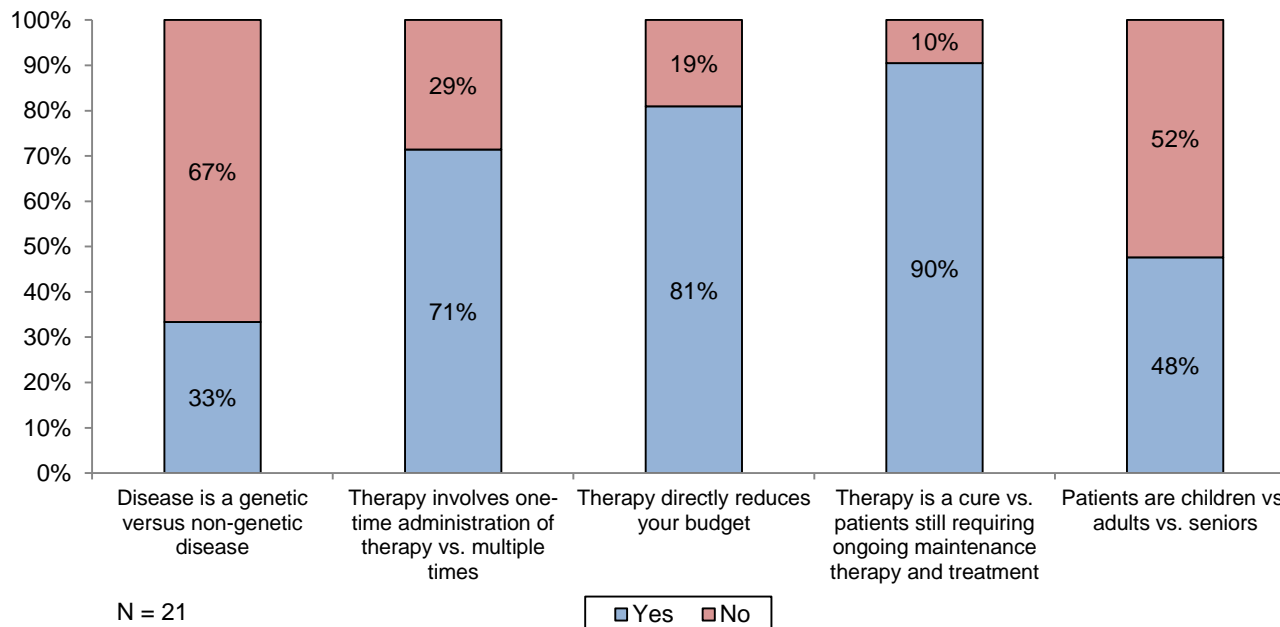
\* I.e., “much more important” and “somewhat more important” ratings.

Note: Bolding indicates most frequently cited trigger for a given LOB (top two box).

## Key Finding #2: Payers would consider alternative payment models to manage plan risk under the right set of circumstances (cont'd)

- **Factors most affecting interest in alternative payment models** affected plan economics:
  - therapy is a cure versus still requiring ongoing maintenance therapy and treatment (90%);
  - therapy directly reduces your budget (81%);
  - therapy involves one-time administration of therapy versus multiple times (71%).
- **Epidemiologic considerations were the least important considerations** for alternative models:
  - patients are children versus adults versus seniors (48%);
  - disease is a genetic versus non-genetic disease (33%).

*Q: All other factors equal, would the following factors affect your organization's willingness to enter into these sort of arrangements?*

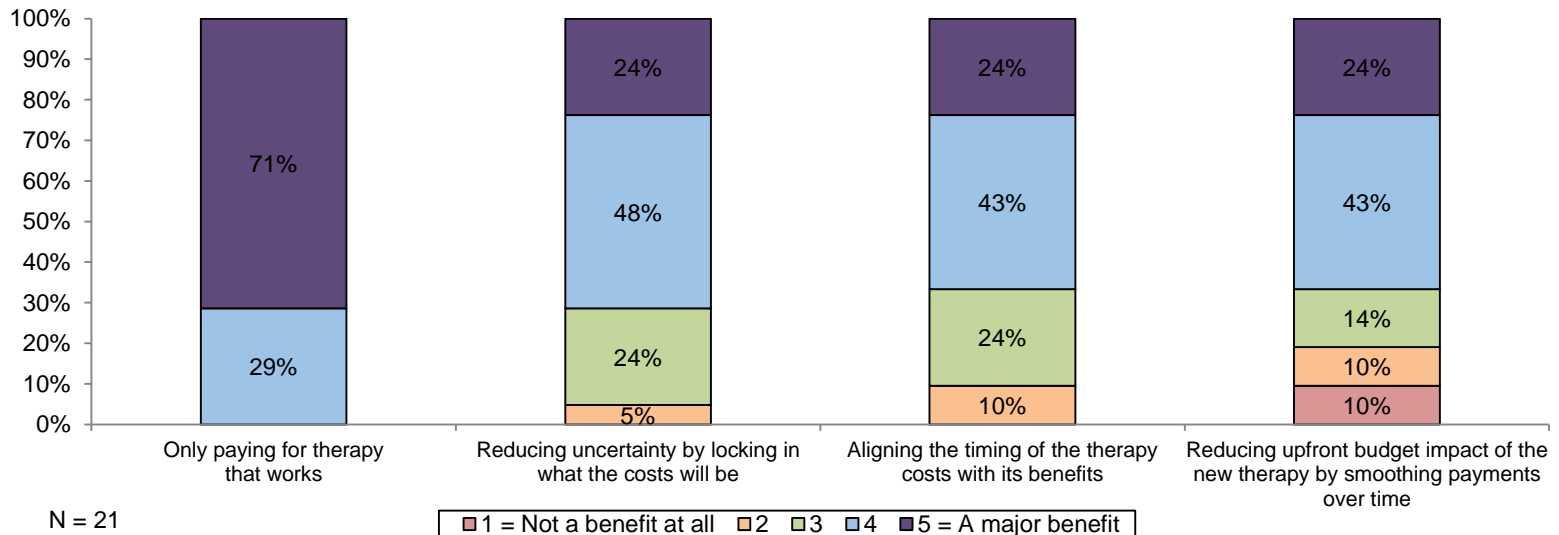


## Key Finding #2: Payers would consider alternative payment models to manage plan risk under the right set of circumstances (cont'd)

- On a scale of 1 (not a benefit at all) to 5 (a major benefit), **greatest benefits** for accepting an alternative payment model were reported for:
  - “only paying for therapy that works” was a major benefit (71% rated it a 5 of 5);
  - “reducing uncertainty by locking in what the costs will be”, “aligning the timing of the therapy costs with its benefits” and “reducing upfront budget impact of the new therapy by smoothing payments over time” had similar top two box scores (24% of respondents rated each a 5 of 5; 43%-48% rated each as a 4 or 5)
  - “Reducing upfront budget impact of the new therapy by smoothing payments over time” had the highest bottom two box score – 20% of respondents rated it a 1 or 2

*Q. What would you say would be the biggest benefits to your organization of accepting such a proposal? (where one is “not a benefit at all” and five is “a major benefit”)*

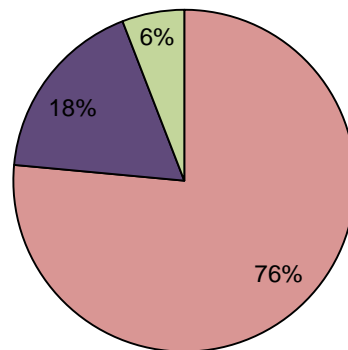
Shown by declining top box score:



## Key Finding #2: Payers would consider alternative payment models to manage plan risk under the right set of circumstances (cont'd)

- Of the respondents who reported they already had a performance-based contract in-place or had one planned soon (n=17), three-quarters said they would consider such alternative payment models arrangements to **be more complicated than P4P contracts**
- Of all respondents, about the same number (48% versus 43%) reported being **as willing to participate in these potential arrangements versus P4P contracts they had administered**, versus being **less willing to participate** (10% said they would be more willing)

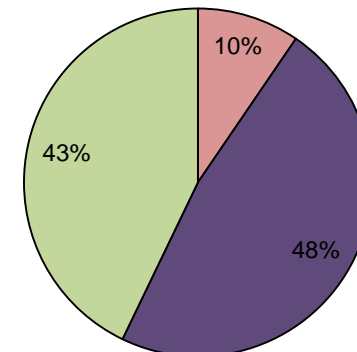
Q. *Would you consider these potential arrangements to be more complicated or less complicated than the performance-based payer contracts your organization has administered in the past?*



N = 17

■ More complicated ■ About the same ■ Less complicated

Q. *Would your organization be more or less willing to participate in these potential arrangements than in performance-based payer contracts?*



N = 21

■ More willing ■ About the same ■ Less willing

## Key Finding #3: Payers expect to use a combination of new and existing approaches to manage the financial risk of gene therapy

- Excluding managed Medicaid, **payers were most likely to use a combination of approaches** to manage gene therapy-related financial risk.
- Managed Medicaid** plans were more likely to expect to either use payment agreements or reinsurance or coverage exclusions (but not a combination) to manage such risk.

*Q: Which of the following approaches do you think your organization will use to manage the financial risk and impact of new gene therapies? (check all that apply)*

	Commercial Fully Insured	Medicare Advantage	Third Party Administrator		Managed Medicaid	All Payers, All Lines of Business
			Large Employer	Small Employer		
Coverage Exclusion	10%	11%	12%	31%	27%	17%
Benefit Design Only	14%	6%	18%	13%	7%	11%
Reinsurance Only	5%	22%	18%	6%	27%	15%
Payment Agreement Only	5%	28%	6%	13%	27%	15%
Combination Approach:	67%	33%	47%	38%	13%	41%
Payment Agreement and Reinsurance	29%	11%	12%	13%	7%	15%
Benefit Design and Reinsurance	19%	11%	12%	6%	0%	10%
Benefit Design and Payment Agreement	5%	0%	6%	6%	0%	3%
All Three Approaches	14%	11%	18%	13%	7%	13%

- Approximately half of the payers in each line of business expected to use a payment agreement (either alone or in combination with other approaches); in these instances, payers **preferred one- to two-year agreements over agreements that span three or more years.**

## Key Finding #4: Given uncertainty about magnitude and duration of clinical effects, payers likely will require performance guarantees with alternative payment approaches

- Across approaches, most respondents (57%) **expected performance-based requirements for payment** – an additional 33% said it depends on the specific circumstances. Of those who expect them, roughly half expect both clinical and financial metrics to be used (another 21% expected only clinical metrics; the remaining 32% expected “either; depends on specific circumstances”).
- For approaches that smooth payments over time, 90% of payers said they would expect payment to be dependent on meeting **performance-based requirements**. Of those who expected such requirements, two-thirds expected both clinical and financial metrics (one-third expected clinical metrics only).
- Moreover, 95% of respondents **said "paying for patients who are no longer responding to therapy" would be a major barrier or a "deal breaker"** (i.e., rated it a 4 or 5 on a scale of 1, not a barrier at all, to 5, a deal-breaker).

## Key Finding #5: Greatest patient access challenges may be encountered at smaller employer plans and managed Medicaid plans

- While payers have high levels of concern about the potential impact of new gene therapies, expected risk management responses, and their potential impact on patient access, vary.
- Nearly one-third of small employer TPAs and managed Medicaid plans indicated they were likely to **exclude coverage for gene therapies**.

*Q: Which of the following approaches do you think your organization will use to manage the financial risk and impact of new gene therapies? (check all that apply)*

Line of Business	Therapies will be excluded from coverage	Stop-loss with reinsurance	Carve-out benefit managed separately	Separate benefit structure	Payment agreement that smooths payments over time		
					1-2 years or less	3-5 years	More than 5 years
Commercial Fully Insured	10%	67%	19%	48%	33%	14%	5%
TPA – Larger ERs	12%	59%	29%	41%	24%	12%	6%
TPA – Small ERs	31%	38%	19%	25%	25%	6%	13%
Medicare Advantage	11%	56%	11%	17%	22%	17%	11%
Managed Medicaid	27%	40%	7%	7%	27%	7%	7%
All Payers, All Lines of Business	17%	53%	17%	29%	26%	11%	8%

Note: Respondents checking “payment agreement that smooths payments over time” restricted to one choice (i.e., either 1-2 years or less, 3-5 years, or more than 5 years)

## Key Finding #6: Payers' preferred approaches may not be entirely effective in addressing their concerns

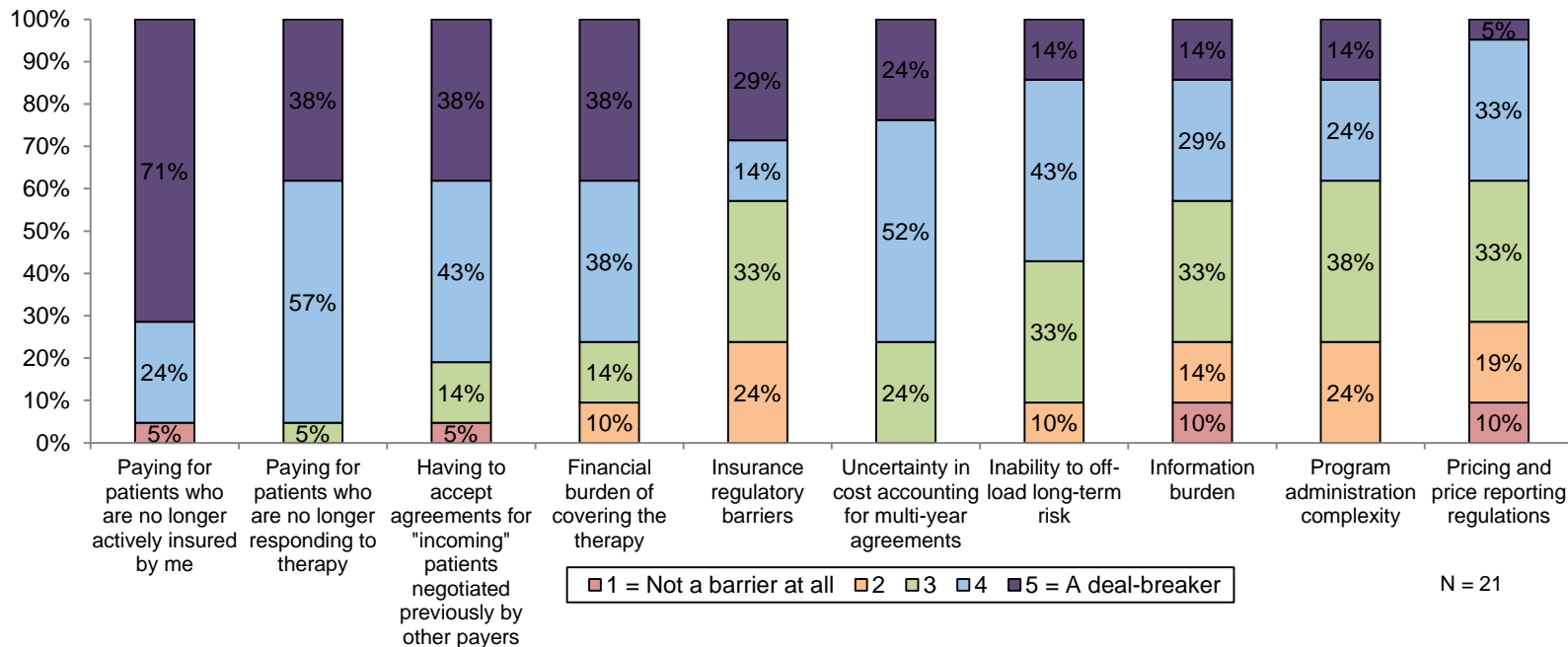
- Excluding managed Medicaid, we found that a substantial percentage of respondents would use **stop-loss/reinsurance in conjunction with a payment agreement** (see Key Finding #3).
- However, this may be a less effective strategy than payers would hope, for several reasons. **Stop-loss/reinsurance**, intended to protect against unforeseen outlier costs, may exclude costs for patients with known high costs, or that extend beyond the first year of a multi-year agreement.
- Moreover, pricing for such coverage would be expected to adjust upwards as costs for the new therapies are fully reflected.
- Finally, stop-loss/reinsurance coverage may not be easily integrated with performance guarantee provisions.



## Key Finding #7: Many payers were uncomfortable with “patient portability” proposals

- Many respondents rejected a central feature of financing proposals that smooth payments over time, where the "payment follows the patient" from payer to payer.
- Almost three-quarters of respondents indicated that continuing to pay for members who switch out of their plans would be a "deal-breaker."**
- Between one-half and two-thirds indicated that they **do not want to accept financial responsibility for similar members who switch into their plans.**

Q. 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?



## Contact Information:

Genia Long  
Analysis Group, Inc.  
[genia.long@analysisgroup.com](mailto:genia.long@analysisgroup.com)

Michael Ciarametaro  
National Pharmaceutical Council  
[MCiarametaro@npcnow.org](mailto:MCiarametaro@npcnow.org)