U.S. drug pricing and value assessment lessons from global health technology assessment systems

ISPOR Panel – Research Summary

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Policy context and objective of today's panel

Policy context

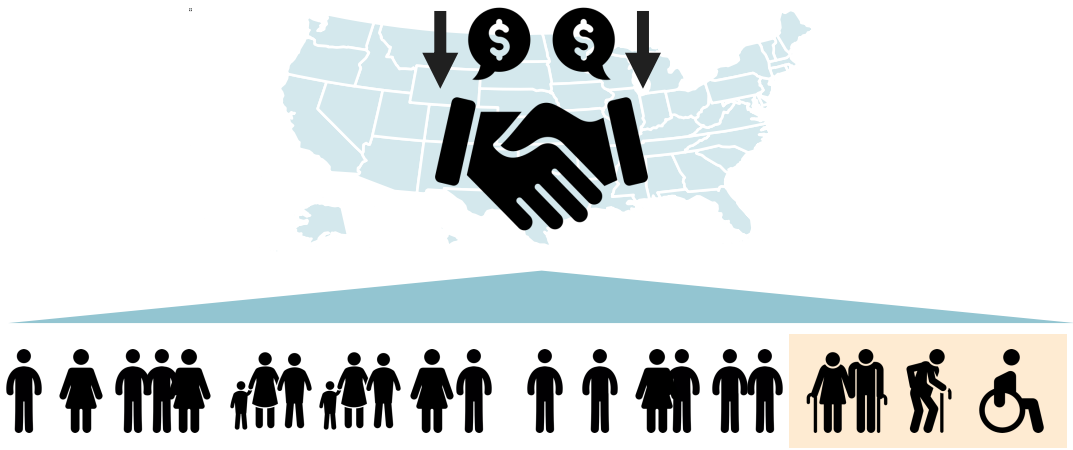
- The Medicare **Drug Price Negotiation Program (DPNP) is not well aligned** with common practices applied by **Health Technology Assessment (HTA)** organizations globally
- Countries that use **HTA-based systems** to determine medicine prices often have access to fewer medicines, even when grounded in established methods

Panel objective

- **Review the key takeaways** from policy assessment of international HTA systems
- **Compare HTA practices** with CMS' guidance for the DPNP
- **Discuss the lessons learned** and how the application of the DPNP can be improved

The DPNP focuses on targeted US drug price reductions, while ex-US systems often conduct comprehensive evaluations

Drug Price Negotiation Program (DPNP)



Medicare beneficiaries

Scope: US Medicare beneficiaries

Objective: Lower the prices of brand name high-cost drugs without generic or biosimilar competition within Medicare

Methodology: Direct price-setting and imposed price ceilings with drug manufacturers to establish a Maximum Fair Price

Ex-US National and/or Regional Systems

All patients within national and/or regional systems

Scope: Global (many countries reference or use HTA for pricing and or access)

Objective: Evaluate the overall value and effectiveness of healthcare technologies, typically near launch

Methodology: Evaluations typically consider a wide range of data to make recommendations regarding coverage

While ex-US systems have issues, DPNP can learn from their successes and missteps

Sources available in slide notes

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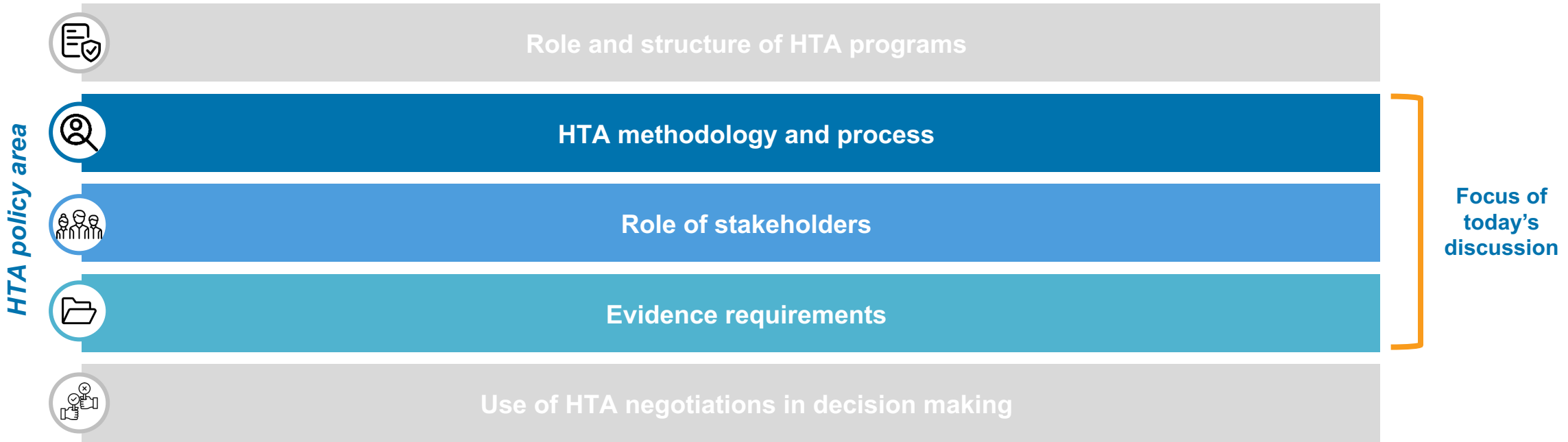
The research objective was to assess international HTA systems and compare approaches to CMS' DPNP

Geographic scope

Australia | Canada | England | France | Germany | Italy | Japan | South Korea

We used the below policy framework to assess HTA systems



Abbreviations: CMS = Centers for Medicare & Medicaid Services; DPNP = drug price negotiation program; HTA = health technology assessment; HCP = healthcare provider

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Selected cross-country assessment learnings include the need for transparency and meaningful stakeholder involvement

Learnings	Detailed findings
The importance of transparency	• Public availability of methodologies and rationale for decision-making plays a **key role in improving public trust and confidence** • Several countries utilize agencies without political influence to conduct assessments; these bodies typically have significant expertise in HTA and can **increase confidence in the assessment process**
The need for stakeholder involvement	• Providing stakeholders including **patients, clinicians, and manufacturers with engagement opportunities** throughout the assessment process, enables them to provide meaningful input
Role of evidence & data flexibility	• There is considerable **variability across systems in the flexibility adopted towards evidence requirements and data that is accepted** particularly as it relates to post market evidence or sub-populations
The impact of HTA process	• **Access to innovative medicines is a consistent challenge** in any price setting system with the overall availability of innovative medicines comparatively lower relative to the US

Abbreviations: HTA = health technology assessment; IQWiG = Insitute for Quality and Efficiency in Health Care; NICE = National Institute for Health and Care Excellence

Across the assessment we find significant variation across HTA policy areas

Policy Area	Potential Learnings	Potential Pitfalls
The importance of transparency	• In a number of countries, HTA bodies publish their assessment methodology and rationale for decision-making	• In South Korea, the final decisions by NHIS and the actual threshold values used are not shared publicly, undermining the transparency and predictability of the process
The need for stakeholder involvement	• In France, patient representatives have a formal role in decision-making, with published rationale as to how patient input contributed to the process	• In several countries, there is a clear absence of opportunities for stakeholder involvement for both patient groups and industry
Role of evidence & data flexibility	• Across countries, there can be considerable flexibility and clarity in the types of evidence that are accepted such as data from non-randomized studies – patient input on evidence is sometimes considered during the process	• In Germany, there are strict evidence requirements that limit flexibility and acceptance of novel date types or endpoints
The impact of HTA process	• In several countries, the use of novel payment models can mitigate evidence uncertainties and support access	• Across multiple countries, there is a significant negative impact on access - both in the number of innovative treatments that are available and the time taken for patients to access them

Abbreviations: HTA = health technology assessment; NICE = National Institute for Health and Care Excellence

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CMS process to establish Maximum Fair Price in IRA

Government publishes a list of selected drugs

- Selected from among the biggest in Medicare
- Occurs 7 or 11 years into the lifecycle, implementation two years later

Considers factors to affect price

- Clinical value and comparative effectiveness
- Investment in research and development, including federal funds
- Unmet medical needs addressed
- Discounts, rebates
- Revenues, units
- FDA approvals, patent information

"Offers" price to drug company

- Includes an explanation for the price "offer"
- Manufacturer may counter-offer if CMS rejects, manufacturer may:

1) accept the price;
2) drop out of Medicare and Medicaid (all of their products)
3) pay excise tax that rises to 1,900% of the selected drug's sales

Publishes the maximum price

- Price remains set until re-setting is triggered
- Ages into new category
- New indication or evidence
- Material change to price setting factors

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We identified areas of improvement for CMS to ensure DPNP implementation does not hinder patient access and innovation

Lack of transparency

Methodology lacks clarity, leaving manufacturers uncertain relative value of evidence in pricing

Limited engagement

Limited involvement of external stakeholders with an unclear bearing on decisions

Generalization

Inflexibility in evidence standards and shortcuts such as ignoring R&D costs from prior NDA holder

Lack of appeal mechanisms

No processes in place for appealing decisions, leaving no recourse

Limited recognition

Minimal incentives or premiums for innovation, discouraging drug development

Cost to patients

Price setting does not necessarily reduce patient cost burden meaningfully

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We leveraged the cross-country assessment learnings to develop considerations for CMS (1/2)

Assessment Learnings	CMS Considerations
The need for a transparent process and clear guidelines that are regularly reviewed **Exemptions and considerations for specialty products e.g. rare diseases** **Risk of inappropriate approaches and measures**	• CMS must **ensure that the MFP is transparent and free from politicization** • Furthermore, **this process should be regularly reviewed** (with stakeholder input) to allow for improvements • The CMS's current policy on orphan drug exemptions is insufficient and **broader exemption that covers all orphan drugs is needed** • CMS should ensure that the **methods it uses to determine prices are rigorous, patient-centered** and reflect up-to-date approaches to capturing a medicine's clinical benefit
Formal opportunities for patients, industry and clinicians throughout the assessment process **Established mechanisms in place to appeal decisions**	• CMS should **provide more opportunities for stakeholders to provide meaningful input during the DPNP process** • **Manufacturers should be able to challenge the MFP process** and address errors or disputes that arise during it

Abbreviations: DPNP = drug price negotiation program; HTA = health technology assessment; QALY = quality-adjusted life year; MFP = maximum fair price

We leveraged the cross-country assessment learnings to develop considerations for CMS (2/2)

Assessment Takeaways	CMS Considerations
Clarity on sources and data types and their use	• CMS should ensure that there is sufficient **clarity around the evidence that it will consider as part of the MFP process** • This should include a **better understanding of the types of evidence and data sources accepted**, cost and clinical outcomes considered, and how individual factors will be weighted
Flexibility in the data that is accepted	
Clear decision-making process on how price is determined	
Use of flexible pricing approaches	• CMS should leverage **a clear and transparent approach for determining the maximum fair price**
Scope to increase prices for new indications	• CMS should ensure that **considerable weighting is given to the importance of innovation-based criteria** such as unmet need and availability of alternative treatment options
Importance of considering the value of innovation in decision-making	• The **focus on cost-containment has had significant impacts on access in all countries**, both in the number of innovative treatments that are available and the time to taken for patients to access them • There are concerns that DPNP price setting may not lead to lower out of pocket costs and utilization management – **CMS needs to monitor formulary coverage of selected drugs and competitors**
Negative impact of cost-containment focus on access and risk of high cost-sharing burden on patients	

Abbreviations: DPNP = drug price negotiation program; HTA = health technology assessment; MFP = maximum fair price

We will now move to the panel discussion

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