OHE

APPENDICES

EXPERT CONSENSUS PROGRAMME:

Payment models for multi-indication therapies

CONSULTING | REPORT
NOVEMBER 2021

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OHE has developed this report commissioned and funded by AstraZeneca

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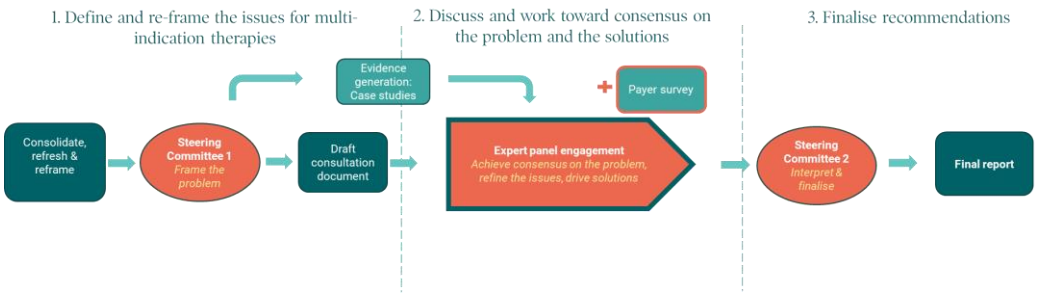
Appendix 1: Expert Panel process and framework of interaction

Between January and July 2021, the project's panel of experts were convened through three main phases, outlined in the figure below and described in more detail underneath.



Phase 1: Define & re-frame the issues for multi-indication therapies. January – March 2021

- Consolidation and re-framing of the relevant literature and issues in the form of a "Where are we now" report, shared with the steering committee as a pre-read for a virtual meeting
- Feedback was sought on the "Where are we now" report and initial case study suggestions via the Within3 platform
- Live two-hour virtual meeting with the steering committee to discuss the resources shared to date, and plans for the engagement plans and line of questioning for the broader Expert Panel
- Revision of the "Where are we now" report into a draft consultation document.

Phase 2: Discuss and work toward consensus on the problem and the solutions (Expert Panel engagement: further details to follow in section 2): April – May 2021

- Expert Panel engagement (further details to follow) through:
    - Pre-read draft consultation document and pre-meeting survey
    - Live two-hour virtual meeting
    - Two-week asynchronous meeting using the Within3 platform
- Separate payer survey

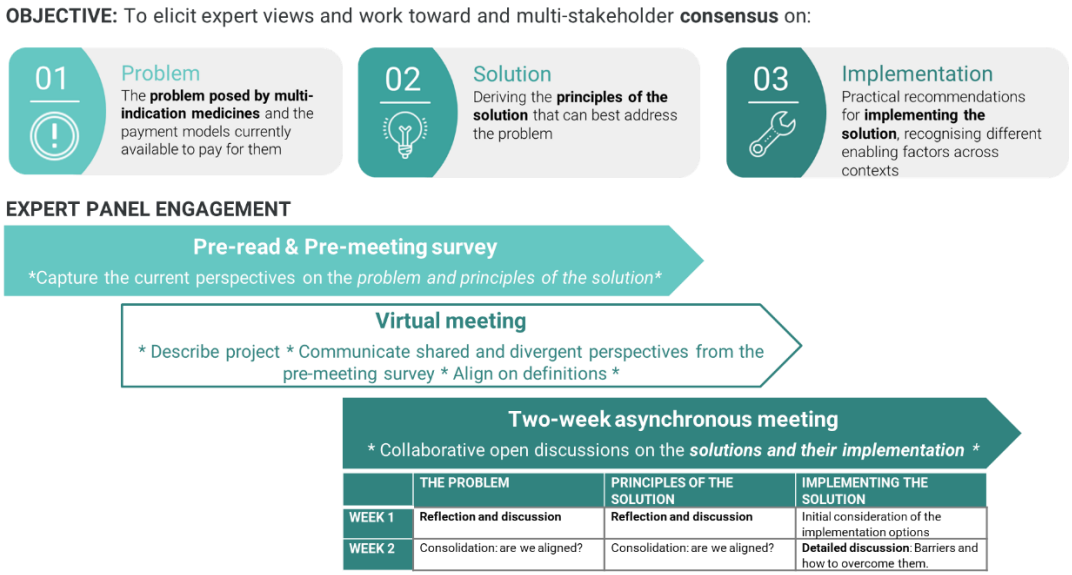
Phase 3: Finalise recommendations. June – July 2021

- Final (two-hour) virtual meeting of the steering committee to discuss and interpret the main findings from the Expert Panel engagement.

- Develop report summarising key results and recommendations, with the review and input of the project steering committee.

The primary objective of the project was to elicit the views of a broad range of stakeholders and work toward consensus on the problem, the principles of the solution, and practical recommendations on implementation. We adopted an adapted Delphi methodology, outlined briefly in the Figure below.

FRAMEWORK OF EXPERT ENGAGEMENT: OBJECTIVES AND METHODS



Before the first virtual meeting, the Expert Panel members read the draft consultation report and were asked to individually complete a pre-meeting survey on the problem and principles of the solution. The results of that survey were played back to the group during the virtual meeting, which was used as an opportunity to *align* on definitions, communicated shared and divergent perspectives, and introduce the topics to be covered in the two-week asynchronous meeting.

Consistent with the Delphi methodology, we used the three phases of engagement as an opportunity to (a) receive individual feedback from each panellist on the relevant topics [pre-meeting survey], (b) play back the overall results and insights to the whole group [virtual meeting], and (c) facilitate a discussion by re-visiting the topics as a group to reflect on commonalities and differences [two-week asynchronous meeting]. The goal is to reduce the range of responses and arrive at something closer to consensus on the key benefits, challenges and proposed policy advancements that gained the largest consensus in the Delphi process.

The two-week asynchronous meeting took place between May 5th and May 19th 2021on the Within3 platform. This meant that Panellists could read the comments and discussions of fellow panellists and participate in those discussions at a time that suited them throughout the two-week window. Questions for discussion, which comprised a mix of multiple choice and open questions, matched the 'Objective' themes outlined in the Figure and were rolled-out in two phases. During the first week, the focus was on the principles of the problem and solution: playing back the previous discussions and themes from the pre-meeting survey and virtual meeting, and facilitating an interactive discussion of those. The focus of the second week was on the implementation of payment models to better recognise value by indication.

All 16 Expert Panel members participated in the pre-meeting survey, which generated 78 pages of content which the project team digested and summarised back to the Expert Panel in the virtual meeting in a graphical and summary anecdotal format. The virtual meeting itself was mainly an opportunity to align on the project objective and framework, report commonalities and differences of opinion, and to amend the language used in order that the whole Panel could discuss the issues on the basis of a shared understanding of the concepts.

During the two-week asynchronous meeting there were 31 discussion items, over which Expert Panellists made a total of 620 individual contributions (amounting to 183 A4 pages of transcript content).

The pre-meeting survey questions and asynchronous meeting questions are presented below.

Expert Panel pre-meeting survey questions

Z4-31965
Date of preparation: 26.03.2021

Introductory questions

	Question Text	Question Details	Question Type	Rating/MC Question Choices & Format
1	Please introduce yourself to your fellow Expert Panel members, and let us know: Your name & institution, and your expertise or experience relevant to this topic. (Feel free to upload your introduction via a short video instead)		Open ended	Comment box (required)
2	Drop a pin on the map to tell us where you are, along with an interesting fact about your location!		Open ended	Comment box (required)

Survey questions relating to the "Where are we now?" report

	Question Text	Question Details	Question Type	Rating/MC Question Choices & Format
1	Do you agree with the objectives of pharmaceutical pricing described in section 2 of the "Where are we now?" report?	Please rate each objective based on your level of agreement/ disagreement.	Rating + open-ended	Rating scale - Yes/no/unsure Rating options: - Maximising access for patients. - Optimising incentives for innovation (development and launch of new

				indications). - Protecting the financial sustainability of payers. - Increasing value for money and competition. - Encourage monitoring and evaluations of results. - Encourage appropriate/ rational use of medicines, avoiding over- and/or inappropriate use. - Ensure transparent process, reducing complexity, bureaucracy and duplication. - Limit the negative impact of one country's pricing and reimbursement system on the access and prices of medicines in other countries Comment box (required) -Please provide a brief explanation of the ratings assigned
2	Are any important objectives of pharmaceutical pricing missing in the description of the "Where are we now?" report (section 2)?		Multiple choice + open-ended	Radio buttons - Yes - No Comment box (required) - If yes, please provide an explanation of the additional objectives and how they are complementary to those already described in the report.
3	Do you think there are trade-offs between the multiple objectives of pharmaceutical pricing? If so, what are they?	Please refer again to the objectives described in section 2 of the "Where are we now?" report.	Open ended	Comment box (required)
4	Do you think uniform pricing per unit of medicine poses any problems in the case of drugs with multiple indications?		Multiple choice + open-ended	Radio button -Yes -No Comment box (required) Please briefly explain your selection. If yes, what are the most obvious symptoms or examples?

5	In the case of drugs with multiple indications, which objectives may not be successfully achieved under uniform pricing per unit of medicine?	Please select all that apply.	Multiple choice + open-ended	Checkboxes: - Maximising access for patients. - Optimising long-term incentives for innovation. - Protecting the financial sustainability of payers. - Increasing value for money and competition. - Encourage monitoring and evaluations of results. - Encourage appropriate/ rational use of medicines, avoiding over- and/or inappropriate use. - Ensure transparent process, reducing complexity, bureaucracy and duplication. - Limit the negative impact of one country's pricing and reimbursement system on the access and prices of medicines in other countries Comment box (required) - Please briefly explain your selection and provide supporting real-world examples.
6	Do you think value-based differential pricing provides a solution to the problems of uniform pricing per unit of medicine, in the case of drugs with multiple indications?	Please consider your answer to question 5 and whether / how value-based differential pricing may solve them.	Multiple choice + comment box	Radio buttons - Yes - No - Sometimes Comment box (required) If your answer was 'yes' please detail the specific circumstances where you think value-based differential pricing provides a solution, providing real-world examples if possible. If your answer was 'no' or 'sometimes' please provide some ideas of how value-based differential pricing should be adapted provide a better solution, providing real-world examples if possible.

7	What do you think are the greatest advantages and/or disadvantages of value-based differential pricing compared to uniform pricing per unit of medicine?		Open ended	Comment box (required)
8	In the "Where Are We Now Report" we described the potential **short-term** effects of value-based differential pricing (such as expanded patient access and overall increase in health care spend), and **long-term** effects (such as optimised incentives for R&D and reduced pressure on payer's affordability through competition). Do you agree with this description?	Please rate each effect based on your level of agreement/ disagreement.	Rating + open-ended	Rating scale - Yes/no/unsure Rating options: - Expanded patient access - Increase in overall health care spending (albeit on cost-effective treatments) - Optimised R&D incentives - Reduced pressure on payers' affordability via increased competition Comment box (required) - If known, please provide real-world examples in support of the ratings assigned.

9	Do you think value-based differential pricing has other effects in addition to those described in section 3 of the "Where are we now?" report?		Multiple choice + open-ended	Radio buttons - Yes - No Comment box (required) - If yes, please provide an explanation of the effects, how they would be complementary to those already described in the report and whether they would be positive or negative.
10	Why do you think value-based differential pricing has not been broadly adopted to date?		Open ended	Comment box (required)
11	What evidence do you think is needed in order to move the conversation forwards?		Open ended	Comment box (required)
12	Is there anything else you would like to tell us?		Open ended	Comment box (required)

Expert Panel asynchronous meeting structure

Date of preparation: 22.04.21
Z4-32668

Expert Consensus Programme on Payment Models for Multi-Indication Therapies: Asynchronous Meeting

Welcome to the virtual platform. As part of this two-week asynchronous meeting, we will work together towards achieving consensus on the challenges raised by payment models for multi-indication medicines, as well as the solutions which could promote better patient outcomes and sustainable health care and innovation.

The asynchronous meeting will use a mix of multiple choice and open questions to guide a collaborative and open discussion on: **the problem** posed by multi-indication medicines and the payment models currently available to pay for them, **the principles of the solution** that can best address the problem, and practical recommendations for **implementing the solution**, recognising different enabling factors across contexts.

The discussion questions will be released on this platform in two consecutive rollouts:

- The **1st Rollout** will launch on 5th May and it will be live until midnight BST on 12th May.
- The **2nd Rollout** will launch on 13th May and will be live until midnight BST on 19th May.

The Expert Panel members are encouraged to maximise the opportunities for a collective discussion by posting personal replies to the platform's questions and reacting to comments from their peers. The Expert Panel is also invited to reflect on to the results of the preliminary engagement activities, available through:

- Slides summarising the results of the pre-meeting survey (posted as relevant alongside the questions)
- Kick-off call meeting slides from 5th May

Additional insights that the Expert Panel may wish to refer to during the asynchronous meeting, available under the "Resources" on the right, are the:

- "Where are we now?" (WAWN) report
- Case studies – new!
- Payer survey [Interim results] *(to be released in week 2)*

Through the *case studies*, our aim is to articulate and generate the evidence needed to work toward consensus on the main issues and uncertainties associated with payment models for multi-indication therapies. The first two case studies are topical for week 1, where we discuss the *principles of the solution. The third case study is topical for week 2: implementing the solution.*

A summary of the topics to be covered in week 1 and week 2 is provided below. Highlighted in bold is the main focus of the weeks' discussion items:

	THE PROBLEM	PRINCIPLES OF THE SOLUTION	IMPLEMENTING THE SOLUTION
WEEK 1	Reflection and discussion	Reflection and discussion *Case studies 1 & 2*	Initial consideration of the implementation options
WEEK 2	Consolidation: are we aligned?	Consolidation: are we aligned? *Reflections on Payer survey [interim results]*	Detailed discussion: Barriers and how to overcome them. *Case study 3*

Questions for rollout 1 (5TH – 12TH MAY)

Problem

	Question Text	Question Details	Question Type	Rating/MC Question Choices & Format
1	Please discuss whether the survey results capture a comprehensive and balanced representation of the most important objectives and principles of pharmaceutical pricing.	*To include:* • *Slide 1* • *Slide 2*	Open question	Comment box
2	Please discuss whether payment models that do not recognise the value of individual indications (or approved uses) of a drug create problems with respect to the most important objectives of pharmaceutical pricing.	Please consider the views emerged in the previous discussion and the survey results. *To include:* • *Slide 3* • *Slide 4*	Open question	Comment box

Principles of the solution

	Question Text	Question Details	Question Type	Rating/MC Question Choices & Format
1	Is value-based differential pricing the right term to capture the concept of recognising indication-level value in payment models?		Multiple choice + open question	Radio box: -Yes -No Comment box -If your response was no, please provide your suggestion for an alternative label.
2	Please discuss your view on the potential opportunities offered by value-based differential pricing to solve the problems created by payment models that do not recognise the value of individual indications (or approved uses) of a drug.	Please consider the discussion of the problem so far, and the survey results. *To include:* • *Slide 5* • *Slide 6* • *Slide 7* Value-based differential pricing is hereby defined as any payment model that recognises and rewards the value of individual indications, or approved uses, of a drug.	Open question	Comment box
3	Please rate and discuss the level of importance of each **general barrier** to the acceptance of value-based differential pricing as a concept	*To include:* • *Slide 8*	Rating + open question	Rating: -High -Moderate -Low Options to rank (obtained from survey results): • Payers' inertia and/ or scepticism • Legislative framework • Inadequate data infrastructure and/or data governance issues

				• Increasingly complex negotiation and reimbursement processes • Lack of public understanding and awareness • Lack of a conceptual framework to determine/demonstrate benefits and suitability of use Comment box - What would be required to progress the acceptance of value-based differential pricing as a solution? Are you aware of any supporting real-world examples?

Principles of the solution: *Case studies*
Case study 1: Demonstrating improved outcomes for patients and health systems

Through the *case studies*, our aim is to articulate and generate the evidence needed to work toward consensus on the main issues and uncertainties associated with payment models for multi-indication therapies. The first case study is topical for our current discussion of the *principles of the solution.* Please review case study 1, and leave your comments against the questions below.
Resource link: case study 1

	Question Text	**Question Details**	**Question Type**	**Rating/MC Question Choices & Format**
1	What may be the best sources of information to capture comparable data on *indication coverage* and *speed of access* to multi-indication therapies, across countries?		Open question	Comment box
2	Do you agree with the comparator groups and proposed examples? Recognising the complexity of factors affecting breadth and speed of access, is it			

	meaningful to compare countries in this way?			
3	Group 2 contains many countries that can be considered 'priority' markets for several reasons, which means we may expect number of indications available to be higher. If we consider the main factors determining the country launch order for new indications: *Is flexible pricing one of those? Can adopting enhanced price flexibility change a country's "position" on the list (e.g. for a small market?) How can we best evidence this?*		Open question	Comment box
4	Alternative approaches: At the within-country level, is it possible to observe **realised impact** on healthcare systems of introducing price flexibility, e.g. on overall availability of medicines across indications, time to treatment access, impact on budgets etc.? Please share any observations, suggestions, or resources to support your answer.	*In particular, we welcome expert insight into the realised impact of introducing VBDP in Belgium, Estonia and Italy.*	Open question	Comment box
5	Do you have any other comments on this case study, or alternative suggestions for		Open question	Comment box

	demonstrating improved outcomes for patients and health systems?			

Case study 2: Protecting financial sustainability for payers

Through the *case studies*, our aim is to articulate and generate the evidence needed to work toward consensus on the main issues and uncertainties associated with payment models for multi-indication therapies. The second case study is topical for our current discussion of the *principles of the solution.* Please review case study 2, and leave your comments against the questions below.

Resource link: case study 2

	Question Text	Question Details	Question Type	Rating/MC Question Choices & Format
1	Is this a useful exercise in demonstrating the relationship between payment models and R&D incentives? Should any elaborations or changes to the assumptions be made?		Open question	Comment box
2	Do you agree that VBDP is compatible with financial sustainability for payers?		Open question	Comment box
3	Can any real-life evidence or experience be brought to bear on: - Indications not pursued due to sub-optimal incentives within current payment models?		Open question	Comment box
4	Can any real-life evidence or experience be brought to bear on: - Increased price flexibility at the indication-level leading		Open question	Comment box

	to smarter payer procurement?			

Implementation of the solution

In the following questions we ask you about the potential positive and negative effects of each payment model type, as well as enabling factors. Note that specific discussion of the *barriers* to each (as well as how to overcome them) is not requested at this point, as these will be a key focus of next week's rollout of questions where we focus on implementation.

	Question Text	**Question Details**	**Question Type**	**Rating/MC Question Choices & Format**
1	Is the taxonomy of payment models for drugs with multiple indications described in section 4 of the WAWN report comprehensive?	Our taxonomy*:* • Different brand names (or delivery/dosage forms) for each indication, or different list prices for each indication of the drug • Single list price and discount levels (applied upfront) or rebates (applied ex-post) that vary by indication and could be confidential • An average weighted price or a "blended" price reflecting the prices appropriate to the different indications, and the volumes associated with each indication • Single list price with confidential discounts or rebates permitted based on sale volumes (rather than indication value)	Multiple question + open box	Rating options -Yes/ no Comment box - If not, what other approaches are known to be used to price drugs with multiple indications?

		[note: as this model does not recognise value at the indication-level, we have not included this in subsequent questions on solutions / implementation]		
2	Considering **different brand names (or delivery/dosage forms) for each indication, or different list prices for each indication**, please discuss the adequacy of this payment model to price drugs with multiple indications, in terms of the expected ***positive and negative effects*** of its use, and the factors or circumstances needed to enable/prevent them.	Note that whilst some examples of different brand names exist, it is generally reserved for reasons of safety, and may not normally be practicable for indications that are closely related. However, for completeness, we would like to collect your thoughts on this model.	Open question	Comment box
3	Considering **single list price and discount levels (applied upfront) or rebates (applied ex-post) that vary by indication and could be confidential**, please discuss the adequacy of this payment model to price drugs with multiple indications, in terms of the expected ***positive and negative effects*** of its use, and the factors or		Open question	Comment box

	circumstances needed to enable/prevent them.			
4	Considering **an average weighted price or a "blended" price reflecting the prices appropriate to the different indications, and the volumes associated with each indication**, please discuss the adequacy of this payment model to price drugs with multiple indications, in terms of the expected ***positive and negative effects*** of its use, and the factors or circumstances needed to enable/prevent them.		Open question	Comment box

ROLLOUT 2: 13TH – 19TH MAY

Problem

	Question Text	Question Details	Question Type	Rating/MC Question Choices & Format
1	Based on the discussion so far, how well-aligned do you think are the expert panel's views on the nature of the problem caused by payment models that do not recognise the value of individual indications (or approved uses) of a drug?		Open question	Comment box

Principles of the solution

	Question Text	Question Details	Question Type	Rating/MC Question Choices & Format
1	Based on the discussion so far, how well-aligned do you think are the expert panel's views on the solutions for drugs with multiple indications? What should be the key principles underpinning the solution?		Open question	Comment box

Payer insights

In recognition of the key voice of payers in this discussion, we have carried out a separate survey with (recent former) payers across several countries, to gain a deeper understanding of payer experience and perceptions of payment models that address multi-indication therapies. We have summarised the findings so far here: *[insert link: payer survey results]*

	Question Text	Question Details	Question Type	Rating/MC Question Choices & Format
1	Please review the new material: *'Payer survey [Interim results]'*. Do you have any comments on the summary of findings, which have a bearing on your answers above on the principles of the problem and solutions? What particular issues raised do we need to carry through to our consideration of implementation models?		Open question	Comment box

Implementation

	Question Text	Question Details	Question Type	Rating/MC Question Choices & Format
1	Based on the discussion so far, please discuss the major **barriers** for the successful implementation of: a payment model based on **different brand names (or delivery/dosage forms) for each indication, or different list prices for each indication**.	Please refer to the previous discussion on the enabling factors and circumstances required to realise the positive effects of each payment model, and reflect on their availability in your country of expertise.	Open question	Comment box
2	Overcoming the barriers towards the implementation of: **different brand names (or delivery/dosage forms) for each indication, or different list prices for each indication**: How? Who? When?	Given the nature of the existing barriers, please discuss: • Potential solutions and terms of real-world examples of success stories (How) • The main stakeholders that would be involved in the achievement of solutions (Who) • The time horizon required to achieve solutions (When)	Open question	Comment box
3	Based on the discussion so far, please discuss the major **barriers** for the successful implementation of: a payment model based on **single list price and discount levels (applied**	Please refer to the previous discussion on the enabling factors and circumstances required to realise the positive effects of each payment model, and reflect	Open question	Comment box

	upfront) or rebates (applied ex-post) that vary by indication and could be confidential.	on their availability in your country of expertise.		
4	Overcoming the barriers towards the implementation of: **single list price and discount levels (applied upfront) or rebates (applied ex-post) that vary by indication and could be confidential**: How? Who? When?	Given the nature of the existing barriers, please discuss: • Potential solutions and terms of real-world examples of success stories (How) • The main stakeholders that would be involved in the achievement of solutions (Who) • The time horizon required to achieve solutions (When)	Open question	Comment box
5	Based on the discussion so far, please discuss the major **barriers** for the successful implementation of: a payment model based on **an average weighted price or a "blended" price reflecting the prices appropriate to the different indications, and the volumes associated with each indication**.	Please refer to the previous discussion on the enabling factors and circumstances required to realise the positive effects of each payment model, and reflect on their availability in your country of expertise.	Open question	Comment box
6	Overcoming the barriers towards the implementation of: **an average weighted**	Given the nature of the existing barriers, please discuss:	Open question	Comment box

	price or a "blended" price reflecting the prices appropriate to the different indications, and the volumes associated with each indication: How? Who? When?	• Potential solutions and terms of real-world examples of success stories (How) • The main stakeholders that would be involved in the achievement of solutions (Who) • The time horizon required to achieve solutions (When)		
7	Are there any alternative implementation models – or characteristics thereof – which should be considered and have not been captured in our discussions so far?		Open question	Comment box

Implementation: *Case study*

Through the *case studies*, our aim is to articulate and generate the evidence needed to work toward consensus on the main issues and uncertainties associated with payment models for multi-indication therapies. The final case study is topical for our current discussion of the *implementation.* Please review case study 3, and leave your comments against the questions below.

Case study 3: Tackling implementation

Resource link: case study 3

	Question Text	Question Details	Question Type	Rating/MC Question Choices & Format
1	Is this characterisation of implementation models helpful in considering what is possible given different availability of data?	*Please explain*	Open question	Comment box

2	What are key distinctions in terms of utilising routinely collected (e.g. as used in Estonia) versus dedicated registries (e.g. as used in Italy) data to support VBDP? What is optimal and why?		Open question	Comment box
3	Looking forwards: Based on your experience of VBDP-enabled health systems, how is the system evolving or adapting, and what are the main reasons?		Open question	Comment box

Payer survey

Introduction

In recent years, we have witnessed a proliferation of innovative drugs with multiple approved indications. Each approved indication corresponds to a different "use" of the drug; for example: in different diseases, at different stages of the same disease, at different points of the treatment regimen, or in combination with other therapies.

It is broadly accepted that a medicine's price should be aligned with the value it provides (over alternative treatments) to patients and the health service. This approach, known as value-based pricing, aims to maximise the efficiency of health care investments by rewarding innovation that most benefits patients. Medicines have historically been priced on a per-pill or per-vial basis. In the case of drugs with multiple indications, a single price for a single drug ("uniform price") may fail to align price to differential value across indications. Whilst some healthcare systems offer more flexibility than others, overall there is a risk that the existing pricing practice may undermine patient access to new treatments, and also undermine R&D incentives, thus failing to maximise the total value to the health system. In real terms, this means fewer treatment options for patients. The challenge, therefore, is to ensure the reimbursement landscape provides a facilitative framework to attract and support clinically beneficial treatment options which also offer good value for money.

The ambition of this survey is to gain a deeper understanding of payer experience and perceptions of payment models that address multi-indication therapies, as well as what are the desirable and realistic options to address them.

Section 1: Understanding the problem and potential solutions (*principles*)

A key part of this survey is to understand any *problem(s)* posed by current payment models for medicines serving multiple indications, and how these manifest for payers.

To understand whether there is a problem, we can first consider what pharmaceutical pricing should aim to achieve. Commonly cited objectives are: **maximisation of patient access**, the **optimisation of the incentives for innovation** and the **protection of the financial sustainability of payers**. It is often argued that these objectives are not well achieved for multi-indication drugs, where a single price fails to reflect value across indications (e.g. not offering a return on investment for developing and testing further indications). While it is difficult to observe the counterfactual – that indications may never make it to market due to current price inflexibilities – we may observe some *symptoms* of the problem, e.g.

- *physical differentiation:* same active substance with different brand names [and prices] for different indications; generally for safety reasons and not possible or practical for most drugs whose indications/mode of administration are more closely related;

- o*ff-label use:* this can compromise patient safety as well as budget predictability, and may arise particularly in rare and untreated disorders, where clinical value may be high but an inflexible single price may not make the R&D investment viable;

- *unrealised benefit of existing drugs:* access constrained where new treatment indications are found to be not cost-effective.

In the following questions we use the term "uniform pricing" to refer to a single price applied to a therapy across indications; we define value-based differential pricing as the application of value-based pricing at the indication level, to recognise differences in clinical and/or economic value across indications.

	Question Text	**Question Details**	**Question Type**	**Rating/MC Question Choices & Format**
1	What do you consider to be the most important objectives / considerations for pricing and access models for drugs with multiple indications?	Please rate each objective based on your level of agreement/ disagreement.	Rating + open-ended	Rating scale - Yes/no/unsure Rating options: - Maximising access for patients. - Optimising incentives for innovation (development and launch of new indications). - Protecting the financial sustainability of payers. - Increasing value for money and competition. - Encourage monitoring and evaluations of results. - Encourage appropriate/ rational use of medicines, avoiding over- and/or inappropriate use. - Ensure transparent process, reducing complexity, bureaucracy and duplication. - Limit the negative impact of one country's pricing and reimbursement system on the access and prices of medicines in other countries Comment box

				-Please provide a brief explanation if relevant, or any other factors missing from the list above
2	What are the problems, if any, associated with uniform pricing when it comes to paying for multi-indication therapies?		Open-ended	Comment box (required) Please briefly explain, along with the most obvious symptoms or examples
3	Specifically, in the case of drugs with multiple indications, which objectives may **not** be successfully achieved under uniform pricing per unit of medicine?	Please select all that apply.	Multiple choice + open-ended	Checkboxes: - Maximising access for patients. - Optimising long-term incentives for innovation. - Protecting the financial sustainability of payers. - Increasing value for money and competition. - Encourage monitoring and evaluations of results. - Encourage appropriate/ rational use of medicines, avoiding over- and/or inappropriate use. - Ensure transparent process, reducing complexity, bureaucracy and duplication. - Limit the negative impact of one country's pricing and reimbursement system on the access and prices of medicines in other countries Comment box (required) - Please briefly explain your selection and provide supporting real-world examples.
4	What do you think are / would be the primary effects of value-based differential pricing, compared to uniform pricing?	Please rate each effect	Rating + open-ended	Rating scale (differs by option) Rating options: - Patient access (increased / reduced / unchanged) - Overall health care spending (increased / reduced / unchanged) - R&D incentives (improved / worsened / unchanged) - Competition at the indication-level (enhanced / reduced/ unchanged) Comment box (required)

				- If known, please provide real-world examples in support of the ratings assigned, and comment on any further advantages or disadvantages of value-based differential pricing
5	Why do you think value-based differential pricing is not commonly applied across countries?		Open ended	Comment box (required)

Section 2: What are the solutions for multi-indication therapies in practice?

In this section we would like to hear how reimbursement of multi-indication therapies is addressed in your country, and what an optimal solution might look like.

6	In what ways have payment models adapted, if at all, for multi-indication medicines in your country?		Open ended	Comment box (required)

7	Do you believe that the solution applied in your country is adequate?	Please consider your answer to question 3 and whether / how the approach in your country addresses these problems	Multiple choice + comment box	Radio buttons - Adequate - Not adequate - Unsure Comment box (required) Please explain your answer
8	If you could design the optimal payment model for multi-indication therapies what would it look like? Do you think value-based differential pricing has a role to play (or could be further improved) in your country?		Open ended	Comment box (required)

9	Are there any barriers to your ideal model and, if so, how might they be overcome?		Open ended	Comment box (required)
10	Is there anything else you would like to tell us?		Open ended	Comment box (required)

Appendix 2: Modelling exercise

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SCENARIO

THE DRUG

- Hypothetical drug, with five potential indications over a 10 year time horizon

Timeline represents when the indication would have been launched, if developed

Indication-level value-based price (VBP). i.e. the value-based differential price

	Indication	Launch year	Clinical benefit	Incremental QALYs per patient	Monotherapy / combination	Relative population size	Annual number of patient	Average treatment duration	Total patient treatment months (p.a.)	Total QALYs	Indication value -based price per patient per month	Total VBP
Launch indication	Pancreatic	2018	Strong	0.29	Mono	Small	300	6	1800	87	€ 1,450	€ 8,700
2nd indication	Prostate	2021	Moderate	0.07	Mono	Large	800	4	3200	53	€ 500	€ 2,000
3rd indication	Mantle cell	2022	Very strong	0.34	Mono	Rare	100	6	600	34	€ 1,700	€ 10,200
4th indication	Gastric	2025	Strong	0.23	Combo	Small	200	9	1800	45	€ 750	€ 6,750
5th indication	Breast	2026	Strong	0.26	Mono	Large	1000	7	7000	257	€ 1,100	€ 7,700

Unit of health benefit. For illustrative purposes we assumed the quality adjusted life year (QALY)

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SCENARIO

THE REIMBURSEMENT ENVIRONMENT

1. Uniform Pricing

- **Launch indication.** Price set for launch indication which becomes the uniform price (P_{U1}); access granted for patient cohort (N_1)
- **Subsequent indications.** Prices permitted to fall but not rise.
  - If relatively *higher* value: indication reimbursed at uniform price (P_{U1})
  - If relatively *lower* value: payer only willing to reimburse if discount offered to correspond with (relatively lower) value based price of new indication ($P_{U2}<P_{U1}$). Only one (net) price is permitted, so manufacturer decides whether to develop/launch new candidate indication by comparing:
    - Revenue from current indication ($P_{U1} * N_1$)
    - Revenue from current + new indication ($P_{U2} * N_{1+2}$)

Assumptions
- ✓ Access: Illustrative valuebased HTA environment: threshold €30,0000/QALY
- ✓ Discounts: Must apply across all current indications.

2. Value-based differential pricing (VBDP)

- Price is permitted to vary by indication

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Uniform price

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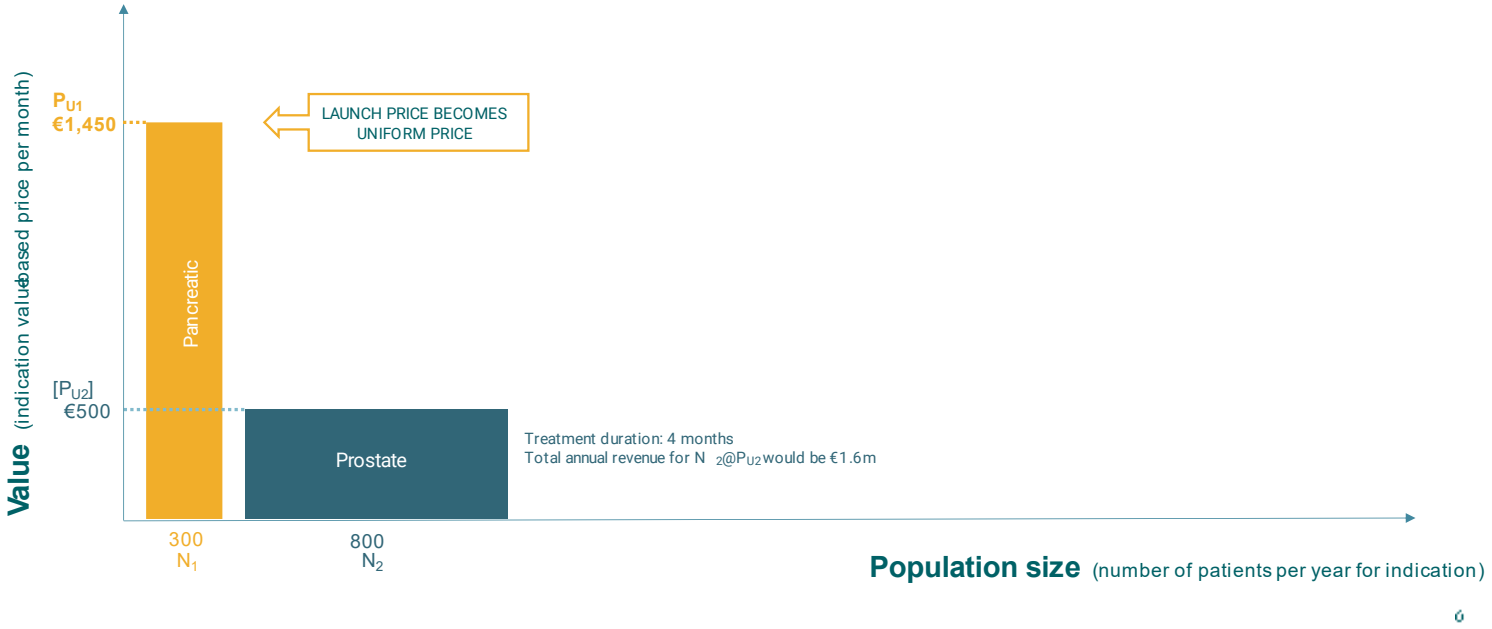
Uniform price



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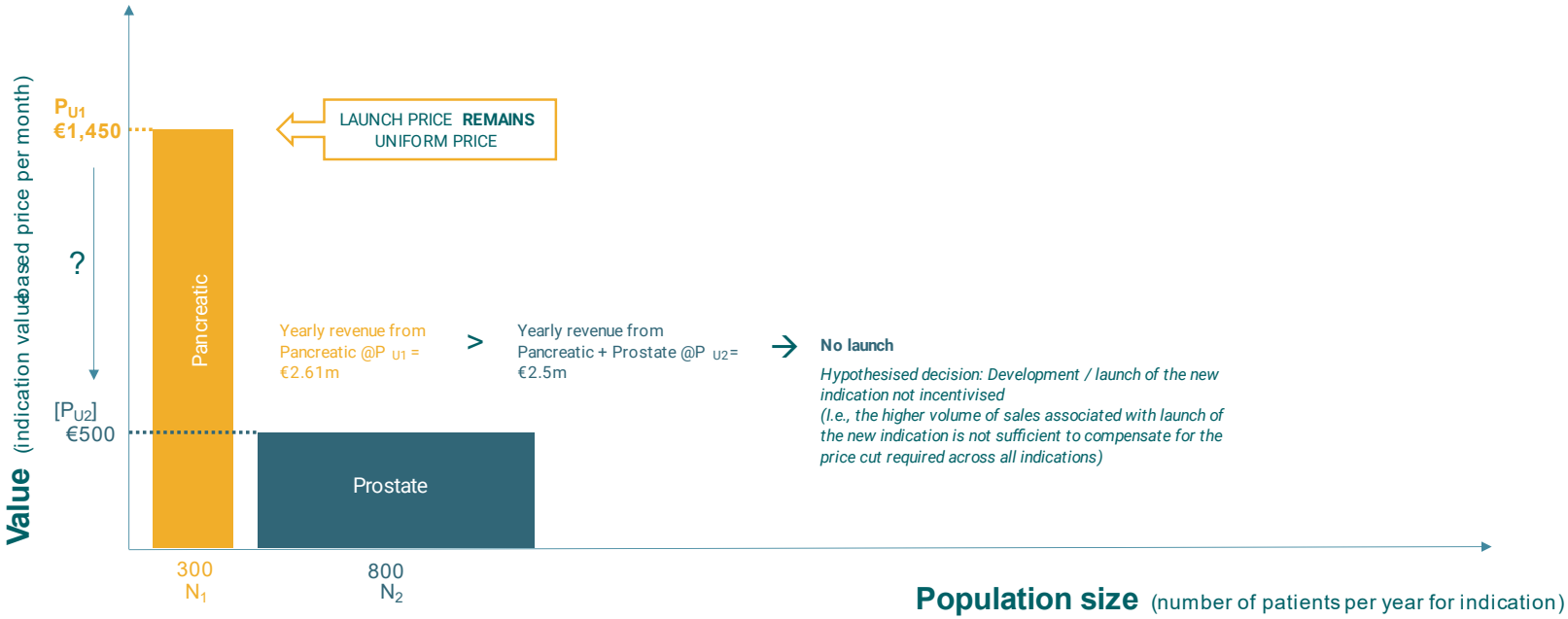
Uniform price



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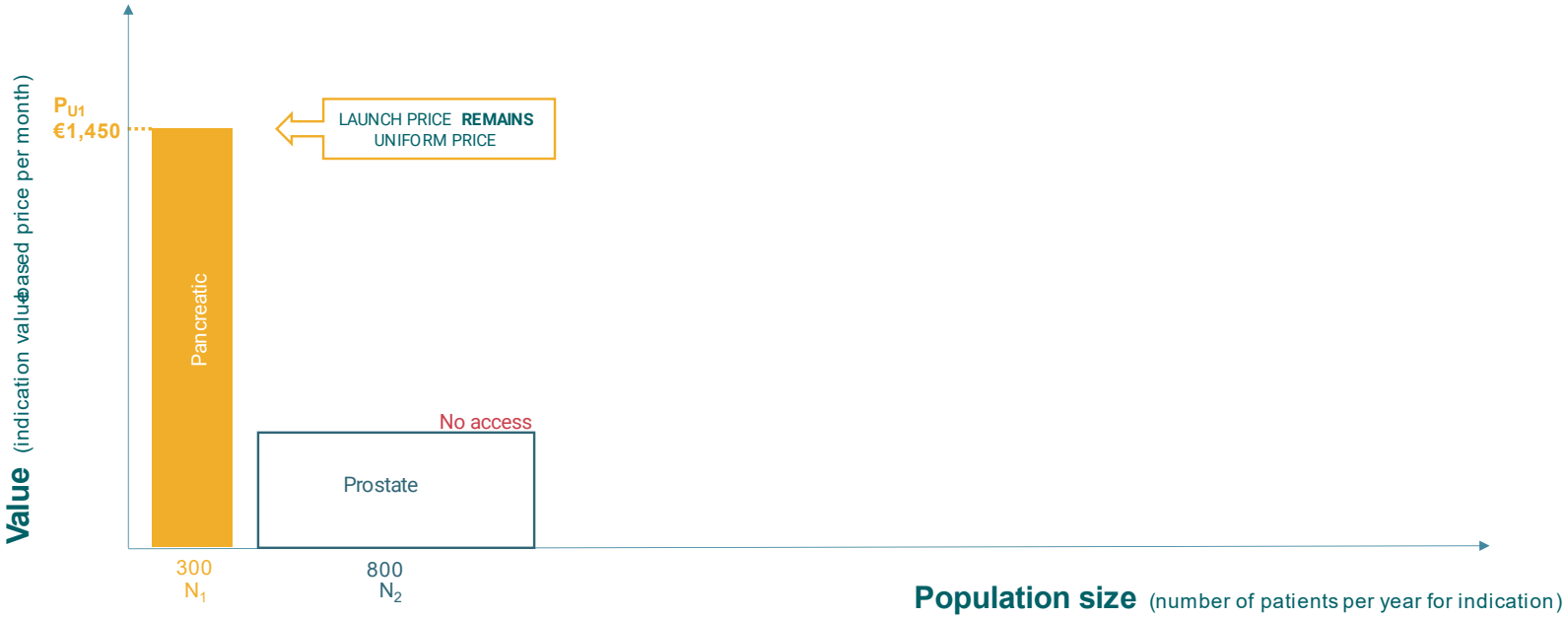
Uniform price



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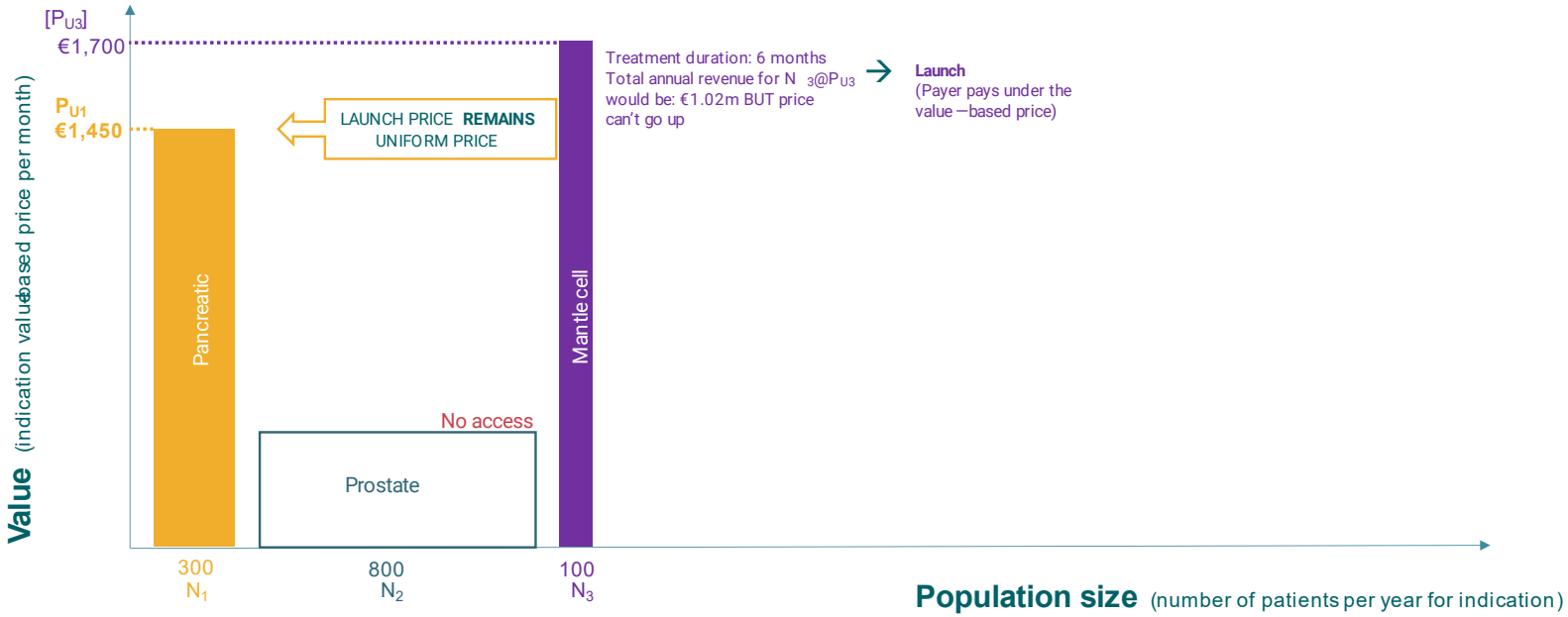

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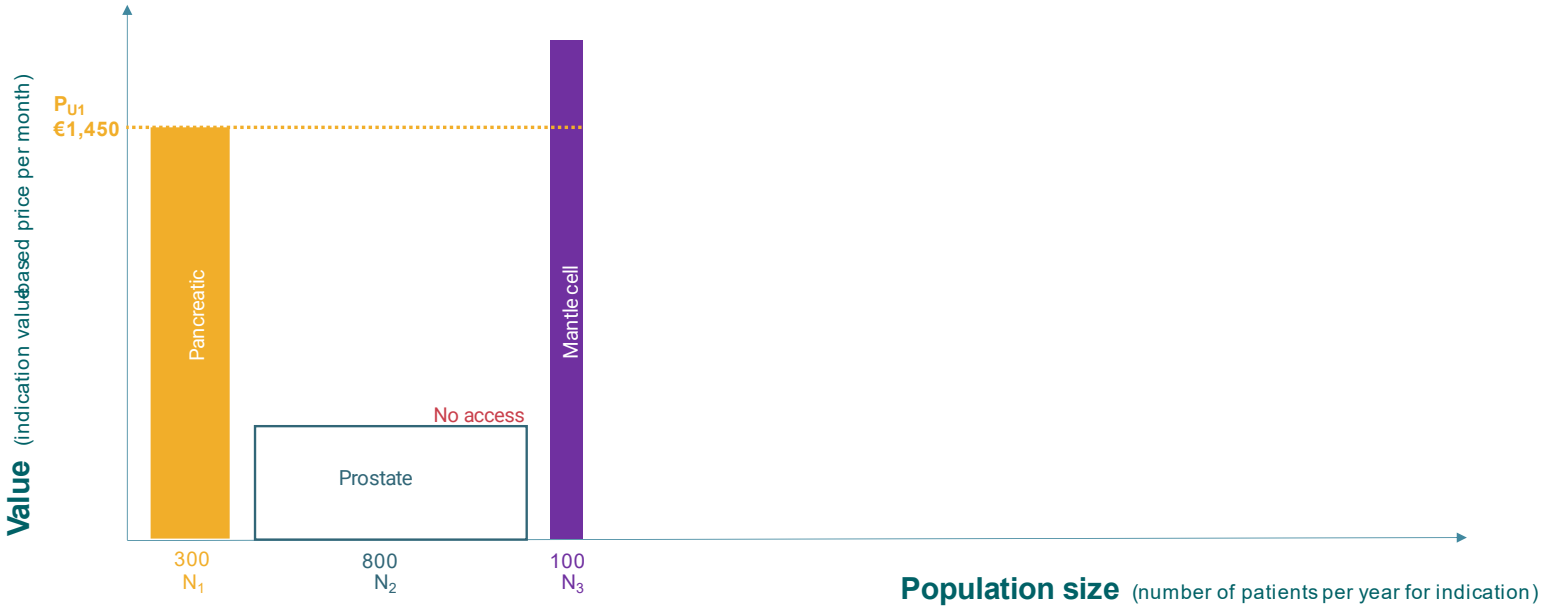

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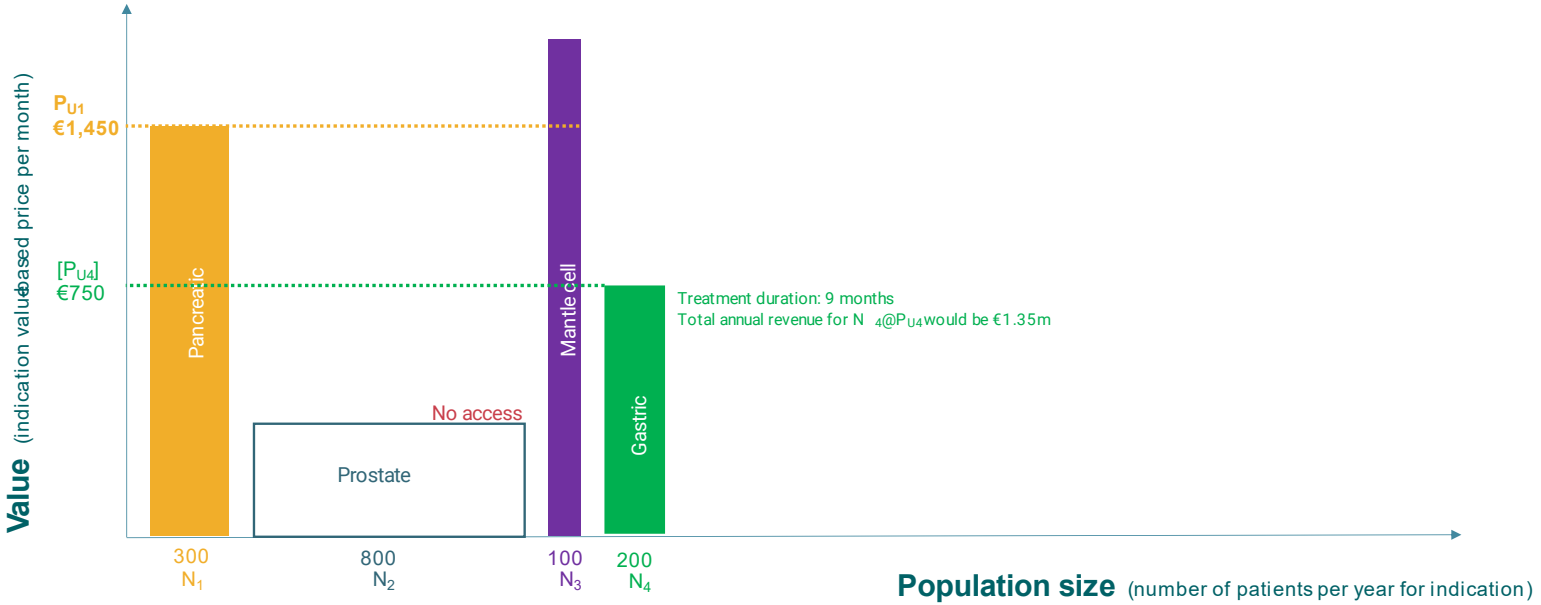

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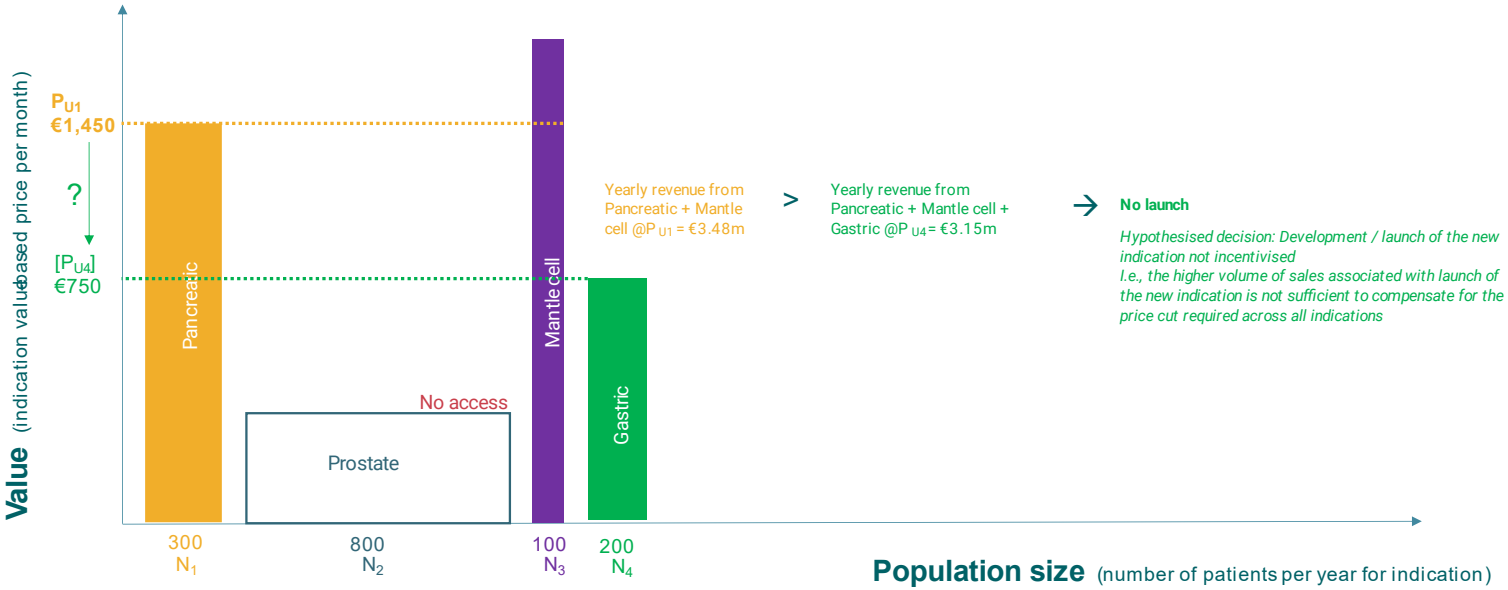

Yearly revenue from Pancreatic + Mantle cell @P_{U1} = €3.48m > Yearly revenue from Pancreatic + Mantle cell + Gastric @P_{U4} = €3.15m → **No launch**

Hypothesised decision: Development / launch of the new indication not incentivised
I.e., the higher volume of sales associated with launch of the new indication is not sufficient to compensate for the price cut required across all indications

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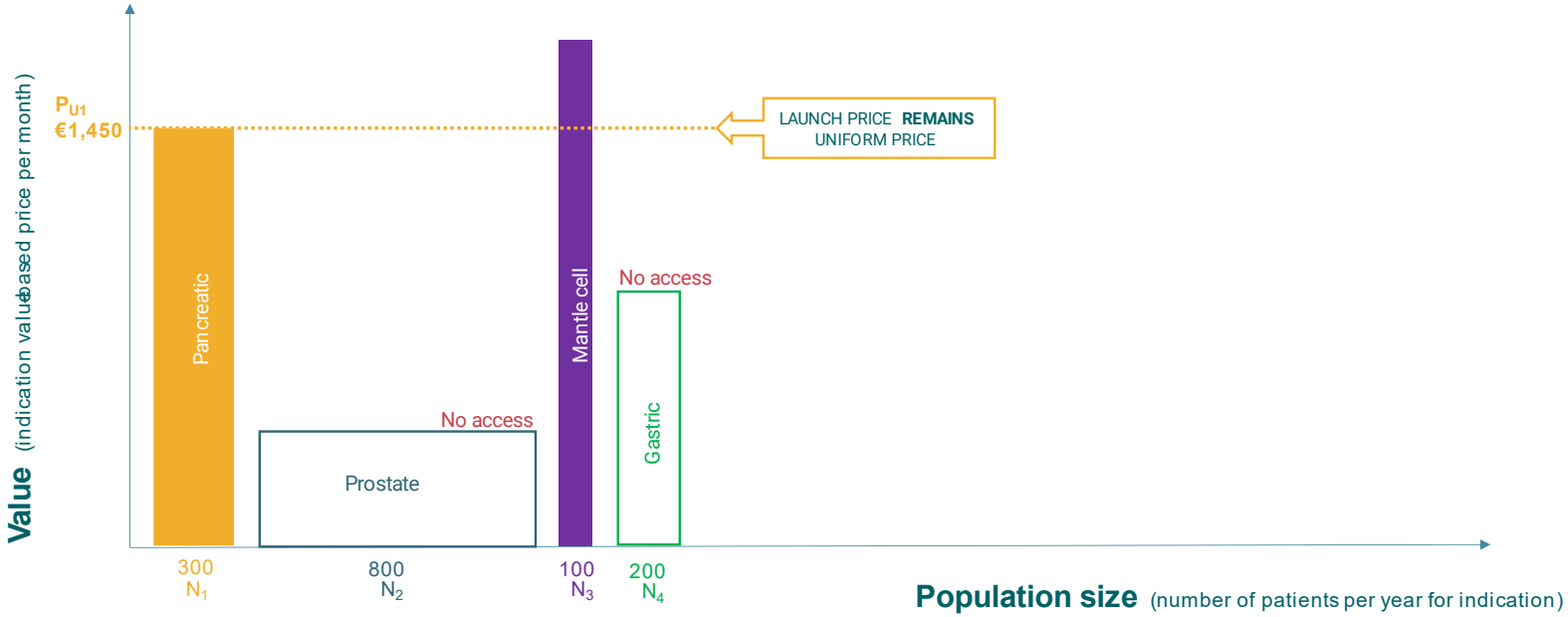
Uniform price



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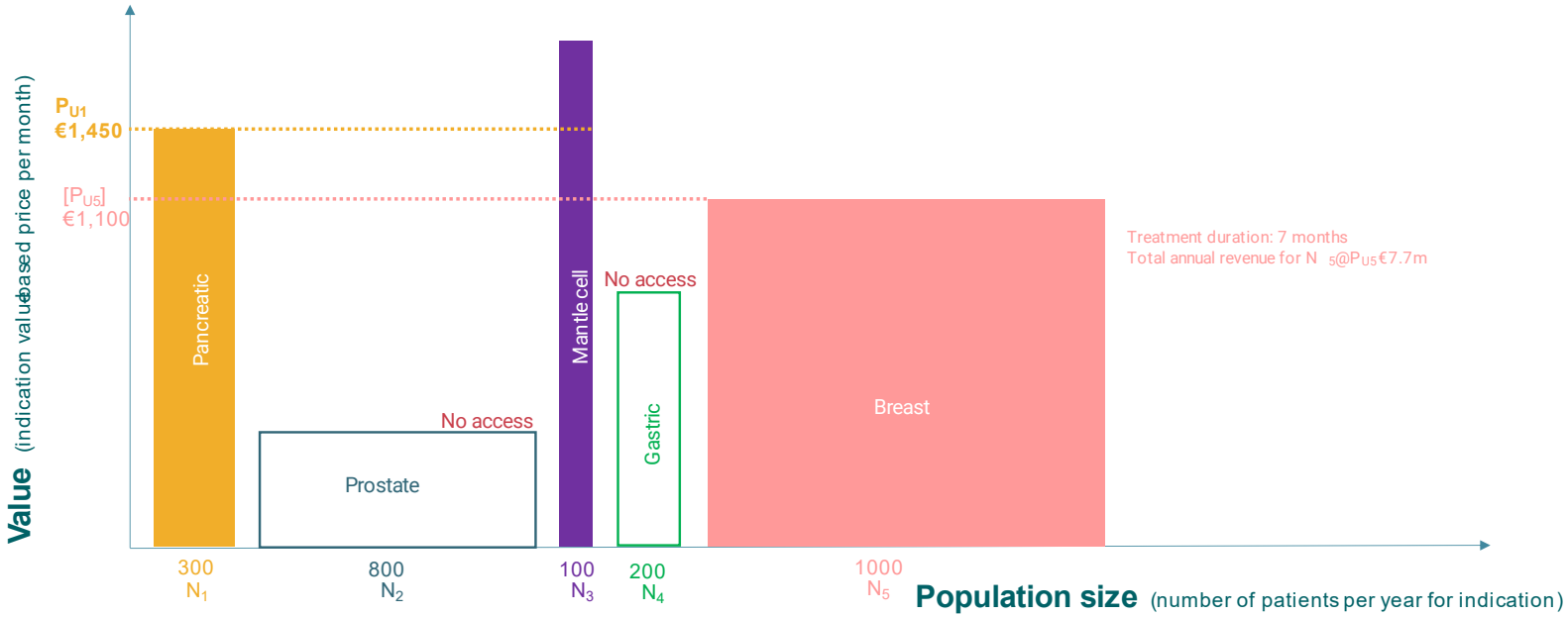

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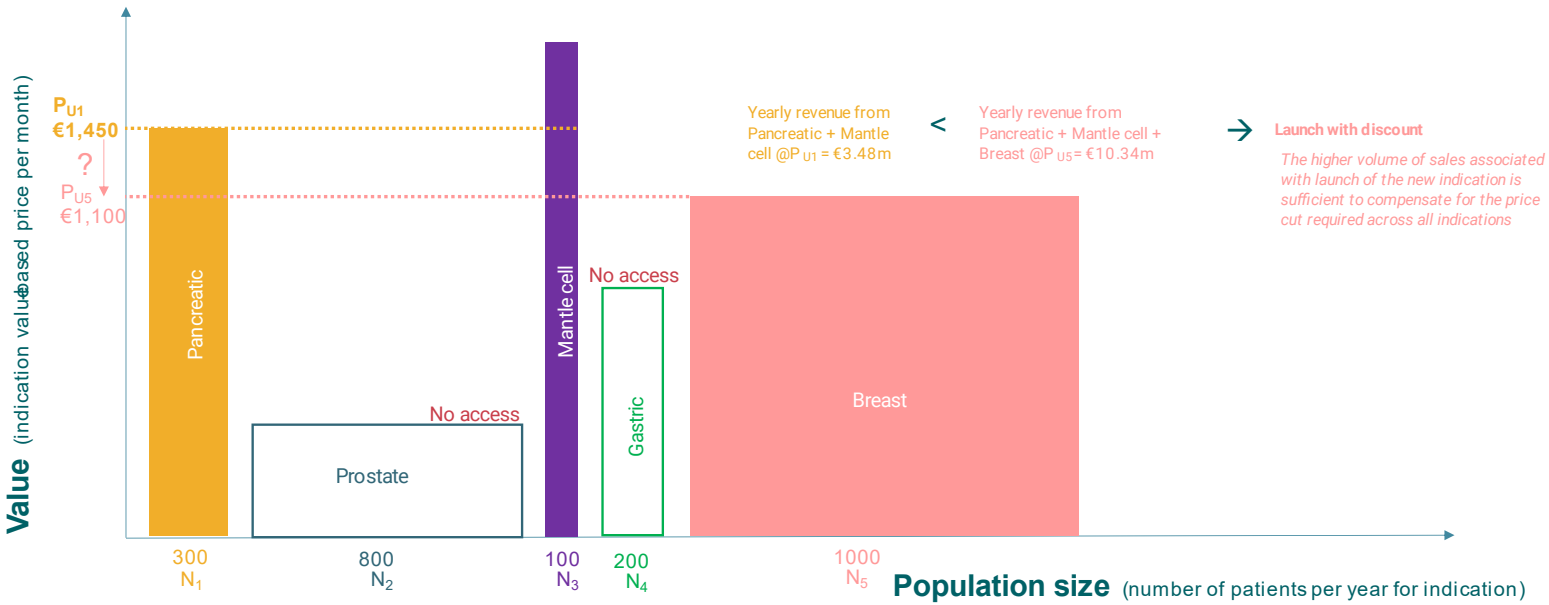

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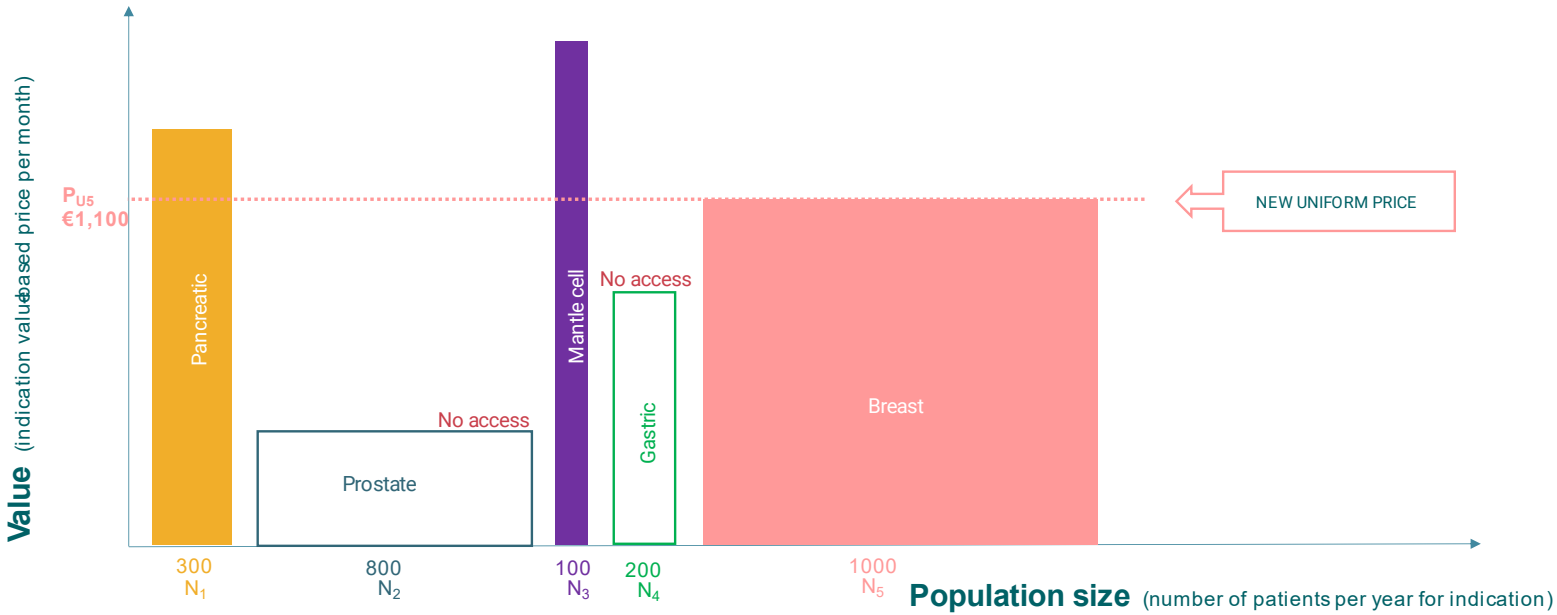

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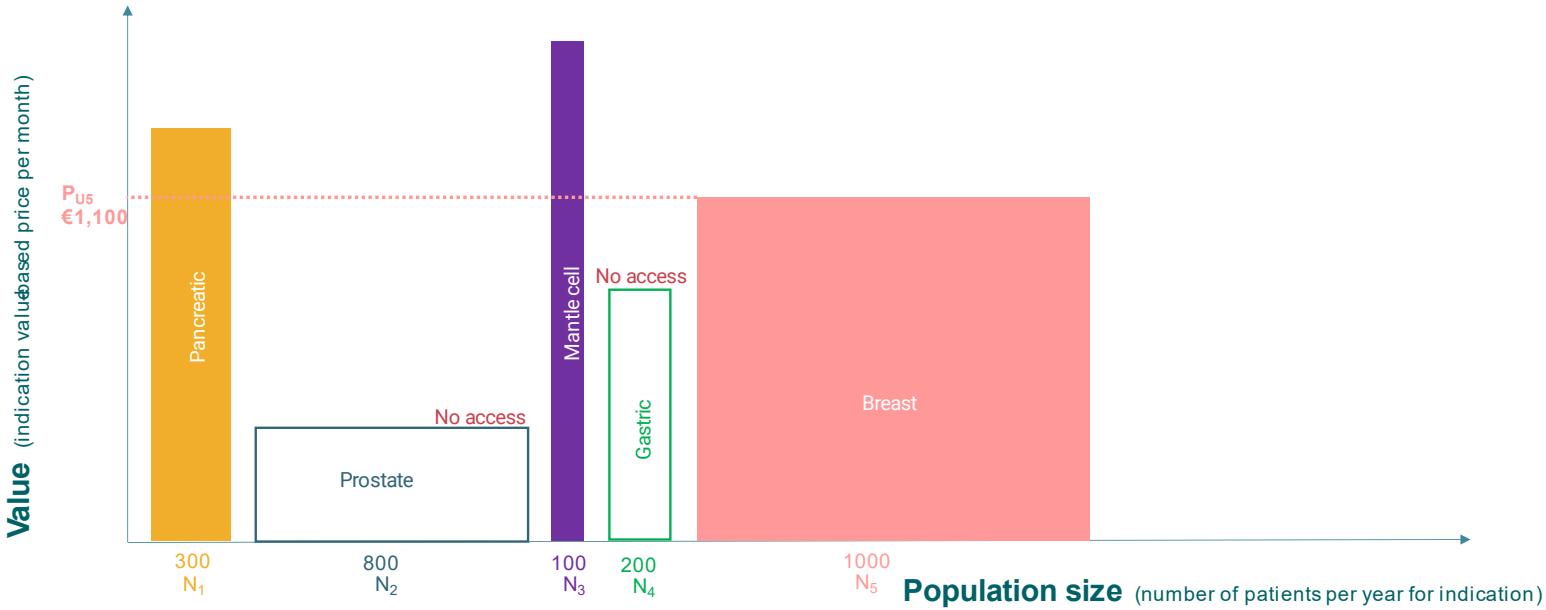
SUMMARY: Uniform price scenario



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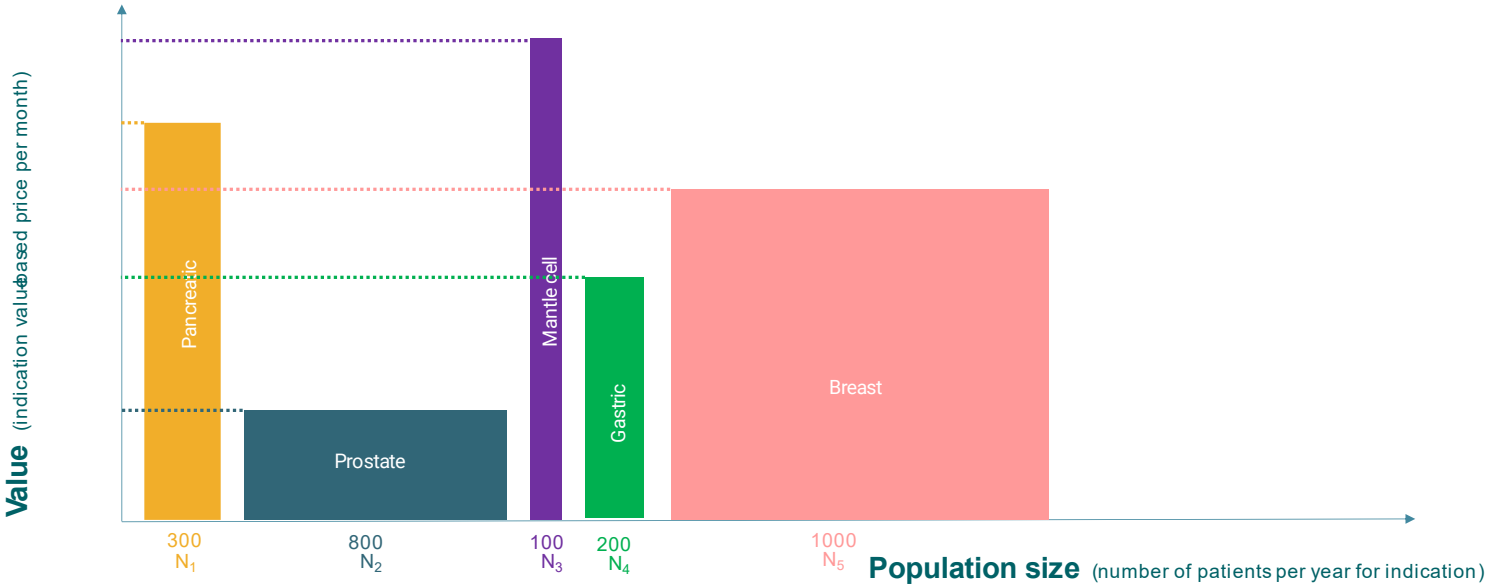
SUMMARY: Valuebased differential pricing scenario



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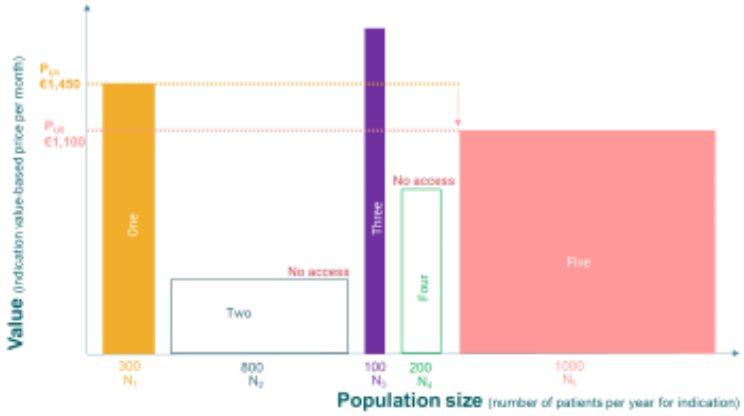
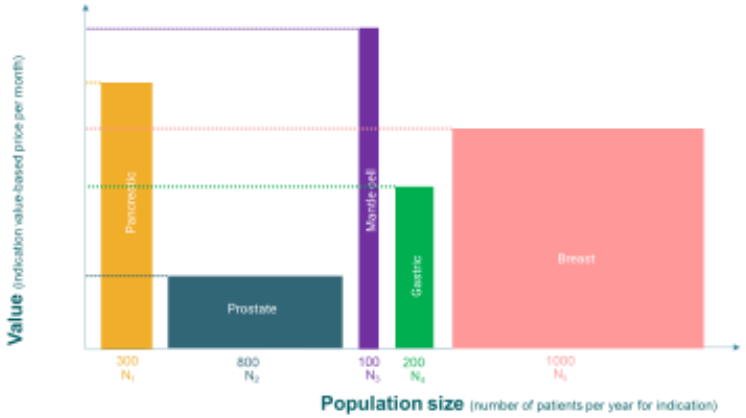
Uniform pricing



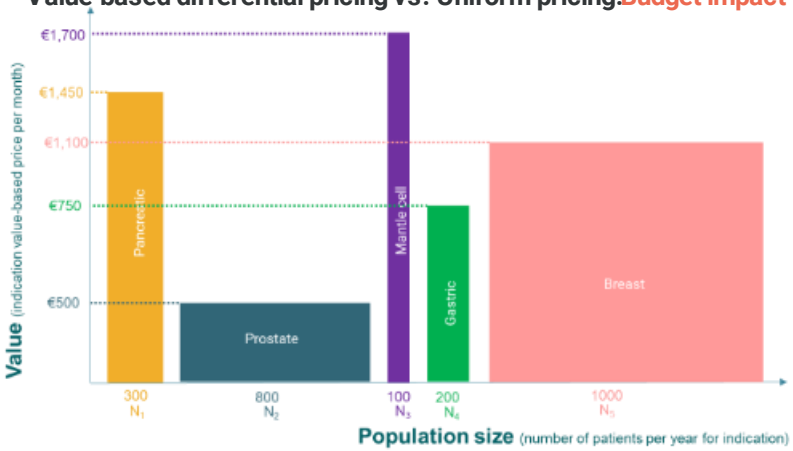
Value-based differential pricing



Value-based differential pricing vs. Uniform pricing

- **Greater patient access & benefit**: 509 QALYs gained (2096 under VBDP scenario; 1587 under uniform pricing)
- **Better incentives for innovation**: Two further indications unlocked
- **Budget impact** for payers: Payers have more granular view / budget control at the indication level; budget impact *depends on what level the indication-based prices are set*

Value-based differential pricing vs. Uniform pricing. Budget impact for payers.

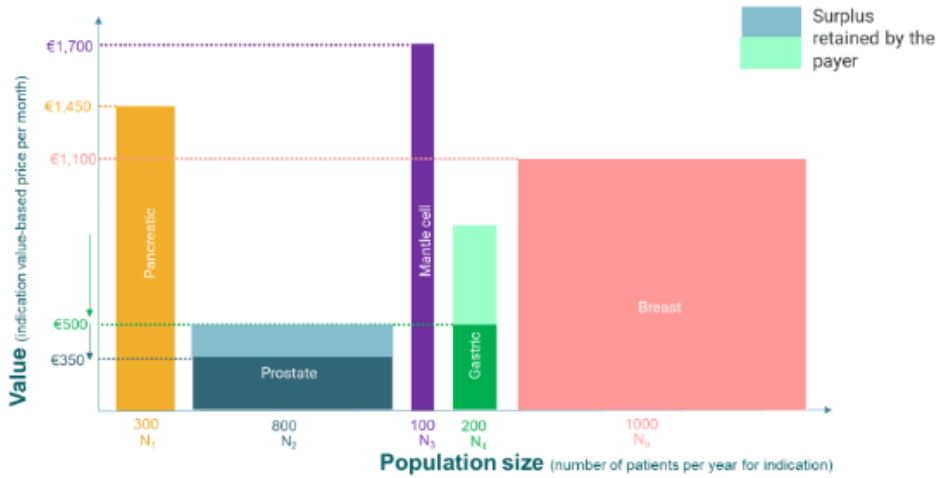


If prices are set to the indication-level value-based price (maximum reimbursable price, i.e. based on an implicit or explicit cost-effectiveness threshold), then:

- VBDP would require a higher investment of €18m (€62.9m versus €45m under uniform pricing)
- Note that this would represent a cost-effective use of resources for the payer, based on the extra health benefit generated.
- In this scenario, the economic "surplus" would be retained by the manufacturer

However, these should be considered price "ceilings". Where prices are set below these ceilings (either naturally via the impact of increased competition– a plausible side-effect of permitting VBDP– or through regulating prices), then the patient benefit from expanded patient access can be maintained, with both payers and manufacturers also being better off.

Value-based differential pricing vs. Uniform pricing.Budget impact for payers.



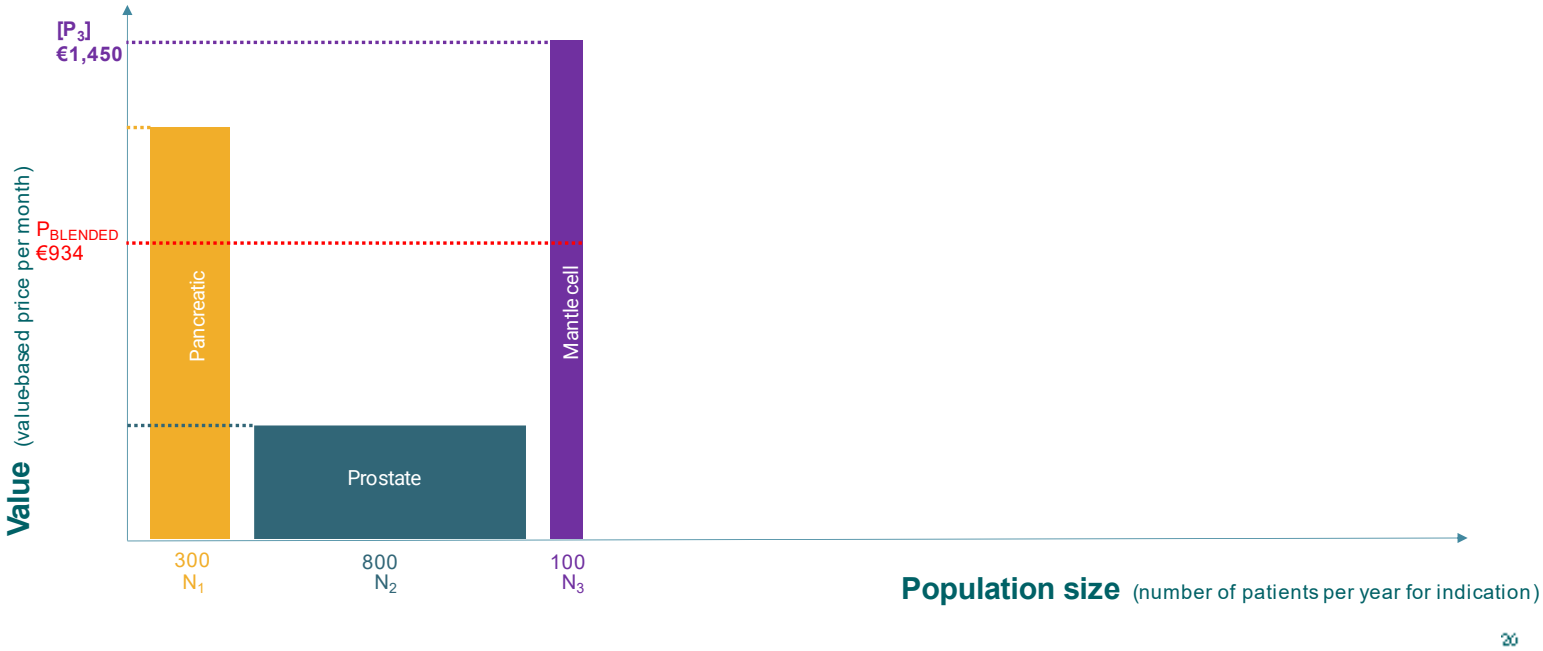
CONCLUSIONS

- Value-based pricing at the indication level is likely to lead to…
  - Better incentives for innovation, and consequently:
  - Expanded patient access and health benefit (particularly in relatively lower -value indications, or indications with smaller patient populations)
- Budget impact will depend on how value -based prices are set at the indication -level
  - Higher budget impact may arise as a consequence of increased investment in expanded patient access
  - The additional value generated by VDBP can be shared in such a way that all parties benefit. This could arise naturally from:
  - Increased competition
  - Smarter and more directive payer procurement at the indication level

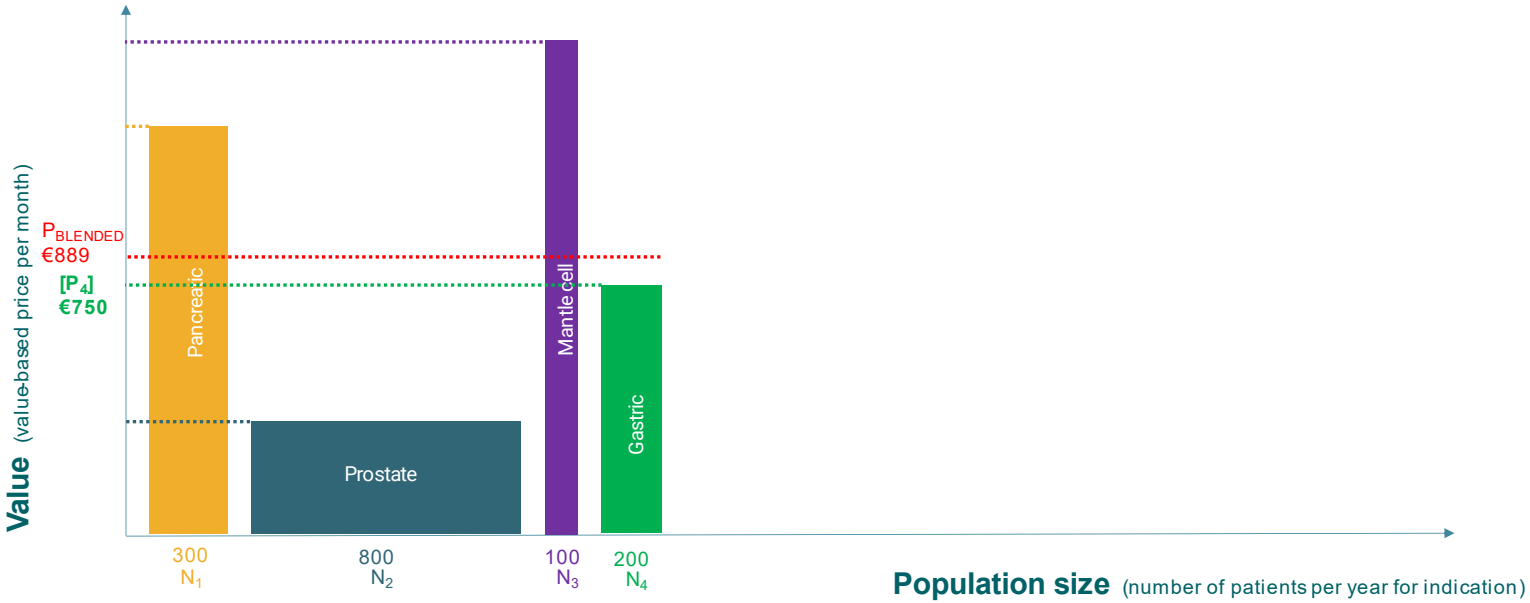
Appendix 2.1: Blended price evolution


Blended Price 2021
[P1]
€1,450
PBLENDED
€842
[P2]
€500
Value (value-based price per month)
Pancreatic
Prostate
300
N1
800
N2
Population size (number of patients per year for indication)

Blended Price 2022-2024



Blended Price 2025



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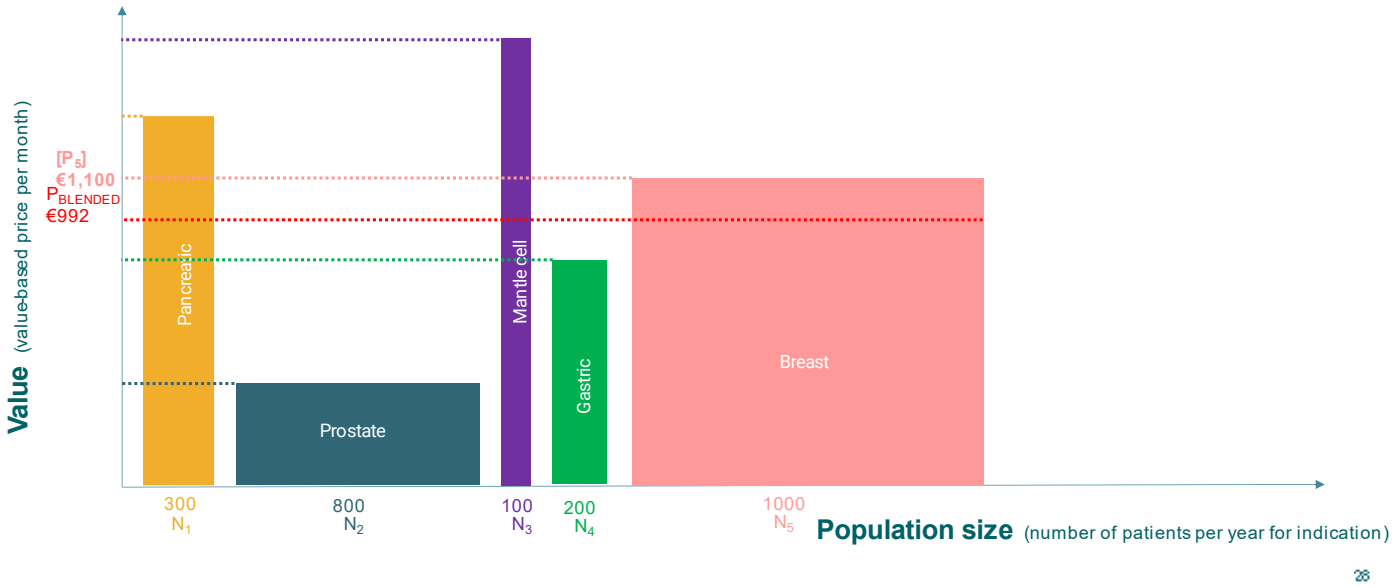
Blended Price 2026-2027



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Founded in 1962 by the Association of the British Pharmaceutical Society, the Office of Health Economics (OHE) is not only the world's oldest health economics research group, but also one of the most prestigious and influential.

OHE provides market-leading insights and in-depth analyses into health economics & health policy. Our pioneering work informs health care and pharmaceutical decision-making across the globe, enabling clients to think differently and to find alternative solutions to the industry's most complex problems.

Our mission is to guide and inform the healthcare industry through today's era of unprecedented change and evolution. We are dedicated to helping policy makers and the pharmaceutical industry make better decisions that ultimately benefit patients, the industry and society as a whole.

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Areas of expertise

- Evaluation of health care policy
- The economics of health care systems
- Health technology assessment (HTA) methodology and approaches
- HTA's impact on decision making, health care spending and the delivery of care
- Pricing and reimbursement for biologics and pharmaceuticals, including value-based pricing, risk sharing and biosimilars market competition
- The costs of treating, or failing to treat, specific diseases and conditions
- Drivers of, and incentives for, the uptake of pharmaceuticals and prescription medicines
- Competition and incentives for improving the quality and efficiency of health care
- Incentives, disincentives, regulation and the costs of R&D for pharmaceuticals and innovation in medicine
- Capturing preferences using patient-reported outcomes measures (PROMs) and time trade-off (TTO) methodology
- Roles of the private and charity sectors in health care and research
- Health and health care statistics

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NOVEMBER 2021

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