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ADDRESSING HEALTHCARE CHALLENGES:

Principles on the Transparency of Evidence from Novel Pricing and Payment Models

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Summary

When used appropriately, novel pricing and payment models bring benefits to patients and healthcare systems by improving access to innovative treatments. Some types of novel pricing and payment models involve the collection of outcomes data in order to address uncertainties about the effectiveness of a treatment. Evidence resulting from the collected data could also indirectly benefit patients, physicians and the scientific community, when collected as part of a protocol, interpreted and used in line with good scientific principles. However, unless a separate scientific study is conducted, currently the evidence generated through these models is often not disclosed.

Recognising the potential for improved patient care, the pharmaceutical industry stated its commitment to work with stakeholders under the Transparency Principle of EFPIA's report on *Addressing Healthcare Challenges: Novel Pricing and Payment Models*. Greater transparency of novel pricing and payment models can be achieved through the disclosure of the resulting evidence in an appropriate manner. The type of evidence to be disclosed should be mutually acceptable to payers, including Health Technology Assessment (HTA) bodies, and the company and it should be interpreted considering the limitations in the underlying outcomes data and the context for data collection.

We consider the merits of evidence disclosure at two points in time: first, when agreeing a novel pricing and payment model, the nature of this model and the protocol for data collection and analysis could be disclosed; and, second, after the conclusion of the data collection and its analysis, the resulting evidence could be disclosed. The merits of disclosure will vary from agreement to agreement and from country to country, but there are six common principles that should be applied to any discussion regarding the greater transparency of novel pricing and payment models:

- **Rationale:** The rationale for collecting outcomes data through the novel pricing and payment model, the questions that the outcomes data and evidence aim to address, and the stakeholders that would benefit from this evidence should be documented
- **Mutual Agreement:** The type of information to be disclosed, the timeline and the stakeholders to whom it will be disclosed should be agreed between the payers and the manufacturer when negotiating the terms of the novel pricing and payment model
- **Data Quality:** Evidence made transparent should be based on high-quality outcomes data, collected through a clear research protocol, in line with accepted scientific standards, and representative of the agreed patient population
- **Context of Data Collection:** The context of data collection, the limitations of the outcomes data and the resulting evidence, and assumptions in the data analysis need to be disclosed
- **Data Interpretation and Use:** Disclosed evidence should be used according to good procedural practices to ensure that it is accurately interpreted
- **Patient Confidentiality:** Patient confidentiality must be maintained when disclosing any information about the novel pricing and payment model, in compliance with the European General Data Protection Regulation (GDPR)

Payers, policy makers and the industry should collaborate to improve the transparency of novel pricing and payment models and ensure that the resulting evidence benefits patients and healthcare systems.

Contents

The value of disclosing evidence from novel pricing and payment models to patients, physicians and the scientific community

The industry is supportive of the development of novel pricing and payment models. When used appropriately, they can bring benefits to patients and healthcare systems by improving access to innovative treatments. Novel pricing and payment models are primarily mechanisms to improve patient access and address any uncertainties about the value of a new technology identified by payers and HTA bodies (hereby jointly referred to as 'payers'), while enabling risk sharing between payers and innovators. Some types of novel pricing and payment models involve the collection of outcomes data (often observational) from which evidence on the medicine's effectiveness can be derived. This evidence can bring benefits to patients, physicians and the wider scientific community by:

- **Providing insights for clinical practice and life science research** on the real-world performance of a treatment and those patients most likely to benefit
- **Increasing the sustainability and efficiency of health systems** through information about the optimal use of therapies over their lifecycle
- **Providing further insights on the value of medicines** to patients, healthcare systems (including regulatory and HTA bodies) and the scientific community

The evidence generated using outcomes data developed through a novel pricing and payment model is not always publicly disclosed, unless a separate scientific study is conducted. There are several reasons for this. It could be that there is a precedent of applying confidentiality requirements that is commonly followed. There are sometimes concerns regarding the quality of the underlying data and whether it is fit for addressing wider questions beyond those asked by the reimbursement body. The lack of transparency leads to the concern that novel pricing and payment models are opaque and to uncertainty whether they are truly a win-win solution for payers, companies and patients.[1] There is a growing debate for greater transparency of the process from which they arise and information about the models including the resulting evidence.[2,3,4]

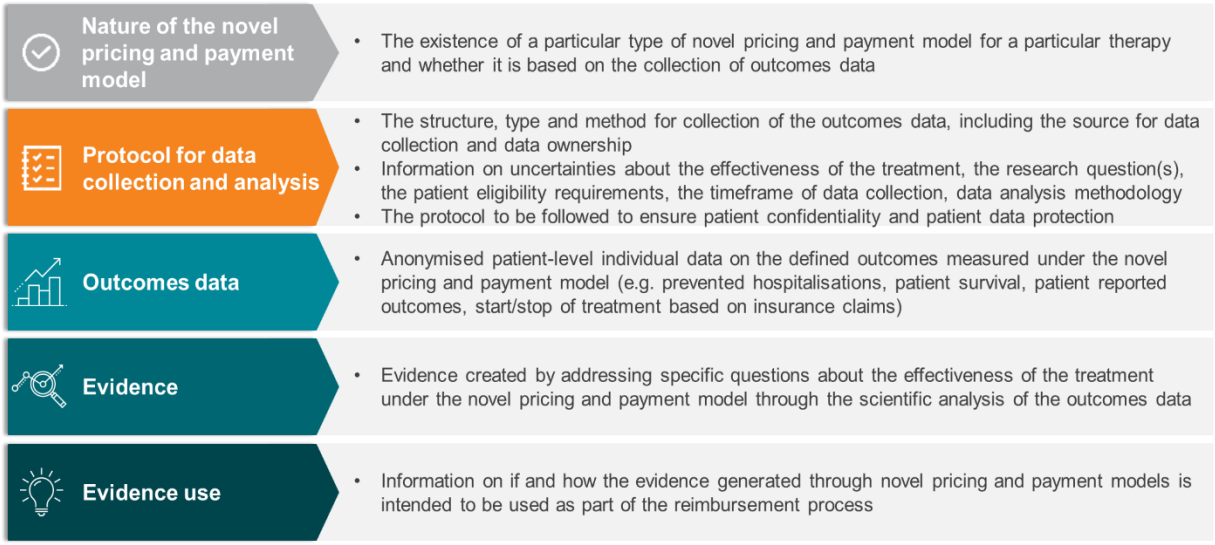
Practices relating to the disclosure of evidence from novel pricing and payment models differ across European countries and are rapidly evolving over time.[5,6,7] As part of the ongoing debate regarding transparency, some stakeholders have questioned **the current level of confidentiality** associated with novel payment and pricing models. For example, it is argued that confidentiality restricts the potential for cross-country learnings from such agreements, given the current limited experience and the ability of patients to engage in the process.[8] **Confidentiality of the evidence could also be a barrier for rigorous independent evaluation**, limiting the opportunity for external review and leading to challenges in ensuring its appropriate interpretation, uncertainty whether payers are getting a good deal in the long term, and failure to ensure the public accountability of decision making.[9,10,11,12] Equally, there are **challenges with ensuring disclosure of the evidence**. Some of those are recognised in the literature – the underlying data may be of poor quality so that the resulting evidence might

not be suitable for publication in a peer-reviewed setting[13,14] and may be difficult to translate across different healthcare settings.[15,16] **The debate highlights the need for specific principles that govern the transparency of evidence generated through novel pricing and payment models**.[17,18] This paper addresses this debate and develops further the Transparency Principle in EFPIA's recent publication on *Novel Pricing and Payment Models*[19] by outlining a set of principles to govern the approach to disclosing the resulting evidence and achieving greater transparency. In addition, the paper incorporates learnings from current standards and practices on the disclosure of real-world evidence from observational studies and evidence from randomised controlled trials (RCTs).

Transparency definitions and scope of evidence disclosure

Novel pricing and payment models are agreements between innovators and payers used in specific cases to improve patient access to innovative medicines. In some cases, novel pricing and payment models can involve the collection of outcomes data. Unlike data from RCTs which aim to establish a treatment's efficacy and safety, evidence collected under novel pricing and payment models aims to address specific questions and uncertainties about the effectiveness of a treatment limited to its use in a defined patient population or a defined real-world clinical setting. The data collected under novel pricing and payment models might also be more high level, in line with requirements by payers, compared to the detailed safety and efficacy data collected under RCTs required by the regulator to grant marketing authorisation. Different types of outcomes data could be collected tracking the effectiveness of a treatment over a set timeframe – for example, prevented hospitalisations, survival rates or patient reported outcomes (Figure 1).

Figure 1: Different types of information associated with novel pricing and payment models

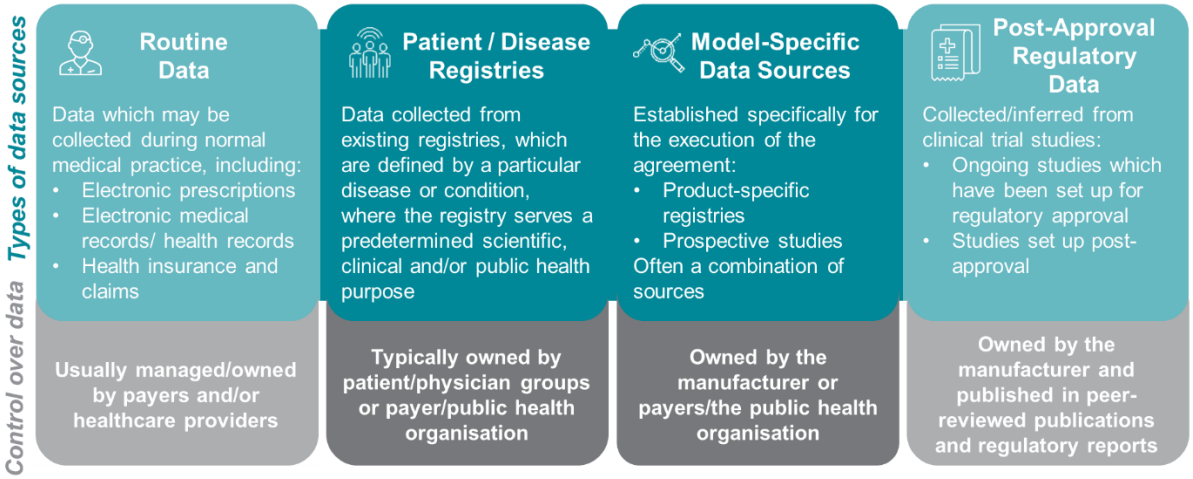


Transparency refers to the overall aspiration to make decisions about novel pricing and payment models publicly accessible in a timely manner with appropriate governance and accountability mechanisms.[20] This paper has a more limited focus and lays out the rationale for the feasibility of achieving transparency through disclosure of different types of information related to a novel pricing and payment model with outcomes data collection – its

existence, the protocol for data collection and analysis including the structure of the outcomes measured and data sources, the evidence based on an analysis of the data, and the intended use of this evidence (Figure 1). Transparency can also be defined by whom the information is disclosed to – patients, physicians, the scientific community, the general public, or the industry. Considering these dimensions, current transparency practices vary among countries.

The decision to disclose the evidence from a particular novel pricing and payment model also depends on who owns the data from which it is derived. In some cases, this will be the company developing the medicine but in others it will be public health organisations, payers, regulatory bodies, professional societies or patient groups (Figure 2). While this is a generalisation, the responsibility for disclosure of evidence resulting from routine data collection belongs to payers or healthcare providers as they have the most control over the data. Although the industry has historically invested in patient/disease registries, these are often owned and managed by patient and physician groups. Post-approval regulatory data, generated through clinical trials following the launch of a treatment, is owned by industry; however, its transparency is guided by existing industry principles and not covered in this paper.[21] **The principles in this paper focus on outcomes data and resulting evidence analysed specifically for the execution of the novel pricing and payment model** which might span patient/disease registries and model-specific data sources depending on the case.

Figure 2: Novel pricing and payment models rely on the following simplified data sources and the principles in this paper focus primarily on model-specific sources of data



Note: This figure is a simplification of the potential data sources and types of stakeholders with control over the data for the purpose of drawing clear definitions in support of the paper. Rather than representing distinct categories, there might be a cross-over between different sources such as, for example, novel pricing and payment models relying on data collected as part of existing patient/disease registries.

In line with the definitions and scope, information generated from novel pricing and payment models could be disclosed at two timepoints. First, **the nature of the novel pricing and payment model and the protocol for data collection and analysis could be disclosed at the**

point of agreeing a model. As outlined in EFPIA's *Novel Pricing and Payment Models*[22] paper, **commercially confidential financial information regarding the novel pricing and payment model should remain confidential**. Second, in cases where the outcomes data is collected in line with a clear protocol for data collection, the resulting evidence could be disclosed once the outcomes data analysis is complete. The processing of **individual-level outcomes data must comply with local legislation and GDPR** (Figure 3).

Figure 3: Opportunities for disclosure along the novel pricing and payment model timeline

Stakeholders to whom the evidence is disclosed need to be defined through the negotiation process for the novel pricing and payment model, as this may differ between agreements. **The disclosure of information on a given novel pricing and payment model should be treated on a case-by-case basis and in line with six disclosure principles** in order to ensure the appropriate quality, use and interpretation of the evidence.

Industry principles to facilitate the disclosure of evidence from novel pricing and payment models

Where the evidence can benefit patients and healthcare systems, every effort should be made to make it accessible, on a case-by-case basis within the following broad six principles:

Transparency Principles

Rationale: The rationale for collecting outcomes data through the novel pricing and payment model, the questions that the outcomes data and evidence aim to address, and the stakeholders that would benefit from this evidence should be documented

Mutual Agreement: The type of information to be disclosed, the timeline and the stakeholders to whom it will be disclosed should be agreed between the payers and the manufacturer when negotiating the terms of the novel pricing and payment model

Data Quality: Evidence made transparent should be based on high-quality outcomes data, collected through a clear research protocol, in line with accepted scientific standards, and representative of the agreed patient population

Context of Data Collection: The context of data collection, the limitations of the outcomes data and the resulting evidence, and assumptions in the data analysis need to be disclosed

Data Interpretation and Use: Disclosed evidence should be used according to good procedural practices to ensure that it is accurately interpreted

Patient Confidentiality: Patient confidentiality must be maintained when disclosing any information about the novel pricing and payment model, in compliance with GDPR

Rationale: The rationale for collecting outcomes data through the novel pricing and payment model, the questions that the outcomes data and evidence aim to address, and the stakeholders that would benefit from this evidence should be documented

- In order to ensure that the evidence is fit for purpose, addresses the underlying uncertainty and provides the most value if disclosed, stakeholders should be clear and transparent on the rationale for implementing the novel pricing and payment model and the protocol for data collection and analysis. How the evidence aims to address the underlying uncertainties should be clear and could be communicated upon agreement of the novel pricing and payment model alongside the nature of the model and the protocol for data collection and analysis.
- The manufacturer and payers need to jointly set out the specific questions that the novel pricing and payment model tries to address within the indicated care setting and patient population and how that is going to be achieved through the collection of data on specific outcomes (e.g. to improve payers' certainty of the effectiveness and value of the medicine).

Mutual Agreement: The type of information to be disclosed, the timeline and the stakeholders to whom it will be disclosed should be agreed between the payers and the manufacturer when negotiating the terms of the novel pricing and payment model

- The disclosure of evidence derived from novel pricing and payment models should always be based on the mutual agreement of payers and the manufacturer. Negotiations typically cover important elements of the novel pricing and payment model including its structure, the protocol for data collection and timeframes for evidence collection and analysis, and data sources to be used. In addition, such discussion should also consider the transparency of the agreement and the potential disclosure of resulting evidence. Where appropriate and possible, payers and the manufacturer should consult relevant healthcare professionals and/or patient representatives.
- As part of the negotiations, payers and manufacturers should agree on whether the protocol for data collection and analysis and which elements of the analysed evidence are to be disclosed. Stakeholders who would benefit from having access to the evidence should be clearly defined. Discussions should consider local legislation and any requirements for the agreement to remain fully confidential. Parallel regulatory or academic processes also need to be considered so that disclosure does not hinder or conflict with any planned dissemination of the evidence in an academic setting.
- Negotiations should also consider ownership of the outcomes data and the resulting evidence. Since sources may be owned/controlled by the manufacturer, payers or a third party, the appropriate permissions for the disclosure of the evidence need to be in place. This implies information sharing between the parties involved in the novel pricing and payment model and ensuring consensus on the appropriateness of disclosing the resulting evidence. Transparency should be reciprocal where possible. Other stakeholders with control over data should also make every effort to disclose the resulting evidence in a timely manner.
- Payers and the manufacturer should agree on the appropriate timeframes for the disclosure of information. As mentioned, the nature of the novel pricing and payment model and the protocol for data collection and analysis could be disclosed upon completion of the negotiations, whereas the evidence could only be disclosed once the data collection and analyses are concluded.
- To address the above considerations, a governance process could ideally be established to enable payers and the manufacturer to mark evidence that should remain confidential (e.g. because the underlying data is of low quality or if the evidence is scientifically confidential) as well as a dispute resolution mechanism to resolve any misalignment.
- To improve the overall transparency and ensure the accountability of the process, payers and the manufacturer should also discuss the feasibility of disclosing their intention of how the evidence derived through an agreement is intended to be used.

- **A good practice example** can be observed in England. Prior to the publication of the National Institute for Health and Care Excellence's (NICE) assessment, the manufacturer can mark data as 'scientifically in confidence'. NICE reviews this and may negotiate with the manufacturer on which data is disclosed in the final report. This ensures a trusting relationship between the parties involved.[23]

Data Quality: Evidence made transparent should be based on high-quality outcomes data, collected through a clear research protocol, in line with accepted scientific standards, and representative of the agreed patient population

- Outcomes data collection under most novel pricing and payment models does not occur in a controlled environment as with interventional RCTs. Instead of establishing clinical efficacy, the aim is to capture the real-world performance of a treatment and in some cases simply to administer a novel pricing and payment model rather than to address any specific questions.
- Several factors can affect the rigour and robustness of the data collection process and the data itself, which should be considered to ensure these do not impact the quality and comparability of the resulting evidence. For example, there may be variation across care settings in the data collection infrastructure, regional variation in the number of patients treated leading to insignificant results (if there are too few patients), or challenges for physicians to follow a strict data collection protocol given competing demands leading to incomplete data.
- Payers, the manufacturer, and healthcare professionals have a shared responsibility in ensuring that any evidence disclosed is based on data of sufficient quality and statistical power. This can be achieved by ensuring data collection is based on a clear protocol, but also by considering the accuracy of the data, its completeness and any gaps, timeliness, and the appropriateness of the patient population including the sample size.
- Data should be collected according to accepted scientific standards. The International Society for Pharmacoeconomics and Outcomes Research (ISPOR) and International Society for Pharmacoepidemiology (ISPE) good practices could be applied to maximise the chances of high-quality data collection and hence ensure that the resulting evidence is suitable for disclosure:[24]
    - **Prior to data collection:** While the data collection process should build on existing infrastructure, a clear, simple and consistent data collection plan must be established, ensuring physicians' support. Efforts could be made to improve the quality of the evidence by leveraging standardised electronic health records.
    - **Upon completion of the novel pricing and payment model:** Grading of Recommendations, Assessment, Development and Evaluations (GRADE) criteria could be used to score the strength of evidence, or an independent process/third party stakeholder could review and ensure the results are of high quality. Use of data analytics can also improve interoperability between datasets and minimise the impact of outliers or poor-quality data points on the resulting evidence.
- Further practical steps such as providing incentives to healthcare professionals would ensure complete and accurate data entry by compensating them for the time spent in

filling out the required data. In addition, implementing routine check points could ensure the patient's confidence in the therapy decision and therefore improve adherence.

- **A good practice example** can be observed with Healthcare Quality Registries in Sweden, where several measures are used to improve data quality and validate the data. Automated checks prevent the input of incorrect data and data is compared across other government-administrated registries, which can also help to assess the completeness of the data.[25]

Context of Data Collection: The context of data collection, the limitations of the resulting evidence, and assumptions in the data analysis need to be disclosed

- The appropriate context of the data collection and the limitations of the evidence itself should be provided to ensure that the evidence is accurately interpreted by external stakeholders. In addition to communicating the purpose of the data collection and uncertainties addressed, the following elements can be specified to clarify how the medicine is used in a specific country:
    - **The line of treatment for which a specific product is used**, which may differ across countries, guided by local treatment guidelines.
    - **Physicians' prescribing behaviour**, which may be guided by individual experience, for example, switching from one product to another, leading to further variance in the recorded outcomes within a country.
    - **Patient eligibility requirements**, because the treated population in one country may be different to that treated in another based on local reimbursement criteria.
    - **The observed patient adherence**, as this may differ between patient groups, and impact patients' response to the treatment.
- In addition, assumptions made in analysing the data and the data sources used should be clear. For example, countries may have different data infrastructure systems which could lead to variability. When surrogate endpoints or proxy measures are used, additional clarifications may be required regarding the extrapolation of the resulting evidence to infer the product's performance.
- While recognising that in some circumstances it might be challenging to collect information on the context in which data is collected, stakeholders should make every effort to include these points in the protocol for data collection and analysis, and ensure they are captured. Disclosure of any evidence should be accompanied by the context of data collection and limitations in the evidence.
- **A good practice example** can be observed for Managed Access Agreements (MAAs) in England. A data collection plan is published through the NICE website, which details the context and purpose of the model, the protocol, planned analyses, patient eligibility and sources of data used.[26]

Data Interpretation and Use: Disclosed evidence should be used according to good procedural practices to ensure that it is accurately interpreted

- Policy makers aiming to use the disclosed evidence should be made aware of the limitations in the data collection process and caveats in the resulting evidence. Furthermore, they should assess the relevance and accuracy of the evidence according to existing good procedural practices, e.g. as set out by ISPOR/ISPE and ISPOR/the Academy of Managed Care Pharmacy (AMCP)/National Pharmaceutical Council (NPC).[27,28]
- While there may be a tendency to compare evidence from outcomes data (derived from observational study approaches) with evidence from RCTs, the relative value of the two needs to be weighed for the specific questions asked when making product-specific decisions. The former is collected in a less structured environment and is to a greater extent impacted by external contextual factors. It is also associated with different analytical challenges such as risk of bias or incomplete data. This makes direct comparisons between evidence from novel pricing and payment models and that resulting from RCTs difficult. Nevertheless, outcomes data collected and analysed as part of novel pricing and payment models can provide credible evidence of product effectiveness and safety.

Patient Confidentiality: Patient confidentiality must be maintained when disclosing any information about the novel pricing and payment model, in compliance with GDPR

Patient confidentiality must be ensured in line with GDPR.[29] Among the strategies that should be applied are:

  - Stakeholders should ensure that there is an appropriate protocol to ensure patient confidentiality and that no evidence is disclosed if it puts at risk patient confidentiality, especially in cases where there is a very small patient population.
  - If personal data from novel pricing and payment models is to be disclosed, patients should be informed about where the evidence will be disclosed and who will have access to it.
  - Wherever possible, stakeholders should seek to ensure that privacy concerns are approached consistently both within and across markets.
- **A good practice example** can be observed in France under the Temporary Authorisation for Use (ATU) programme. Participating patients are notified about the process and how their data is going to be treated. Patient-level data is anonymised and stored in a central database. The data remains confidential (limited to the treating physician and relevant authorities for pharmacovigilance purposes) and only an aggregated analysis is referred to during the HTA.[30]

Building stakeholder consensus on transparency

The industry recognises that real-world evidence based on outcomes data collected under novel pricing and payment models can potentially bring benefits to patients, physicians and the wider scientific community, when the above principles are applied. While this paper outlines the industry's position on the transparency of novel pricing and payment models, a cross-stakeholder dialogue and consensus on the issue is also needed.

The industry continues to support the development of data collection infrastructure across countries to enable the use of novel pricing and payment models to improve patient access. However, health systems have varying capabilities for collecting high-quality outcomes data under novel pricing and payment models that result in robust evidence. This can disadvantage some countries and potentially act as a barrier to patient access and transparency.

To overcome this challenge, open collaboration and a shared commitment among payers, policy makers and the industry are required with the involvement of healthcare professionals and patient organisations. Transparency discussions are also needed, building on existing debates regarding the use of real-world evidence. Topics for further alignment include who can have access to the evidence (e.g. physicians, researchers, patients, payers, and/or the general public), the process of how the evidence is going to be used in the decision making process, and potential platforms for its disclosure. Such platforms can build on existing efforts across the European Union (EU), for example the Health Outcomes Observatories, which aims to collect and standardise patient reported outcomes data, and the European Health Data Space, which provides funding for investments in data infrastructure and promotes improved exchange and access to different types of health data.[31,32] Further to these efforts, policy makers and the industry need to agree on a set of common principles for the disclosure of evidence from novel pricing and payment models, and mechanisms to improve their transparency. The industry therefore commits to:

1. Working constructively with the European Commission and other stakeholders with the intention to align on **principles for the disclosure of evidence from novel pricing and payment models**.
2. **Continuing the discussion at the Member State level with an overall objective to improve the transparency of novel pricing and payment models.** This could start with a discussion with policy makers and payers at the Member State level **to develop a transparency mechanism that promotes good governance and accountability**, building on the broader flagship initiative of the European Commission on ensuring affordability of medicines.[33] Such a mechanism has already been implemented in some EU Member States, such as Belgium, and can provide useful learnings for future state level discussions.[34] Further topics for discussion include the most appropriate platform for data collection, the mechanisms of disclosure of the evidence, including stakeholders with access to the evidence, and how this evidence is going to be used.

The current paper and its principles represent a starting point to continue the debate on transparency, so that the potential benefits of outcomes data and the resulting evidence from novel pricing and payment models are realised by patients and healthcare systems.

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