Access to innovative treatment through

HPR166

Performance-based contracts in France

— Recommendations from multidisciplinary experts' workshops

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Introduction

- Financing access to medical innovation under increasing budgetary constraints is challenging.
- Randomized controlled trials are carried out on selected populations. However, healthcare payers aspire to commit public resources based on product's real-life value, calling for continuous evaluation in the real-life setting.
- In this context, the so-called performance-based contracts (PBC) between manufacturers and payers are gaining traction to ensure access to medical innovation when uncertainty remains too high.
- The aim of PBC contracts is to align the price and reimbursement levels with the real-life performance of the product.[1]
- In France, prices and eventual PBC are negotiated between manufacturers and the *Comité Économique des Produits de Santé* (CEPS).[2]
- Negotiations are governed by a framework agreement for chronic treatments, revised every 3 to 4 years.[3]
- However, despite this legal-administrative framework that outlines PBC negotiations, French experience has been mixed so far.[4]
- Some contracts have not satisfactorily addressed the CEPS's expectations or did not even meet their initial engagement, raising up a form of mistrust towards PBC.[5]

Methods

- Multidisciplinary group of 12 experts discussed opportunities and challenges of PBC, with a view of drawing up concrete recommendations for the implementation of PBC in France.
- Experts panel: 3 patients organizations representatives, 2 clinicians, 4 academics (public health, health law, health economics), 1 think tank representative, 1 former member of the French HTA agency (HAS), and 1 former member of the CEPS. Two pharma industry representatives also attended the discussion (Fig. 1).
- Four sequential workshops: 1) Situation analysis and definitions, 2 & 3) recommendations listing, 4) practical and hypothetical cases discussion.

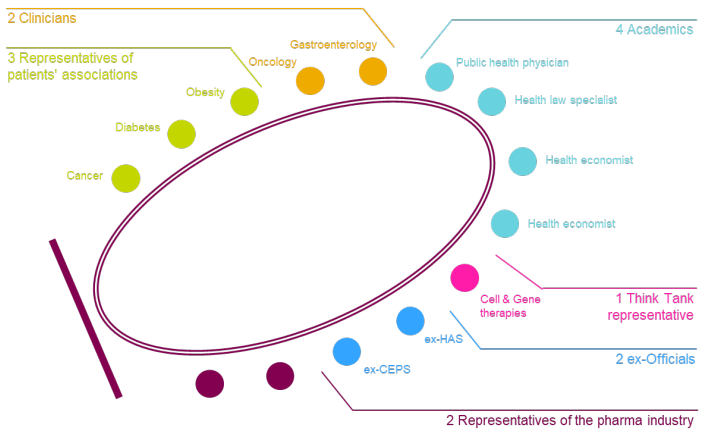

Fig. 1 The experts' workshop composition

Objective

- **To seek recommendations from multidisciplinary experts in the future set-up of performance-based contracts (PBC) in France.**

Results

- Participants agreed on the pitfalls and prerequisites of PBC, including a clear characterization of the uncertainty and a performance variable that can be easily objectivized from reliable information systems.
- Consensus was also reached on the need to distinguish between single-dose and repeated-dose treatments (*i.e.*, chronic treatments).
- However, the very notion of performance was not understood and defined in the same way by all. Differences in perception between stakeholders have emerged, with patients/clinicians on one side and health authorities on the other.
  - — The former embraces dynamic and real-life evaluations based on patient-reported outcomes (PRO) and day-to-day disease management *i.e.*, a micro-level.
  - — The latter focuses on the demonstration of efficacy, safety, and efficiency with robust methodological requirements and under budget constraints *i.e.*, a macro-level (Table 1).
- Reconciling the two seems challenging with the current legal-administrative framework. However, it should be kept in mind that this framework allows for the implementation of service-based agreements.
- Indeed, several stakeholders would be eager to have management and clinical decision support tools (*e.g.*, patient follow-up and education, therapeutic compliance support). Opportunities exist to meet a whole range of clinical needs, and to initiate dialogue on new types of financing agreements based on services and/or decision-support tools.
- This type of agreement would hold the potential to meet both micro and macro-levels stakeholders' perspectives.

Table 1 Two perspectives in the setting of PBCs

	Micro-level	Macro-level
Objective	- Fast and fair access to innovation	- Global efficacy assessment - Healthcare expenditure control
Performance	- Continuous evaluation in routine practice	- Transposability of clinical trials results into the real-world setting
Uncertainty	- Day-to-day practice, usage and experience	- Real-world effectiveness
Risk	- Product safety - No access or access restriction to some treatment	- Product safety - Financial risk for the healthcare system
PROMs/PREMs	- Co-primary endpoints	- Secondary/exploratory endpoints
Variable of interest	- Multidimensional and objectifiable - Practical	- Binary and objectifiable - Practical
Healthcare data system	- Willingness to engage into continuous evaluation in real-time if it contributes to better patients' management and outcomes - Aspire to move forward a "virtuous circle" of healthcare data (*i.e.*, more and better data leading to better patients' management who then become more involved and willing to share their data)	- Sense of duty and responsibility to ensure evaluation is based on exhaustive, representative, reliable, accurate, and rapidly available healthcare data

Conclusions

- France has a legal and administrative framework that governs the negotiation of PBC.
- Patients and clinicians acknowledge the value of PBCs for access to medical innovation but deplore that real-life PROs and experiences are not given greater consideration.
- Future legal-administrative developments should:
  - — ensure that differences of opinion are better considered;
  - — provide information systems and negotiation frameworks that better satisfy all stakeholders and enable the effective operationalization of PBCs.
- Proposed solutions must be:
  - — easy to implement;
  - — based on endpoints that are seamlessly measurable and objectifiable.
- Failing this, service-based agreements offer a promising alternative and seem more likely to achieve consensus among all stakeholders.

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