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SHOULD DRUG PRICES DIFFER BY INDICATION?

Indication-Based Pricing (IBP) Discussion Paper

CONSULTATION BRIEFING
MAY 2019

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Foreword

How we pay for our medicines has very important implications for how (and whether) medicines are developed and subsequently used. Although the science underlying the way in which medicines are developed has evolved, there has been little change in how we pay for them. Increasingly, medicines will have multiple indications. New payment mechanisms are being proposed to address this. One such mechanism is allowing the healthcare system to support different prices for different indications of a medicine: indication-based pricing (IBP). Whilst there are some limited examples of IBP implementation, they are few.

The purpose of this consultation exercise is to concisely explain the major issues that should be considered around IBP, and to elicit your thoughts on the best way forward. More specifically, in this Discussion Paper we set out:

- **What is IBP** and **why it is relevant now?**
- What are the potential **benefits?**
- What are the potential **draw-backs?**
- What do we need to think about when we consider **implementation?**

We then ask:

- **What do you think, and where would you go next?**

By doing so, we hope to work towards a shared understanding of IBP and to capture a range of perspectives on its use. We walk you through our explanation of IBP, and its potential merits and disadvantages. At the end of the Paper, we ask you to respond to a number of questions.

After collecting the thoughts of all stakeholders consulted, we will publish the results in a "way forward" editorial. We very much value your insight, and we thank you for participating in this important exercise.

1. What is IBP, and why is it relevant now?

Scientific advances are delivering new medicines, with gains in survival and improved quality of life, which have a number of different clinical applications across different disease areas and patient populations, and/or in combination with other therapies. On the one hand, the take up of these drugs varies in part because of the (single) price, which may not represent value for money across all treatment uses. On the other hand, innovators' ability to deliver meaningful treatment advancements to patients depends on obtaining revenues. It is therefore critical that we get the balance right.

Price should be linked with value, but a single price may not accurately reflect value across multiple indications of a medicine

It is a broadly accepted economic concept that price should be linked in some way to the value of a good; how that happens varies according to the structure of the market. The "value" of a drug is generally considered in relation to the health gain that a medicine generates for a patient, and in some countries to the impact on health system costs/savings as well. Linking price with value means that the health system achieves value for money, and innovators are appropriately rewarded (and therefore incentivised).

Whilst the science underpinning drug discovery and development has evolved and changed significantly – with knock-on implications for the pharmaceutical pipeline – the way we pay for drugs is changing only slowly. Medicines are generally paid for on a per-unit basis, where a single price is attached to each unit (e.g. pill or pack or vial) of a medicine. However, individual medicines are being increasingly used to help benefit patients in varied contexts. These uses can be associated with different treatment regimens or dosage and deliver different clinical and/or economic value for patients and payers. We use the term **indication** to refer to different uses of a medicine, for example:

- for different disease (e.g. different cancers);
- at different stages of disease;
- at different points in the treatment regimen, and;
- in various combinations with other therapies.

In 2014, over half of major anti-cancer medicines were licensed for multiple indications (Aitken, Blansett and Mawrie, 2015); in 2018, three-quarters of cancer drugs are used in multiple indications, with an average of five indications per new active substance (Aitken et al., 2018).

Indication-based pricing allows price to vary by indication

How can a medicine's price be linked in some way to the value it generates when the same medicine is being used in many indications, and the incremental value[1] that is achieved is likely to differ substantially by indication? Indication-based pricing (IBP) has been proposed as a way to tackle this issue, permitting price to vary according to indication and – critically – according to value. In other words, moving away from a price for a drug to a price for each use of a drug. We use the term Indication-Based Pricing (IBP) throughout this paper, but other terms that are used include multi-indication pricing (MIP) and indication-specific pricing (ISP).

In previous reports (Towse, Cole and Zamora, 2018; Cole et al., 2018), we summarise the key points of debate around IBP as described in the literature, its implementation to date, and we explore the economic arguments for and against. Below, we summarise these key arguments.

[1] *By incremental value we mean the additional health and health-related gain delivered to the patient over and above the current standard of care.*

2. Potential benefits

A single price for a single drug creates a disconnect between price and incremental value. IBP could address this disconnect, by linking payments for a medicine with the incremental value at the indication-level

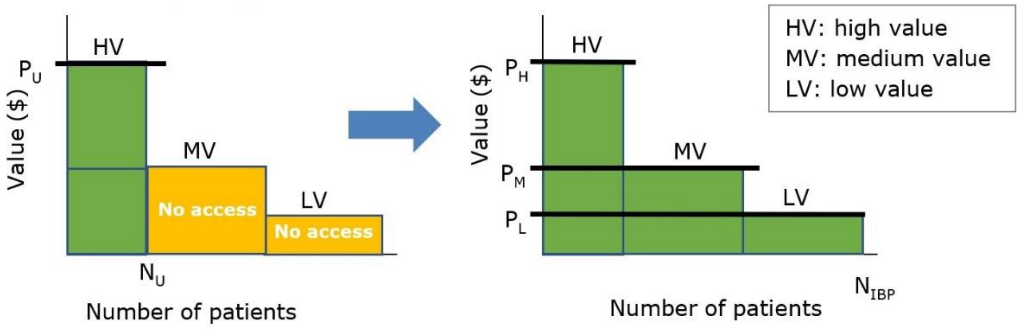
IBP involves charging different prices for different indications. It is therefore a form of price discrimination. Without IBP, a single (uniform) price is set, often anchored on the value of treatment in the medicine's first launch indication. This means that, in a system that uses health technology assessment (HTA) to regulate price, the costs and benefits of a drug are evaluated in relation to price determined for the launch indication. Subsequent research and development (R&D) or use in clinical practice may unearth new beneficial uses of the medicine. Some of these may already be under development when the first indication is licensed. Others may not have been anticipated when initial uses were developed.

A single price system does not allow value-based prices to be set across all uses of a multi-indication medicine. IBP, on the other hand, can permit prices which reflect true differences in value across indications (Bach, 2014; Pearson et al., 2017; Flume et al., 2016). Where indications are already being reimbursed but at a price that is too high to represent good value in that particular indication, then IBP can permit price to fall to a more appropriate level.

IBP can expand patient access and increase societal welfare

Perhaps the most compelling argument in favour of IBP is that, in countries where there is both a collective purchaser acting on behalf of patients, and that purchaser restricts reimbursement based on a medicine's cost-effectiveness or therapeutic added value, it could expand patient access to medicines. By permitting price to vary by indication, uses that (based on the current, single or "uniform" price) are judged to be of lower value and rejected could be reimbursed at a new, lower price. Drug manufacturers would be better incentivised to develop new indications, without the risk of undermining price in the product's anchor indication. Indications that in a single price-world are not reimbursed (because manufacturers and payers are unable to agree a single price which includes these indications) can be used to benefit a broader population of patients.

Figure 1. A move from single price (set to correspond with HV indication) to Indication-based pricing



This is demonstrated in Figure 1. In a single price world, the left-hand figure demonstrates a scenario whereby the price is set to correspond with the high value (HV) indication. This means that, at this uniform price (PU), the medium- and low- value (MV and LV) indications are not good value for money, and therefore are not reimbursed. The green shaded area represents economic "surplus" (or value) accrued from providing access to NU number of patients from the high value indication, and the yellow shaded area represents value that is not realised as patients who could benefit from the medium and low value indications do not have access. In economic terms, this "surplus" (value) accrues to the producer in the form of revenue. Moving to IBP, three prices are permitted, which correspond with the high, medium and low value indications respectively (PH, PM and PL). The revenues accruing to the producer increase. More importantly, the number of patients who now have access to the drug has expanded significantly (NIBP > NU). This means that societal welfare has increased.

It should be noted, however, that in countries where the objective is to negotiate a single price at which access is provided to all indications, then the issue becomes whether the single price is some sort of weighted average (one of the ways in which IBP can be implemented), or whether it is based on other, less formal mechanisms. In either scenario, this single price may not be reflective of true differential value or usage. This has implications for realising the longer term benefits discussed in Section 4.

IBP sends the right signals to stimulate R&D

IBP could support the development of new indications that may otherwise not have been launched, by encouraging research into further treatment targets. As well as encouraging the development of relatively lower-value indications by permitting a system that can offer a lower price, a system of IBP could also expand access to some low-volume high-value indications, which may not be economic to develop at the current single price for the drug.

Future-proofing the reimbursement landscape for innovative medicines

As well as supporting the expansion of new indications that could be serviced by today and tomorrow's innovative medicines, IBP could also address specific challenges such as combination pricing. Increasingly, medicines are being found to be of incremental value when delivered alongside another therapy, yet, payers and HTA agencies are struggling to find ways to approve these combinations, where an additive pricing model yields total treatment costs that are not affordable. Whilst not the "solution" in itself to solve the complex problem of how to assign value to combination therapies, IBP is a pre-requisite for finding a solution. Using a product in combination with another product is a different indication to using it in mono-therapy.

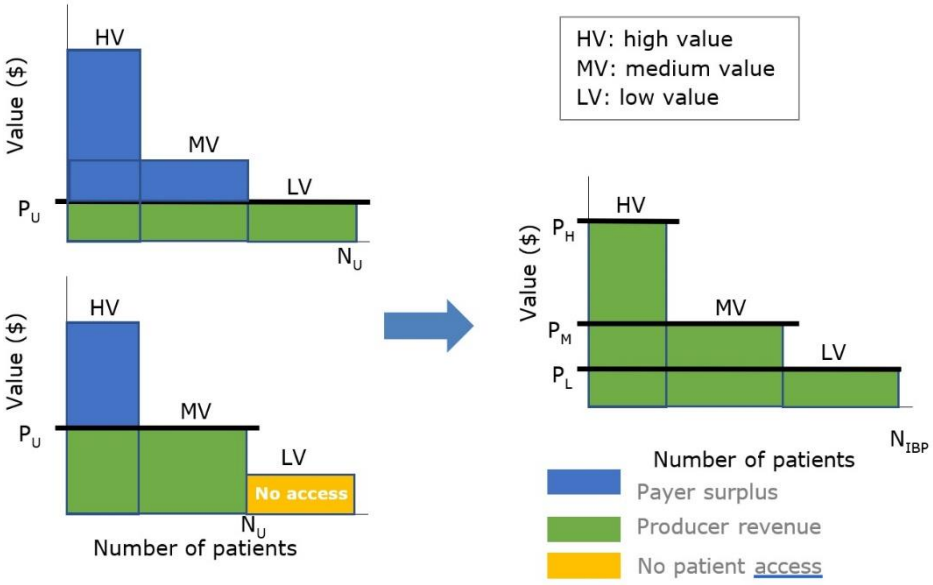
3. Potential draw-backs

Depending how it is implemented, IBP could lead to higher prices for some indications

Figure 1, above, demonstrated how, in cases where the single price is set at the high value indication, IBP means that payers spend more and in doing so derive additional value for patients (or, if the medium/low value indications are already reimbursed at the high value price, then they would save money). Figure 2 (below), however, demonstrates the transfer of surplus from the payer (represented by the blue shaded area) that would occur if the current uniform price corresponds with the medium or low value indications, and IBP enabled a rise in price for higher value indications.

Figure 2 again describes a situation where a treatment delivers different value in different indications and refers to a market that uses HTA to determine cost-effectiveness, where price is aligned to value. Moving from a single-price world where price is set to correspond with the lowest-value indication (top-left), a move to IBP would be associated with no increase in patient access, and a transfer of value from the payer to the producer (Chandra and Garthwaite, 2017). However, this starting scenario is unlikely. In reality the low value indication would be less likely to be launched if that would drive a low single price, potentially reducing total revenue despite increased volume. In a world where the single price corresponds with the medium value indication, the high-value indication price will rise (thus increasing producer revenues), and price would be allowed to fall for the low-value indication, thus expanding patient access, and, as a consequence, increasing producer revenues.

Figure 2. A move from single price (set to correspond with LV and MV indications) to Indication-based pricing



IBP could add to short term expenditure while not addressing the problem of affordability

Whilst price discrimination has the effect of expanding patient access, it is also likely to increase drug spend in the short term, thus adding to payers' affordability challenges. The only exception would be if all indications (high, medium, and low) were reimbursed when the single price was set at the high-priced indication. This would mean that payers are obtaining poor value-for-money in the single price system and is the scenario Bach (2014) sets out in a US context. However, with HTA-based value assessment in a health system this is unlikely to happen.

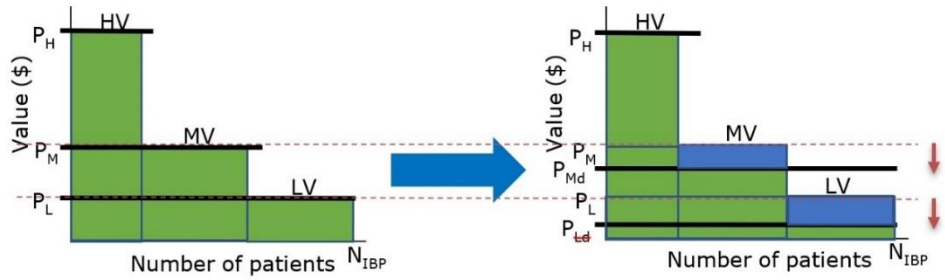
Some payers have argued that the single price should go down to that of the lowest indication, in order to address affordability from additional patient access, and in recognition of the additional volume that the innovator will be getting from new indications. The reality, however, is that new indications may not be launched, or indeed developed, if the effect is to lead to price cuts for existing indications that reduce overall manufacturers' revenues from all uses of the medicine.

4. What might be the longer-term impact?

In the long-run IBP should provide the right incentives for R&D and could increase price competition at the indication-level, driving down prices and delivering better value to the health system

Manufacturers are not price-setting monopolists, because there is strong competition in this field of research. A new scientific breakthrough often leads (sometimes with a lag) to the entry of competing patent protected products in a new therapy area. Value-based indication prices (based on setting price at the maximum willingness to pay) should therefore be seen as price 'ceilings'. If IBP were implemented, more indications would be launched, which would drive more competition at the indication-level. This means that prices would likely be driven down, reducing spend for payers and generating more value.

Figure 3. The projected impact of IBP in the longer-term "dynamic" scenario



In summary, whilst IBP may mean increased spending in the short-run, it increases patient access and provides a supportive environment for R&D innovation, and in the long-run, if it increases price competition, could help secure health care sustainability.

5. Considerations for implementation

IBP requires a shared understanding among stakeholders of how and when new indications should be assessed and valued

A pre-requisite of IBP is a shared understanding among stakeholders in a health care system of how health benefits should be valued, and the criteria against which price is assessed. IBP ought not to be implemented without the agreement of key stakeholders.

Constraints around data collection are regularly cited as a barrier to IBP

Data collection to support IBP must be objective, collected with minimal burden and accessible to the right parties only. At a minimum, in order to pay by indication, the system must have the capability to

track which indication medicines are prescribed for. In reality, although there are some pockets of good practice, across many health care systems there is poor data availability for tracking use by indication. In addition to the lack of data infrastructure and linkage opportunities, the administrative burden for clinical staff could be significant. On the other hand, IBP could facilitate the creation of richer real-world data sets, and greater transparency in the utilisation of drugs, notably in the area of cancer where so many drugs in development could serve multiple indications.

There could be additional legal and contractual barriers

Depending on the national arrangements for reimbursement, there could be market-specific pricing law and contractual barriers as well as privacy concerns around data sharing. In particular, depending on the form of IBP adopted, there may be required changes to the billing infrastructure in a health care system in order to allow rebates, or new ways may need to be found to maintain net price confidentiality.

IBP could take a number of forms

Given the complexities of creating systems that allow multiple prices for a single drug, implementation of the concept of IBP may take a number of forms. Examples include:

- A blended price, which accounts for the differential value across different indications and renders an "average" payment value (price) which is linked to actual utilisation;
- Discount levels (applied upfront) or rebates (applied ex-post) that are able to vary by indication, and could be confidential;
- A different brand name for each individual product indication;
- Agreements between payers and manufacturers which adjust price according to realised performance, which are intended to address use in different indications. This type of arrangement e.g. managed entry agreements (agreements between manufacturer and payer to withhold or pay-back money depending on performance) are typically used for a single indication, and are often regarded by both parties as complex to negotiate and difficult to implement, but this form of outcomes-based contracting could be used to implement IBP to pay for outcomes at the individual patient level.

6. Where next?

We would be very grateful if you could respond to each of the following consultation questions, from your own perspective given your job role and national context.

In order to contribute your thoughts, please access and complete the questions by clicking on this link, which takes you to the online survey: Indication-Based Pricing (IBP) Consultation

Consultation closing date: Monday, 30 September 2019.

About you

Which stakeholder group do you belong to or represent?

☐ Payer
☐ Patient, carer, or patient/carer organisation
☐ Industry
☐ Regulator
☐ Clinician
☐ Academic scientist
☐ Consultant
☐ Other. *Please specify:* Click or tap here to enter text.

In what country do you live and/or work professionally?

Please specify: Click or tap here to enter text.

The need for IBP

Would some form of IBP be a good thing?

☐ Yes
☐ No
Please explain: Click or tap here to enter text.

Understanding of IBP

To what extent do you think there is a broad understanding of IBP and its implications among relevant stakeholders?

	Payers	Patient groups	Industry	Regulators	Medical societies	Academic scientists	Consultants
Not at all	☐	☐	☐	☐	☐	☐	☐
Somewhat	☐	☐	☐	☐	☐	☐	☐
Good understanding	☐	☐	☐	☐	☐	☐	☐

Please explain: Click or tap here to enter text.

Your thoughts on IBP

Who is most likely to (or does) benefit the most from IBP?

☐ Patients
☐ Industry
☐ Payers
☐ All stakeholders could gain
☐ No-one gains from IBP
Please explain: Click or tap here to enter text.

What impact would (or does) IBP have in terms of delivering sustainable access to future treatments?

☐ A significant impact
☐ A small impact
☐ No impact
☐ Other: Click or tap here to enter text.
Please explain: Click or tap here to enter text.

Do you have any practical experience of IBP?
☐ No
☐ Yes

If yes, please explain what model of IBP you are familiar with: Click or tap here to enter text.

What are the potential impacts of IBP?

What might the impact of IBP be on patient access?

☐ Patient access reduced
☐ Patient access unchanged
☐ Patient access expanded
☐ Other: Click or tap here to enter text.
Please explain: Click or tap here to enter text.

What might the impact of IBP be on industry? (if desired, you may select more than one)

☐ No impact on industry
☐ IBP would allow industry to optimise R&D spending, and may increase profits
☐ IBP would complicate market access activities unnecessarily
☐ Other: Click or tap here to enter text.
Please explain: Click or tap here to enter text.

What might the impact of IBP be on payers?

☐ No budget impact
☐ IBP would raise expenditure, with no meaningful benefits
☐ IBP would put pressure on payer budget, but deliver greater health gain for patients
☐ As above, but in the long-run market forces will lead to lower prices
☐ Other: Click or tap here to enter text.
Please explain: Click or tap here to enter text.

Would IBP impact on manufacturers' decisions about how and when to bring new indications to market?

Please explain: Click or tap here to enter text.

Is IBP likely to have any unintended consequences?

Please explain: Click or tap here to enter text.

Implementing IBP

Optimally, how should IBP be implemented?

☐ Different brand names for individual products
☐ Differential list prices aligned with value for each indication
☐ A single price based on a weighted average of value and usage across indications
☐ Price received by the manufacturer (or discount level) should be determined not at the indication-level, but by the individual patient-level outcome
☐ IBP implementation is not desirable
☐ Other: Click or tap here to enter text.
Please explain: Click or tap here to enter text.

In practice, how do you think IBP could most realistically be implemented and why?

Please explain: Click or tap here to enter text.

How does the issue of more flexible pricing (such as that permitted by IBP) fit as a policy priority among the wider pressures / issues that you observe for patient access to medicines?

Please explain: Click or tap here to enter text.

Practical challenges

What is the single most significant barrier to the implementation of IBP?
☐ Political will and lack of stakeholder buy-in
☐ Data collection in terms of burden to the clinical staff providing patient care (effort / workload)
☐ Data infrastructure (technical capacity to collect the information required)
☐ The ability to make changes to the current billing infrastructure for reimbursement of pharmaceuticals
☐ Concern about short-term payer budget impact
☐ Other: Click or tap here to enter text.
Please explain: Click or tap here to enter text.

What steps could be taken to address these challenges?

Please explain: Click or tap here to enter text.

Thank you very much for participating in this consultation exercise

Following the consultation period, we will be analysing responses and writing-up the results. If you would like to receive a copy of the output, please leave an email address we can send it to: Click or tap here to enter text.

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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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