SHOULD DRUG PRICES DIFFER BY INDICATION?

Indication-Based Pricing (IBP) Consultation Report

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Executive Summary

The notion that the price of a medicine should be linked in some way to the value it generates for patients and the health system is generally accepted. Yet, it is not clear how this can be achieved when medicines are being developed that increasingly offer patient benefit across many different indications. Indication-based pricing (IBP) has been proposed to tackle this issue, permitting price for any individual medicine to vary according to indication and, importantly, according to value.

In 2019 OHE published a Discussion Paper on IBP, which was accompanied by a Consultation Survey (Cole et al., 2019). The survey was designed to collect responses – from a range of stakeholders – around the potential benefits or drawbacks of IBP and considerations for implementation. In this report, we summarise and discuss the results of this consultation exercise.

The survey was launched in English, French and Spanish, and consisted of a range of multiple choice and open questions. Where possible, results of multiple-choice questions are reported by country, type of stakeholder, and whether or not respondents have practical experience of IBP; open questions were analysed thematically.

A total of sixteen countries were represented in the 73 valid responses collected, the most common being the UK, Belgium, France, Spain and Switzerland. Respondents represented a diverse range of stakeholders, including (but not limited to) industry, payers, regulators and academics; nearly half of respondents had some practical experience of IBP.

Most respondents agreed that some form of IBP would be a good thing (78%), although this belief was held most strongly by industry (96%) and regulators (83%), and less strongly by payers (65%) and academics (57%). Reasons noted by respondents included that IBP is:

- value-based
- expands and accelerates access to innovative medicines
- supports R&D, by allowing manufacturers to target unmet need (regardless of the price of current indications, if that would otherwise be an issue)
- supports launching new medicines (regardless of the price of current indications, if that would otherwise be an issue)
- helps sustainability and management of healthcare budgets
- is needed due to R&D sequencing

Reasons given for why some form of IBP would be unfavourable included that IBP:

- could be difficult to implement
- could plausibly incentivise gaming in prescribing
- puts pressure on budgets
A pre-requisite of meaningful progress towards any kind of pricing reform is a shared understanding of what IBP is. When asked about their perceptions of understanding among the relevant stakeholders, respondents ranked the pharmaceutical industry as having the best understanding of IBP, and patient groups the least understanding. Lack of understanding is clearly a challenge for an informed debate on the use of IBP.

Who benefits from IBP? In our discussion paper, we present how IBP can expand patient access and increase societal welfare. More than half of all respondents (57%) thought that all stakeholders could stand to gain from IBP. Of the remaining respondents, 31% considered industry to be the single stakeholder that stood to gain the most from IBP, followed by payers (6%), patients (3%) and no-one (3%).

A majority of respondents agreed that IBP could have a positive impact in terms of delivering sustainable access to future treatments (48% thought a significant impact; 83% at least some impact) and would be likely to expand patient access (70% of respondents agreed). Most survey respondents thought that IBP would allow the industry to optimise R&D spending, but some suggested that it may complicate market access. When asked to consider the impact on payers, three-quarters of respondents thought that IBP would put pressure on payer budgets: 21% thought that this expenditure rise would be accompanied by no meaningful benefits, whilst 56% of respondents thought that IBP would put pressure on payer budgets but deliver greater health gains for patients; a subset of these (20% of the whole sample) believed that the budget pressure for payers would be in the short-term, as in the long-run market forces would lead to lower prices.

Respondents commented on whether IBP would affect manufacturers’ decisions about how and when to bring new indications to the market. Many commented on how IBP would send the right signals to the pharmaceutical industry and enable investment in R&D across indications; they also believed that it would reduce concern around indication sequencing (relative to the current situation, where there is no flexibility up/down for price despite the introduction of new indications for which a value-based price could be vastly different). Moreover, IBP could encourage earlier launches (by avoiding protracted negotiations when the prevailing price doesn’t align with a new indication), accelerate pricing negotiations, and make development in some indications more viable, particularly for medicines with indications for small patient populations.

How exactly should IBP be implemented? There was a wide spread of opinions among respondents about the optimal implementation of IBP, with the most popular choice being a single price based on a weighted average of value and usage across indications (accounting for 32% of responses). Differential list prices for different indications were considered optimal by 22% of our sample, whilst the implementation of different brand names for individual products was favoured by 17%. Other suggestions mainly revolved around the utilisation of a single list price, but different net prices by indication with confidential discounts based on (aligned to) value, with invoicing based on usage.

The most significant perceived barriers to IBP were lack of stakeholder buy-in / political will (30%) and data infrastructure (technical capacity to collect the indication-specific information required) (30%). Suggested solutions to barriers included improved communication between payers and industry; sharing of best practices; pilot and scenario evaluations; and standardized datasets for capturing medicine usage and/or measuring health outcomes.

There was a general perception among respondents that IBP does not appear to be a policy priority in most countries. Yet, many felt that it should be, suggesting it is the only rational way to price a drug whose effectiveness varies across indications. This consultation exercise indicates that the potential benefits of IBP are numerous, and not limited to one individual stakeholder group. Furthermore, IBP may be necessary to future-proof society’s ability to incentivise and benefit from the evolving nature of drug development.
1 Introduction

Indication-based pricing (IBP) refers to the concept of 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. Other terms that are used include multi-indication pricing and indication-specific pricing. The OHE has explored different aspects of IBP, from the theoretical basis to the practical challenges to implementation. As part of a collaboration with AstraZeneca, OHE launched a Discussion Paper in May 2019, attached to which was a survey setting out several consultation questions collecting respondents’ thoughts around IBP and the best way forward (Cole et al., 2019). The analysis presented in the current report offers an account of the survey and its results.

The consultation exercise was designed to collect thoughts on the major issues that should be considered around IBP. The previously published Discussion Paper (which can serve as a useful reference alongside the current report, to give context to the survey questions and responses) concisely explained some of the major issues by setting out, exploring and explaining:

▪ **What is IBP and why it is relevant now?**
  - Price should be linked with value, but a single price may not accurately reflect value across multiple indications of a medicine
  - Indication-based pricing allows the price to vary by indication

▪ **What are the 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 can expand patient access and increase societal welfare
  - IBP sends the right signals to stimulate R&D
  - Future-proofing the reimbursement landscape for innovative medicines

▪ **What are the potential drawbacks?**
  - Depending on how it is implemented, IBP could lead to higher prices for some indications
  - IBP could add to short term expenditure while not addressing the problem of affordability

▪ **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

▪ **What do we need to think about when we consider implementation?**
  - IBP requires a shared understanding among stakeholders of how and when new indications should be assessed and valued
Constraints around data collection are regularly cited as a barrier to IBP

There could be additional legal and contractual barriers

IBP could take a number of forms

The online survey that was launched alongside this Discussion Paper (see Appendix I: Consultation survey) consisted of a mixture of multiple choice and open questions, designed to explore stakeholders’ thoughts and reactions to these themes. While we recognise that empirical analysis of the realised impact of IBP schemes (or a predictive modelling exercise attempting to forecast the same) would be required to reach more certain answers to some of these questions, the first step in understanding the appetite for reform is to understand stakeholder perspectives. Through this survey, we obtain and analyse the beliefs and perceptions of a range of stakeholders, to bring to light this range of perspectives, with the end goal of supporting a constructive dialogue around IBP.

The English version of the survey was issued in May 2019, with Spanish and French translations being distributed in June 2019\(^1\). The closing date for the consultation was 30th September 2019.

We present when possible the quantitative answers to multiple-choice questions by country, type of stakeholder, and whether or not they have practical experience of IBP. However, a minimum of 5 results within a specific sub-group (e.g. country or stakeholder type) is probably the minimum number required to comment significantly on any meaningful pattern/attribution of answers to a particular group. For this reason, although we can usually comment on results according to respondents’ experience with IBP (as there are only two large sub-groups for this dimension), we are often unable to present detailed results by country or stakeholder type. Qualitative data from the survey’s open questions are presented alongside the relevant quantitative results.

The sections of this report largely follow the sequence of questions asked in the consultation survey. The characteristics of respondents are described in section 2 by type of stakeholder, country and experience of IBP. These characteristics are then used in subsequent sections to present results by stakeholder type and country, where sufficient data allow, and by IBP experience. Section 3 describes respondents’ understanding of IBP and the perceived need for such pricing arrangements, whilst section 4 explores perceptions around who could gain from IBP and its potential impact on patient access, industry, payers and manufacturers. Section 5 considers the implementation barriers and enablers as perceived by respondents to the survey.

It is important to note that the inherent limitation of this survey is that the results obtained are from a self-selecting sample of individuals. Therefore, we must be cautious in our interpretation of findings, and not overstate the generalisability of results.

\(^1\) The Discussion paper and online survey were professionally translated to Spanish and French, with translations certificated by TransPerfect. In parallel - to drive the response rate - an online platform was utilised with a fee for each complete response.
2 Characteristics of respondents

A detailed summary of survey respondent characteristics can be found in Appendix II. 82 responses were received, 73 of which contained sufficient data to include in our analysis. 16 countries were represented, the most common being the UK, Belgium, France, Spain and Switzerland. Respondents represented a diverse range of stakeholders, including (but not limited to) industry (37%), payers (27%), regulators (16%) and academics (10%); nearly half of respondents had some practical experience of IBP.
3 The need for and understanding of IBP

3.1 The need for IBP: would some form of IBP be a good thing?

Most survey respondents are advocates of IBP, with 78% responding "Yes" to the question "would some form of IBP be a good thing"? When considering only those respondents who have practical experience of IBP, this positive stance is further reinforced, with 82% agreeing that some form of IBP would be a good thing, as shown in Figure 1.

By country, positive opinion of IBP is the most frequent response in all countries except France (where 4 out of the 7 respondents responded negatively; however, all 4 had no experience of IBP, whereas those that did have IBP experience responded positively to this question). Notably, all 5 respondents from Switzerland answered "yes". As shown in Figure 2, there were more positive than negative responses among all stakeholder groups represented in our sample. However, this was most extreme for industry (96% positive) and regulators (83% positive), whereas academics and payers were more evenly divided (57% and 65% positive, respectively).

FIGURE 1 BY PRACTICAL EXPERIENCE: WOULD SOME FORM OF IBP BE A GOOD THING?

<table>
<thead>
<tr>
<th>Experience: Yes</th>
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<td>28</td>
<td>20</td>
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<td>82%</td>
<td>74%</td>
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<td>6</td>
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<td>26%</td>
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Total (non-missing) responses: 61
Missing: 12
Below we present a number of themes that summarise the more detailed explanations offered by respondents in free-text form relating to why – according to some respondents – IBP would be a good thing:

- "IBP is value-based": The possibility of allowing price to reflect the indication-specific value of a medicine is mentioned by several respondents, along with the positive impact of enhanced efforts to collect real-world data of clinical benefit to support IBP. Some respondents considered IBP as essential to pricing combination therapies (i.e. setting prices for medicines used in combination with each other within a particular treatment pathway).

- "IBP expands and accelerates access to innovative medicines": Many respondents described this positive impact in relation to indications with small populations in particular, such as rare cancers, where value could be very high but patient numbers are low, and therefore without price flexibility manufacturers may not be incentivised to develop the evidence to support use in those areas. To cite one respondent:

  "A more flexible approach to the pricing of cancer medicines can help accelerate NHS patients’ access to treatment by reducing the risk of systemic delays and addressing the current disincentive to manufacturers of launching new indications in the UK. The benefits are likely to be particularly seen in rarer cancers with small patient populations."

- Also, some respondents cited the potential benefits in terms of equity of access, referring to availability in the public system decreasing the need for private and out-of-pocket payments.

- "IBP supports R&D": It is proposed that IBP supports innovation, allowing manufacturers the freedom to explore and target unmet needs of patients (regardless of prevailing price if that would otherwise be an issue), especially for niche indications such as rare diseases, as well as relatively lower value indications.
“IBP supports launching new medicines”: IBP could improve manufacturers’ incentives and remove commercial barriers to launch new medicines. One such mechanism is described as IBP alleviating the current situation where launching new indications re-opens already finalised pricing/access agreements for a medicine, thereby negatively impacting a previously secure revenue stream (and potentially disincentivising launch).

“IBP helps sustainability and management of healthcare budgets”: By potentially increasing competition through more medicines being made available for a given indication, prices could be lowered (an explanation of this school of thought is described under Section 4 [“What might be the longer-term impact?”] of the Discussion Paper that accompanied this survey (Cole, Towse and Zamora, 2019)). More efficient management of healthcare budgets was also mentioned as a potential consequence of IBP, due to better insights into usage patterns and ability to manage price and therefore spend at the indication level. Importantly, allowing the sharing of risk between industry and payer could contribute to the sustainability of the healthcare system.

“IBP is needed due to R&D sequencing”: Some respondents remarked that R&D and approval often starts with relatively higher-value orphan indications. This is because launch indications are often late-line, as they represent the highest unmet need. These are also the indications for which evidence of impact on overall survival is most easily and quickly demonstrated. Without IBP, subsequent indications that are relevant for wider populations may be denied access if the price is too high, or – if the medicine is taken up – could drive up cost inappropriately.

The topics raised by respondents in the detailed explanation of why – according to some respondents – IBP would not be good thing include:

“IBP could be difficult to implement”: Implementation issues were the most frequently cited, and largely related to lack of information. Respondents referred to: the administrative burden required to ascribe indication to the prescription; technical difficulties, for example, to manage cost information and predict usage per indication and to process reimbursement claims electronically; complications in managing multiple price points for the same product when provided to patients via outsourced services (e.g. home care providers); management complications for pharmacists; and the necessary level of trust that would be required between industry and hospitals to process usage data.

“IBP could plausibly incentivise gaming in prescribing”: Having different prices for a single medicine could incentivise dishonesty in prescribing, particularly if information systems are not able to fully support IBP.

“IBP does not support R&D for new indications”: One respondent challenged the assertion that IBP would support expanded R&D for new indications, stating that that industry would only focus on indications delivering a higher return on investment.

“IBP puts pressure on budgets and price”: A few respondents explained that they believed IBP would increase prices in general. If this were the case, the resultant pressure on payer budgets would be a negative consequence of IBP.

Some respondents explain that their responses are conditional upon other factors. Various “conditions” for IBP were put forward, including: a single payer health system, a required change in the financing of medicines; and the need to test IBP in a pilot programme. In addition, in settings where there is already a weighted average price, it was suggested that IBP would only be beneficial if there is a mismatch between assumed utilisation in price negotiations and real utilisation in practice.
3.2 Understanding of IBP

Transforming policy requires a shared understanding among stakeholders of the options for reform. As shown in Figure 3, respondents rank the pharmaceutical industry as the stakeholder with a better understanding of IBP, only 6% of respondents think that the industry does not understand IBP at all, while 58% think there is a good understanding of IBP among the industry. Patient groups are ranked as having the least understanding of IBP. However, it should be noted that industry represents the largest group of respondents to the survey whereas patient groups are under-represented, which may have influenced our findings.

**FIGURE 3** TO WHAT EXTENT DO YOU THINK THERE IS A BROAD UNDERSTANDING OF IBP AND ITS IMPLICATIONS AMONG RELEVANT STAKEHOLDERS?
4 The potential impacts of IBP

4.1 Potential impacts on patient access, industry, and payers

Who gains?

As an opening question – under a header of “Your thoughts on IBP – respondents were asked “who is most likely to (or does) benefit most from IBP?”. The intention of this broad question was to gauge general perception about who could or does “win” from IBP (specific thoughts around the mechanisms are obtained in later questions). There was a general opinion that all stakeholders could stand to gain from IBP, according to 57% of responses (Figure 4), though this opinion was held relatively more strongly among respondents with no practical experience of IBP (Figure 5). Respondents thought that industry, in particular, may be most likely to benefit (according to 31% of all respondents, and according to 41% of those with practical experience of IBP). Only 3% of respondents considered patients to be the single stakeholder group which stood to gain the most from IBP. Two respondents (3%) believed that no-one gains from IBP (although neither had practical experience of IBP).

By stakeholder group: industry, academic scientists and regulators most commonly selected that “all stakeholders could gain”, whereas most payers consider industry as the stakeholder who is most likely to (or does) benefit the most from IBP (65% of responses). By country, respondents from the UK, Belgium and Spain were most likely to respond that all stakeholders could gain from IBP.

FIGURE 4 WHO IS MOST LIKELY TO (OR DOES) BENEFIT THE MOST FROM IBP?
FIGURE 5 BY YES/NO PRACTICAL EXPERIENCE OF IBP: WHO IS MOST LIKELY TO (OR DOES) BENEFIT THE MOST FROM IBP?

**Sustainable access**

Regarding the impact of IBP in terms of delivering sustainable access to future treatments, almost half of respondents (48%) think IBP would (or does) have a **significant** impact (Figure 6). Only 5 respondents (8%) think there would be no impact. Of those selecting ‘other’ three didn’t know; one said that there is no problem with access to innovation (in France); one said sustainable access would be delivered only if the new indication is lower value; and one said only if the budgetary impact to treat a specific indication is not increased compared to the current system. Results did not differ significantly when considered according to whether respondents had experience of IBP.

By stakeholder, 80% of industry responses and 50% of regulators’ responses consider that there would be a significant positive impact on sustainability of access. For payers, the majority of responses (59%) consider that there would be a small impact on sustainability of access.
FIGURE 6 WHAT IMPACT WOULD (OR DOES) IBP HAVE IN TERMS OF DELIVERING SUSTAINABLE ACCESS TO FUTURE TREATMENTS?

**Patient access**

A core premise of IBP is that new, relatively lower-value indications – that are not reimbursed (or possibly even developed) under a uniform price – could be reimbursed under IBP, thus expanding patient access to beneficial medicinal treatments. Again, there is a positive general opinion of the impact of IBP on patient access (Figure 7). This opinion is reflected in 70% of responses despite the low number of responses ranking patients as the group benefiting the most, as discussed above. This opinion is reinforced with practical experience of IBP and reaches 73% (24 out of 33 responses). On the contrary, a total of 6 respondents (9%) believe patient access is reduced, but this rises to 12% (4 out of 33) among those that have experience of IBP.
**Impact on industry**

In the discussion paper that accompanied the survey, it is explained that IBP could support the development of new indications that may otherwise not have been launched, by encouraging research into further treatment targets. For this question, respondents were allowed to select more than one response. The most selected option was that "IBP would allow industry to optimise R&D spending, and may increase profits" (51% of the total number of options selected: 38 out of 74). 14 respondents (19%) selected "IBP would complicate market access activities unnecessarily", and 2 thought it would have no impact on industry. Among the 20 respondents who selected "Other" (either by itself or in addition to another option), a variety of impacts were suggested, including: supporting patient access and launching medicines (especially personalised treatment with the current developments in cancer therapies); supporting efficient management of budgets; aligning industry strategy across international markets; and increased competition.

**Impact on payers**

The distribution of responses for the expected impact of IBP on payers is displayed in Figure 8, and shows that 59 out of 61 respondents expect IBP to have some impact on payers. 56% of respondents point to pressure on payer budgets, but these include 21% that consider some alleviation of this pressure in the long-run through lower prices. 21% of respondents thought that IBP would raise expenditure with no meaningful benefits.

The most commonly selected option for Industry and regulators was that IBP would put pressure on payer budget but deliver greater health gains for patients (42% for industry, 50% for regulators). However, for payers, the most common choice (53%) was that IBP would raise expenditure with no meaningful benefit.

The number of responses under the miscellaneous choice ‘other’ is important, comprising 20% of responses. These include reference to savings for payers across several indications; complications of market access arrangements; and payers becoming selective in reimbursement of high-value indications.
FIGURE 8 WHAT MIGHT BE THE IMPACT OF IBP ON PAYERS?

4.2 Potential impact on manufacturers’ decisions

The survey included the open question: “Would IBP impact on manufacturers’ decisions about how and when to bring new indications to market?”.

According to survey respondents, there are two main ways in which pricing arrangements can affect manufacturers’ decisions: 1. R&D decisions about the development of the product, and 2. Decisions around product launch.

**R&D decisions**

Explanations of the various ways in which IBP would impact R&D were provided, with general opinion aligning with a response that states:

“[Implementing IBP would] send the right signals to the pharmaceutical industry, encouraging investment in R&D across indications to maximise medicine use and exploring all viable data routes during trials”

Other aspects raised relate to sequencing in R&D, whereby IBP would diminish the problem that arises from a uniform pricing scenario if a new indication undermines the (price of) indications already on the market. In addition, there was reference to the unlikely development of a new indication that would be marketed at a low price if generics have entered the market for this indication. A few respondents thought that IBP will incentivise selection of the most profitable or high price indications.

**Launching decisions**

Most responses concerned the sequencing of indications (already referenced in the previous section) but at the point of commercialisation. In a uniform price setting, manufacturers may be forced to reduce the price for existing indications, which could discourage or delay the decision to launch.
Therefore, many respondents indicated that IBP could have a positive impact on decisions to launch new indications, encourage earlier launches, accelerate pricing negotiations, or make launches viable in more countries. In particular, this is emphasised for medicines affecting small patient populations. As one respondent remarks:

"IBP would enable launches across all indications (provided cost-effective) as and when clinical development is completed. Manufacturers would no longer have the incentive to delay launching medicines for small patient populations with differential value."

Some respondents believed that IBP would further incentivise the launch of high price, profitable indications, in line with the expectations of shareholders, and that this could reduce coverage. One respondent expressed their opinion that IBP would delay launch due to the required use of multiple confidential prices or discounts.

The relevance of national-level IBP in the context of launch decisions for global pharmaceutical companies was questioned by some respondents, with one respondent commenting that IBP must be adopted in a consistent manner across jurisdictions for it to have an impact.

4.3 Unintended impacts

In response to the question "Is IBP likely to have any unintended consequences?" the free-text answers from the 54 respondents who completed this question can be summarised under the following four themes:

**Value:** The introduction of IBP offers significant opportunities for the delivery of (and dialogue around) value-driven healthcare in general.

**Prices:** There was reference to a reduction in price transparency, and the impact on international reference pricing which can also act as a barrier to the adoption of IBP (if IBP is applied at the list price level).

**Access and uptake:** Some respondents believed that IBP would lead to lower uptake in indications with the highest value (and therefore price), which would have a detrimental impact on equity of patient access in markets dominated by differentiated insurance offerings.

**Added complexity and administrative burden:** Remarks included complications to the procurement process as well as pricing and reimbursement review. Off label use and "utilisation leakage" were mentioned, whereby the medicine's use does not align with the relevant indication-price. In addition, including indication information on a prescription could add complexity and may also have implications for stock management.
5 Implementation of IBP

5.1 Optimal implementation of IBP

Considerations around the implementation of IBP need necessarily entail consideration of relationships between stakeholders, negotiation with partners, societal preferences, etc., which would all be country- and context-specific. However, there are various broad models of implementation that could be considered, which survey respondents were asked to "select", to get an idea of the preferences of respondents. A weighted average single price appears to be the most popular way to implement IBP, accounting for 32% of responses, as shown in Figure 9. The second and third choices were differential list prices, and the use of different brand names, while the choice of a price based on the individual patient-level outcome was only considered optimal by 3% of respondents.

Within stakeholder groups, there does not appear to be a single preferred form of implementation of IBP for industry, payers, or regulators. The responses from each of these groups result in an equal balance between weighted average single price and some other form (different brands for payers, differential list prices for regulators, and "other" for industry).

A total of 22% of respondents selected "other"; just over half of these mentioned a single list price, but a different net price based on the value of each indication, proposing confidential discounts based on real use of each indication or determined by patient-level outcomes. This approach could be achieved through either financial or performance-based managed entry agreements.

One respondent argued that the use of a single weighted average price may not be desirable or feasible, arguing that this would force down the price associated with high-value indications through prescribers switching from a product with a unique high-value indication (to which a high price is attached) to competitor products which treat several indications (for which the “average” price is therefore lower). This would represent a win for payers but may limit the viability of high-value indications being developed. Arguments against the use of differential list prices (aligned with value for each indication) included the prohibitive complexity associated with international reference pricing.
Respondents were asked: “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?” We summarise 50 total responses by country:

**Policy priority in the UK**

There were 11 responses for the UK. Two respondents consider that IBP is not a priority because “[..] IBP causes operational issues which the NHS doesn’t want” or “[..] at the moment the NICE process is not broken”. Other responses remark on a setting allowing and/or requiring price flexibility:

- “The 2019 Voluntary scheme (PPRS) for the next 5 years includes a role for flexible pricing, but the previous iteration did also without major changes happening.”
- “[..] The need for flexibility in pricing models is especially important in oncology due to the increasing number of multi-indication treatments, which will demonstrate different types of value for different tumour types and present the need for a responsive pricing system. Without this flexibility, there will be increasing challenges in patients accessing new cancer treatments in the UK.”

Some respondents argue that IBP should be a priority, referencing the need for greater pricing flexibility as a result of more personalised medicine, the increasing number of medicines which are
licensed to treat multiple indications, to help address the financial sustainability challenge, or to increase patient access and modernise the UK’s approach.

**Policy priority in Belgium**

None of the respondents from Belgium see IBP as a particular policy priority, perhaps because IBP is already possible in Belgium (for example through managed entry agreements):

> "At the lower end of prioritisation as there are already some mechanisms available if payers would like to implement IBP. Price setting and evolving to a demand-driven system are of greater priority".

**Policy priority in France**

None of the respondents from France indicated that IBP is currently a policy priority; one respondent states that IBP is already permitted in France, and another that there is no restriction on access to medicines in France.

**Policy priority in Spain**

Respondents to our survey from Spain indicated that IBP is not a policy priority currently. For example, it was stated that "[...] regulators are still trying to implement a system of value-based price-fixing", or that "[...] it is perceived as mainly a preoccupation for the industry."

**Policy priority in Switzerland**

Most Swiss respondents considered that whilst IBP does not currently feature as a policy priority, it might in the future.

**Political priority in other countries**

Most responses referring to current priorities in “other” countries do not consider IBP among them. In the U.S.: “it is a moderate priority. Managing cost trends, movement to value and outcomes-based payment and risk-based payments are a higher priority”. In Canada: “IBP is a lower priority due to lack of understanding of the benefits which accrue i.e. linking value with price, and the need to secure additional funding in the short term”. According to one respondent in Germany “IBP is currently not a health policy priority. Drug prices are (currently!) not a priority, and if the politicians did care it would be for Gene Therapies, CAR-T and combinations in oncology first.”

Other experiences include "... [In Norway] There is also some debate on whether it is feasible to limit HTA-evaluations to x-number of indications before doing volume-negotiations.” One respondent from Argentina stated that it not a priority in low- and middle-income countries in general.

Representing the opinion that IBP should be a priority, one respondent from Australia states:

> “It is essential because the PBAC is required by law to consider the comparative cost and effectiveness of a drug and cannot recommend a drug or indication for listing on the PBS that it does not consider to be cost-effective. As the effectiveness of a particular drug may vary across indications, the only way to price the drug is on an indication basis.”
5.3 Barriers to the implementation of IBP

Figure 10 shows that – according to survey respondents – there are two principal barriers to the implementation of IBP, each one accounting for 30% of responses: 1. Political will and lack of stakeholder buy-in, and 2. Data infrastructure (technical capacity to collect the information required). Responses to the choice “other” mostly combine several of these barriers, mentioning data collection, and (lack of) credible data that can be used to set up budget impact models. Some trends by country can be observed, notably that in Belgium data infrastructure is cited to be the main challenge, whereas in the UK it is thought to be political will and lack of stakeholder buy-in.

Industry and regulators consider political will and lack of stakeholder buy-in as the main challenge to implementation. Whereas for payers, the most frequent response is the data infrastructure (technical capacity to collect the information required).

What steps could be taken to address these challenges?

There were 47 responses discussing some solutions to the challenges reported in Figure 10. A summary of solutions by barrier is presented below:

1. Political will and lack of stakeholder buy-in

Three representative responses of a total of 15 state:

"Improved communication between industry and payers to share information; Pilot schemes; Sharing of international best practice between payers/Governments; Stakeholder education on the topic of IBP, which can be perceived as a complex topic."
“Multi-stakeholder discussions, focused initially on examples with the highest need. Arranged in a collaborative manner, with carefully selected participants.”

“There is a need to further engage and educate stakeholders and demonstrate that IBP works through scenario modelling and piloting IBP in the real-world using real-world data”

Therefore, most of the responses refer to the need for productive engagement between stakeholders to reach an understanding of the mutual benefits of IBP. One respondent proposed a pilot to be applied to a very select number of drugs which currently have a very high price, through which comparative data could be collected for two scenarios (with and without IBP), demonstrating budgetary impact.

2. **Data collection in terms of burden to the clinical staff providing patient care (effort/workload)**

   There was one US-specific response proposing a solution to this barrier: In the US, doctors are often required to provide information at the time of prescribing, which can either be by “streamline authorities” (using an electronic indication code number, which would be compatible with IBP), or “authority required” (where approval to prescribe is sought from Medicare by phone or in writing, which would not be compatible with IBP). “The solution is to increase the number of drugs listed with “streamline authority” restrictions and reduce the number listed as ‘authority required’ items.”

3. **Data infrastructure (technical capacity to collect the information required)**

   There are several responses pointing out the need for better systems for monitoring exactly which patient/indication a pharmaceutical is being prescribed for. One specific example of a data set proposed to be useful for this purpose is the SACT chemotherapy treatment database in the UK. On the general use of data and IT infrastructure, one respondent stated that “[..] the solutions must reflect local reimbursement systems. The use of electronic health records may facilitate IBP, as well as serialization systems and data networks as well as innovative technologies such as blockchain and artificial intelligence (AI). Defining standardized datasets for measuring health outcomes for priority diseases and conditions, including Patient Reported Outcomes, allows for systematic monitoring, measurement and comparisons across providers, regions and even countries [..]”. One respondent suggests that this would not be an issue if claims data are used to support IBP.

4. **The ability to make changes to the current billing infrastructure for reimbursement of pharmaceuticals**

   Some respondents suggested investment in more sophisticated billing systems (without detailing what these might look like). In general, it was purported that making the case for IBP clear and attractive to health authorities would pave the way and appetite for the requisite changes in the billing infrastructure. On the other hand, one respondent suggested that the most practical and feasible way forward is not to change the billing system at all, but rather to have a single list price with indication-adjusted hidden rebates (monitored by claims data).

5. **Concern about short-term payer impact**

   There were two responses proposing solutions to concerns about payer impact, one who proposed that payers need to be provided with a clear rationale and evidence of the benefits of IBP, and another who recommended advancing in small steps, by pursuing a model that does not require changes to operational reimbursement procedures or workload for clinical staff.
6 Conclusions

Transforming policy – and improving how we incentivise, fund and access beneficial medicines – requires constructive dialogue. A constructive dialogue can be better supported if we understand different stakeholders’ beliefs and perceptions. Through this consultation exercise, we bring to light a range of perspectives on the potential benefits and drawbacks of IBP, and considerations for implementation. The conclusions drawn are not based on any empirical analysis, but rather the opinions of survey respondents. As well as offering a useful platform to guide further research, we hope that the insight provided can support a constructive dialogue and aid a shared understanding among stakeholders of the main issues.

Whilst our sample of 73 survey respondents represented a range of stakeholders and countries, a larger sample and broader representation (for example by more patient groups and medical societies, and from a wider range of countries) would have been preferable. It is also important to acknowledge that our sample is inherently biased; individuals completing the survey will, by and large, be those who have a particular interest in IBP. This is exemplified by the fact that nearly half of respondents had some experience of IBP. However, our analysis of the consultation results brought to light some interesting patterns within our sample. For example, those with experience of IBP were more positive than those without, and industry and regulators were relatively more positive about the prospect of IBP than payers and academics (even though the majority of payers and academics thought some form of IBP would be a good thing). Our results also revealed patterns and differences in how some stakeholders perceive whether others would benefit or not from IBP. For example, 31% of respondents believed that industry was the single stakeholder that would gain the most from IBP, but among payers, this perception was held by 65%. Opinion was similarly divided as to impact on payers, with industry and regulators most frequently citing pressure on payer budget which would be offset by greater health gains for patients, but with a significant number of payers (53%) suggesting that IBP could raise expenditure with no meaningful benefit.

A majority of respondents to this survey argue that IBP would put pressure on payer budgets, resulting from expanded patient access to new medicines, but a subset of these believe that in the long-run IBP will generate competition and lead to lower prices. The majority of respondents to the survey believed that IBP would send the right signals to the pharmaceutical industry by encouraging investment in R&D across indications; this could enable earlier launches and accelerate price negotiations. There was a widespread opinion on the optimal implementation form for IBP; the most popular options among survey respondents were: a single price based on a weighted average of value and usage across indications; a single price with confidential differential discounts by indication; differential list prices aligned with value for each indication; and different brand names for different indications of a single product. The most significant barriers to IBP were considered to be inadequate data infrastructure (in particular capturing actual usage by indication), and political will/lack of stakeholder buy-in; the latter may explain the perception that IBP is not currently a policy priority in most countries.

There was a general perception among respondents that IBP does not appear to be a policy priority in most countries. Yet, many felt that it should be, suggesting it is the only rational way to price a drug whose effectiveness varies across indications. This consultation exercise indicates that the potential benefits of IBP are numerous, and not limited to one individual stakeholder group. Furthermore, IBP may be necessary to future-proof society’s ability to incentivise and benefit from the evolving nature of drug development.
References

Appendix I: Consultation survey

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?

<table>
<thead>
<tr>
<th></th>
<th>Payers</th>
<th>Patient groups</th>
<th>Industry</th>
<th>Regulators</th>
<th>Medical societies</th>
<th>Academic scientists</th>
<th>Consultants</th>
</tr>
</thead>
<tbody>
<tr>
<td>Not at all</td>
<td>☐</td>
<td>☐</td>
<td>☐</td>
<td>☐</td>
<td>☐</td>
<td>☐</td>
<td>☐</td>
</tr>
</tbody>
</table>
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
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.
Appendix II: Respondent characteristics

Total responses and non-response

82 responses were received\(^2\): the initial online survey in English was completed by 40 respondents; both the Spanish and French translations received 7 responses each, and 28 paid responses were collected through the online platform (see footnote in Introduction). Although we did not want to restrict responses by making all questions compulsory, 9 respondents completed no questions beyond the initial two compulsory ones and were therefore excluded. The analysis consequently includes only 73 responses. Four respondents completed only the two compulsory questions and “would some form of IBP be a good thing?” (3 Yes, 1 No). We have included these 4 responses in the analysis. Total response rates for each question are reported in our analyses.

Respondents by country, stakeholder and experience of IBP

Three identifiers - country, stakeholder type, and experience of IBP - differentiate sub-groups of respondents. We present our analysis for the UK, Belgium, France, Spain, Switzerland and “Other” (which includes 11 countries providing 24 responses). Stakeholder types are reported according to payer, industry, regulator, academic scientist and “other”. Tables A1 and A2 show the distribution of responses by country and stakeholder type respectively.

Table A1 In what country do you live and/or work professionally?

<table>
<thead>
<tr>
<th>Country</th>
<th>Number</th>
<th>Percent</th>
</tr>
</thead>
<tbody>
<tr>
<td>UK</td>
<td>17</td>
<td>23.29</td>
</tr>
<tr>
<td>Belgium</td>
<td>14</td>
<td>19.18</td>
</tr>
<tr>
<td>France</td>
<td>7</td>
<td>9.59</td>
</tr>
<tr>
<td>Spain</td>
<td>6</td>
<td>8.22</td>
</tr>
<tr>
<td>Switzerland</td>
<td>5</td>
<td>6.85</td>
</tr>
<tr>
<td>‘Other’</td>
<td>24</td>
<td>32.88</td>
</tr>
<tr>
<td>Australia</td>
<td>4</td>
<td>5.48</td>
</tr>
<tr>
<td>Germany</td>
<td>4</td>
<td>5.48</td>
</tr>
<tr>
<td>US</td>
<td>4</td>
<td>5.48</td>
</tr>
<tr>
<td>Italy</td>
<td>3</td>
<td>4.11</td>
</tr>
<tr>
<td>Costa Rica</td>
<td>2</td>
<td>2.74</td>
</tr>
<tr>
<td>Norway</td>
<td>2</td>
<td>2.74</td>
</tr>
<tr>
<td>Argentina</td>
<td>1</td>
<td>1.37</td>
</tr>
<tr>
<td>Canada</td>
<td>1</td>
<td>1.37</td>
</tr>
<tr>
<td>Israel</td>
<td>1</td>
<td>1.37</td>
</tr>
</tbody>
</table>

\(^2\) Responses were registered if the respondent submitted information on at least the two compulsory survey questions: 1. “What stakeholder group do you belong to or represent?”; and 2. “In what country do you live and/or work professionally?”
The UK had the largest number of respondents, followed by Belgium and France. The questionnaire permitted respondents to identify as more than one type of stakeholder. However, only 8 selected more than one type; for practical purposes we ascribed those respondents to the (single) stakeholder group (among the types selected) with the largest number (industry, payers and regulators).

<table>
<thead>
<tr>
<th>Stakeholder</th>
<th>Number</th>
<th>Percent</th>
</tr>
</thead>
<tbody>
<tr>
<td>Industry</td>
<td>27</td>
<td>36.99</td>
</tr>
<tr>
<td>Payer</td>
<td>20</td>
<td>27.40</td>
</tr>
<tr>
<td>Regulator</td>
<td>12</td>
<td>16.44</td>
</tr>
<tr>
<td>Academic</td>
<td>7</td>
<td>9.59</td>
</tr>
<tr>
<td>Other</td>
<td>7</td>
<td>9.59</td>
</tr>
<tr>
<td>Total</td>
<td>73</td>
<td>100</td>
</tr>
</tbody>
</table>

The largest stakeholder group was industry; 70% of industry respondents were from the UK or Belgium (37% and 33% respectively). This was followed by payers, regulators and academics; the group “other” mostly represented consultants and pharmacists with various responsibilities including hospital care and procurement.

Respondents were asked whether they had practical experience of IBP (no specific criteria were given as to what this entailed, and therefore this was left to respondents’ interpretation). Respondents were relatively evenly split. Practical experience of IBP was reported by 47% of respondents; 37% reported that they had no practical experience, with 16% who did not answer this question.

Figure A1 presents the breakdown by type of stakeholder, which shows that practical experience amongst our respondents is more common amongst industry, payers and the miscellaneous group (largely pharmacists and consultants).
The breakdown of practical experience of IBP by country is presented in Figure A2, which demonstrates that experience of IBP is more common in Switzerland and Belgium, and less common in France and Spain.
About us
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.

OHE. For better healthcare decisions.

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- 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