Quantifying Trends in Price Setting for Traditional and Innovative Advanced Rare Disease Therapies

RWD93

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Introduction

- Advancements in medicinal research have catapulted a growing number of treatments for patients with rare diseases into either approval or development, bringing promise for improved symptom management and longer survival for these patients[1,2]
- However, these innovative treatments for rare diseases are costly, and may contribute to drug availability differences and overall financial burden for health care systems in the short term[1,2]
- As the number of available treatments for rare diseases increases, payer decision-making processes are expected to shift within health care systems to control financial strain while expanding and accelerating public access to unique treatments[1,2]

Objective

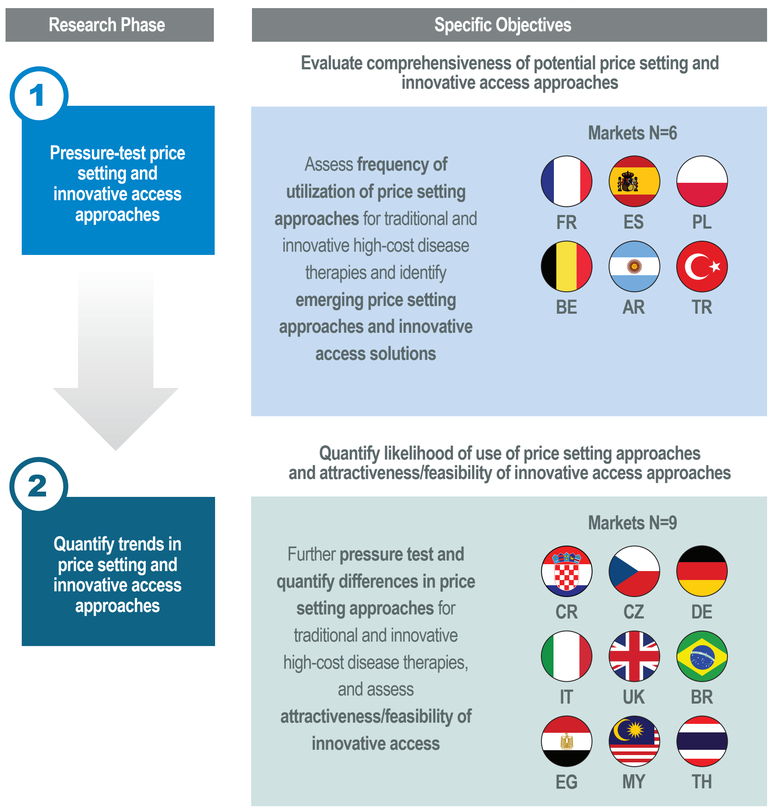
- We sought to evaluate current and emerging international price setting trends and novel access approaches for traditional versus innovative high-cost therapies typically utilized for the treatment of rare diseases

Methods

Stakeholder interviews

- A product-profile semi-quantitative approach was taken in two phases: Phase 1 assessed price setting approaches, Phase 2 quantified differences in price setting approaches for traditional and innovative high-cost therapies (**Figure 1**)
- Interviews were conducted over 60 minutes each with national ex-payers or ex-payer advisors who met the following recruitment criteria:
  - More than 3 years in former payer role
  - Familiarity with assessment of pricing and reimbursement and experience with pricing decisions for formal and informal pricing methods for traditional and innovative high-cost disease therapies
  - Moderate-to-high level of familiarity with price assessment approaches and outcomes for cell and/or gene therapies
- Target markets included both those with more mature health care systems (e.g., greater gross national income and health expenditures) and those with less mature, emerging health care systems (e.g., lower gross national income and health expenditures)
- Discussion topics for Phase 1 focused on current and potential future pricing dynamics for traditional and innovative high-cost therapies, with questions around drivers of visible/list and net price decisions and approaches for price setting, including setting or changing informal and formal reference pricing methods
- Discussion topics for Phase 2 focused on leveraging high-level hypothetical product profiles (e.g., net price in the United States, dosing, efficacy/safety, estimated number of eligible patients, anticipated budget impact) to:
  - Assess price-setting approaches and differences between traditional versus innovative high-cost disease therapies
  - Understand attractiveness and feasibility in implementing access approaches for traditional versus high-cost disease therapies

Figure 1. Objectives for Phases 1 and 2 of price setting evaluation



AR, Argentina; BE, Belgium; BR, Brazil; CR, Croatia; CZ, Czech Republic; DE, Germany; ES, Spain; EG, Egypt; FR, France; IT, Italy; MY, Malaysia; PL, Poland; TH, Thailand; TR, Turkey; UK, United Kingdom.

Results

- Phase 1 included a total of six participants, from both countries with more mature health care systems (France, Spain, Poland, Belgium) and countries with emerging health care systems (Argentina, Turkey) (**Table 1**)
  - Mature health care systems followed generally similar pricing approaches for both list and net prices via a standardized process; any changes in pricing typically relied on new policies and/or legislation
  - Emerging health systems provided flexibility in pricing assessments and relied on informal price referencing to markets with visible net pricing details
  - Payers across all markets prioritized clinical value assessments when setting list and net prices for innovative high-cost therapies to justify the higher price per patient

Table 1. Overview of price setting approach across market archetypes

	More mature health care systems (e.g., France, Spain, Belgium, Poland)	Emerging health care systems (e.g., Argentina, Turkey)
Summary of insights	• Largely rely on well-established approaches and policies to assess **clinical value, budget impact, and/or cost effectiveness** and inform net price levels • Use informal price referencing to **assess baseline for list/visible price** • Do not cite use of **informal price referencing to assess net price** due to **lack of visibility** into net price in relevant reference markets • Often utilize **outcomes-based contracting or annuities** in net price negotiations for innovative high-cost therapies to mitigate high upfront costs	• **No formal procedures** for setting list or net prices • Rely on non-regimented approaches to assess **clinical value, budget impact, and/or cost effectiveness to further refine net prices** • Increasingly leverage informal price referencing for more high-cost therapies to assess **baseline for list/visible price** and to **pressure-test net price level** • **Affordability concerns** drive payers to increasingly explore opportunities to leverage payment-over-time contracts (e.g., outcomes-based contracting, annuities)

- Phase 2 included nine participants from nine countries, including those with more mature health care systems (Croatia, the Czech Republic, Germany, Italy, the United Kingdom) and emerging health care systems (Brazil, Egypt, Malaysia, Thailand)
- Payers across all markets displayed a high likelihood of using clinical value assessment and referencing other countries' prices for formal price setting approaches, while only payers in markets with emerging health care systems were more likely to leverage informal price setting approaches in general, and specifically with a high likelihood of referencing other countries' prices (**Table 2**)

Table 2. Price setting approaches in mature versus emerging markets

	Price setting approach	Likelihood to use by market archetype: More mature health care system	Likelihood to use by market archetype: Emerging health care system	Additional details
Formal Approaches	Clinical value assessment	*High*	*High*	• Payers are **highly likely to consider clinical value** when setting list and net prices across market archetypes to ensure prices appropriately reflect relative clinical value
Formal Approaches	Cost-effectiveness assessment	*High*	*Moderate*	• Markets with more mature health care systems are more likely to have well-established approaches for evaluating **cost effectiveness**
Formal Approaches	Budget impact assessment	*Moderate*	*High*	• Markets with emerging health care systems are broadly more likely to analyze budget impact when setting list/net prices due to **greater constraint on health care budgets**
Formal Approaches	Referencing other countries' prices	*High*	*High*	• Payers across market archetypes are **highly likely** to use external price referencing to ensure list/net price is optimized relative to the value/price assessed by other markets
Informal Approaches	Referencing other countries' prices	*Moderate*	*High*	• Markets with emerging health care systems are **more likely to reference prices beyond standard basket markets** to ensure list/net price aligns to lowest visible price
Informal Approaches	Competitor benchmarking	*Moderate*	*Moderate*	• Across market archetypes, payers are moderately likely to benchmark list/net price of traditional and innovative high-cost therapies against **in-market competitors** to ensure that **relative clinical value** is reflected in the final negotiated list/net price
Informal Approaches	Budget impact assessment	*Low*	*Low*	• Payers across market archetypes are unlikely to informally assess budget impact as it is typically considered in the context of **formal price setting approaches**
Informal Approaches	Referencing ICER/other markets' cost-effectiveness evaluations	*Low*	*Low*	• Payers across market archetypes are **unlikely to evaluate cost-effectiveness reports from other organizations/ markets**, often due to inability to translate report findings into actionable insights to inform list/net pricing decisions

Average likelihood to use approach across therapy types: *Low* *Moderate* *High*

ICER, Institute for Clinical and Economic Review.

- Payers were more likely to scrutinize clinical value (high priority) and budget impact (moderate priority) for high-cost therapies versus traditional therapies; however, a similar approach was expected for cost-effectiveness and price referencing for both high-cost and traditional therapies (**Table 3**)

Table 3. Formal list and net price setting approaches for traditional versus innovative high-cost therapies

	Formal price setting approaches	Anticipated difference in use for traditional vs. high-cost treatment[a]	Additional details
Increasing Level of Difference ↑	Clinical value assessment	*High*	• Payers across markets (🇧🇷🇭🇷🇨🇿🇺🇸🇬🇧) reported a higher likelihood to focus on and/or scrutinize **clinical value** when evaluating list/net price for **high-cost therapies** (vs. traditional therapies) to ensure the price reflected the relative clinical improvement and/or the data demonstrated convincing **durability of efficacy**
Increasing Level of Difference ↑	Budget impact assessment	*Moderate*	• Overall, payers across most markets noted they were likely to **assess budget impact similarly** across both therapy types for list and net price setting decisions to minimize increases in year-over-year health care budget • Payers in some markets (🇨🇿🇺🇸) stated they were more likely to emphasize **budget impact assessments** when setting list price for **one-time, high-cost therapies** to mitigate potential risks of **high upfront costs**
Increasing Level of Difference ↑	Cost-effectiveness assessment	*Low*	• Payers across most markets highlighted they were likely to **utilize cost-effectiveness assessments similarly** across therapy types for list and net price setting decisions, with most markets either **requiring an analysis** for every new therapy or unlikely to consider cost effectiveness **regardless of therapy type** • However, one payer (🇭🇷) noted they were less likely to rely on **cost-effectiveness evaluations** in list/net price setting of one-time, high-cost therapies due to challenges in assessing an **appropriate time horizon** for cost-effectiveness analyses given **uncertainties in durability of efficacy**
Increasing Level of Difference ↑	Referencing other countries' prices	*Low*	• Payers across markets reported they were likely to use formal price referencing **similarly** across therapy types for list/net price setting, with most markets considering it a key factor for optimizing the **final list and/or net price** • However, one payer (🇭🇷) stated they were **more likely** to utilize formal price referencing when setting **list prices for traditional therapies** due to a greater likelihood of having traditional therapies approved and reimbursed, enabling greater availability of/access to list prices

[a]Average across all markets.

- Payers reported being more likely to reference other countries' prices and use competitor benchmarking for high-cost therapies than for traditional therapies (**Table 4**)

Table 4. Informal list and net price setting approaches for traditional versus innovative high-cost therapies

	Informal price setting approaches	Anticipated difference in use for traditional vs. high-cost treatment[a]	Additional details
Increasing Level of Difference ↑	Referencing other countries' prices	*Moderate*	• Given the higher anticipated **budget impact**, one payer (🇮🇹) stated they may utilize **informal external price referencing** for innovative high-cost therapies to ensure **list/net price is optimized** relative to other markets • Payers in some markets (🇩🇪🇬🇧) noted that informal external price referencing to non-standard basket markets may be considered in price negotiations for innovative high-cost therapies to understand how other markets **perceive the clinical value** and/or help **inform final negotiated net price**
Increasing Level of Difference ↑	Competitor benchmarking	*Moderate*	• Payers in some markets (🇧🇷🇨🇿) reported they were more likely to use competitor benchmarking for setting the list/net price of **innovative high-cost therapies** to ensure the negotiated price **reflects the product's relative clinical value**
Increasing Level of Difference ↑	Budget impact assessment	*Low*	• Payers across markets reported that they were **unlikely to implement informal budget impact assessments** across therapy types since it was usually considered in the context of formal list/net price setting approaches • However, one payer (🇩🇪) stated they may informally analyze budget impact for therapies with **large anticipated patient populations** or **high costs** if budget impact was not emphasized in the formal list/net price setting approach
Increasing Level of Difference ↑	Referencing ICER reporting	*Low*	• Payers across some markets stated they were **unlikely to reference ICER reports** in list/net price setting decisions for both therapy types and did not expect this approach to change in the future • However, payers in other markets(🇮🇹🇬🇧🇺🇸🇹🇭) reported they may reference ICER reports to provide **additional context on the cost utility** for both types of therapies
Increasing Level of Difference ↑	Cost effectiveness from other markets	*Low*	• Payers across most markets noted they were **unlikely to reference cost-effectiveness reports from other markets** given lack of ability to translate the impact of findings into list/net price and did not expect this to change • However, some payers (🇮🇹🇺🇸) noted **potential to refer to cost-effectiveness reports in other markets** for both types of therapies to confirm outcomes of internal cost-effectiveness/cost-utility evaluations for novel therapies

[a]Average across all markets.
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- Payers across markets stated that outcomes-based agreements and sales caps were more attractive and feasible to implement for innovative high-cost therapies versus annuity payments (moderate priority), "Netflix" models, or indication-based discounts (low priority) (**Table 5**)

Table 5. Innovative access approaches for traditional versus innovative high-cost therapies

	Innovative access approach	Anticipated difference in use for traditional vs. high-cost treatment[a]	Additional details
Increasing Level of Difference ↑	Outcomes-based agreements	*High*	• Payers across markets noted that **outcomes-based agreements** were more attractive for innovative high-cost therapies to mitigate the risk of **high upfront cost** and long-term risks due to **uncertainty in durability of efficacy**
Increasing Level of Difference ↑	Sales cap	*High*	• Some payers (🇭🇷🇨🇿🇩🇪) stated that **sales caps** were more attractive for innovative high-cost therapies due to the potential to mitigate **high anticipated budget impact** • However, one payer (🇺🇸) noted that it was **more challenging to negotiate sales caps** for innovative high-cost therapies due to budget restrictions that may require sales caps to be below the level required for commercial viability
Increasing Level of Difference ↑	Annuity payments	*Moderate*	• Some payers (🇭🇷🇨🇿🇪🇬🇮🇹🇬🇧) stated that **annuity payments** were more attractive and feasible to implement for innovative high-cost therapies due to the ability to mitigate **high upfront cost** and **smaller relative size of the anticipated patient population**, respectively
Increasing Level of Difference ↑	"Netflix" model	*Low*	• Payers stated that the "Netflix" model was similarly attractive and feasible to implement across therapy types, and sometimes noted that **changes to legal frameworks** would be required for implementation (🇨🇿🇪🇬🇺🇸) • However, one payer (🇮🇹) stated that the **"Netflix" model** was more attractive for traditional therapies based on the perception that the approach was more suitable for therapies treating **larger volumes of patients**
Increasing Level of Difference ↑	Indication-based discounts	*Low*	• Payers state the attractiveness and feasibility to implement indication-based discounts **was similar across therapy types**, and often noted anticipated **administrative efforts** required to link discounts to specific indications

[a]Average across all markets.
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Limitations

- The analysis included the perspectives of only 15 participants (six in Phase 1, nine in Phase 2); further analyses will aim to expand to more markets and more participants

Conclusions

- While more mature health care systems used a standardized pricing process, emerging health care systems were more flexible and relied on informal price referencing to optimize prices relative to other markets
- Payers across markets placed a great emphasis on clinical value assessments when setting list and net prices for innovative high-cost therapies versus traditional therapies to justify the higher price per patient, reinforcing the importance of ensuring value
- Payers acknowledged the need to offset the potential impact of high upfront costs. Payers also noted that outcomes-based agreements and sales caps were more attractive to implement for innovative high-cost therapies to manage budget impact and/or mitigate concerns of durability of efficacy
- There are a growing number of innovative high-cost therapies seeking reimbursement, which may increase constraints on health care budgets and shift payer price setting approaches
  - Payers may have a greater likelihood of using more informal methods, such as informal competitor benchmarking and non-conventional external price referencing when evaluating list or net prices
  - Payers may request more innovative access approaches during pricing and reimbursement negotiations, including annuity payments, indication-based discounts, and outcomes-based agreements

References

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Abbreviations

AR, Argentina; BE, Belgium; BR, Brazil; CR, Croatia; CZ, Czech Republic; DE, Germany; ES, Spain; EG, Egypt; FR, France; GR, Greece; ICER, Institute for Clinical and Economic Review; ISPOR, The Professional Society of Health Economics and Outcomes Research; IT, Italy; MY, Malaysia; PL, Poland; TH, Thailand; TR, Turkey; UK, United Kingdom.

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