The devil in the detail: the challenges of evaluating patient access to medicines

Andrea Hamlin, Gemma Bolton, Roslyn Weaver, Nikki Atkins
Avalere Health, London, UK

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Introduction

- With increasing focus on equitable and faster access to medicines, it is worth considering how initiatives such as the Joint Clinical Assessment and other stakeholder activities will impact daily patient access to therapies (Table 1).

Table 1: Key stakeholder aims (JCA and EFPIA)

Joint Clinical Assessment (JCA)	Collaborative initiative among EU member states to streamline the clinical assessment of health technologies for faster access
European Federation of Pharmaceutical Industries and Associations (EFPIA)	Industry-led group has highlighted delays in timelines and the need to increase patient access

- It is unclear if such initiatives are aligned on what "access" actually means, and whether metrics are sufficiently holistic to consider all elements affecting patients having full access to medicines.
- This study explores the importance of being aligned on what patient access truly means and how success in such initiatives can be measured, considering the key issues for patient access are time, inequities between marketing authorization and reimbursement, and fully enabled in-market access.

Methods

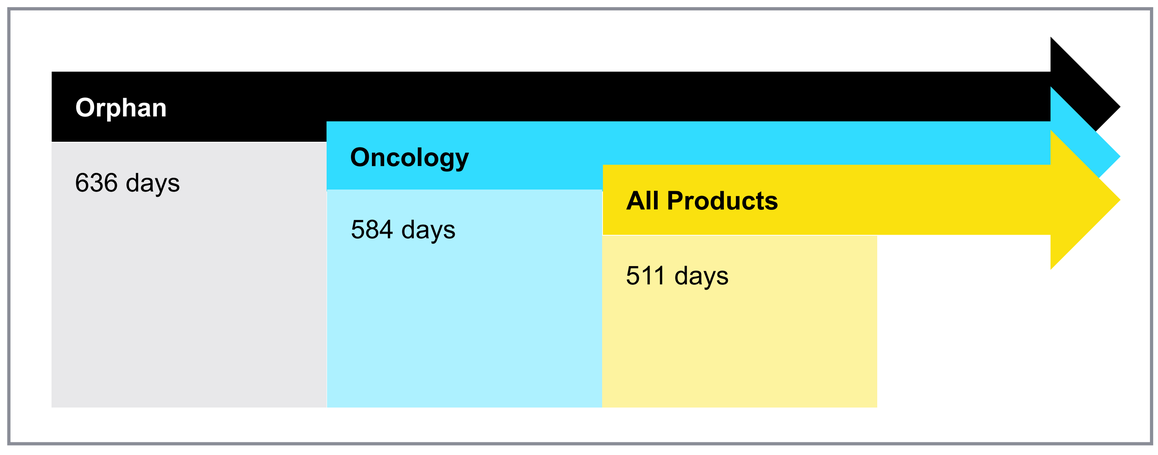
- We undertook targeted gray literature searches, including key European stakeholder websites (eg, JCA, EFPIA).
- We assessed how key stakeholders talk about and measure patient access (eg, definition of and time to access value drivers).
- We evaluated specific examples, novel anticoagulants (NOACs) and advanced therapy medicinal products (ATMPs), to contextualize the transition from national access to patient access and the implications for health equity and accelerated patient access.

Results

Delays in time to patient access

The EFPIA shows patient access to innovative products in Europe can be significantly delayed, with average time to availability being 511 days, and longer for orphan and oncology products (Figure 1).[1]

Figure 1: EU Average Time to Availability



Source: EFPIA Patients W.A.I.T. Indicator 2023 survey[1]

Definitions of patient access

Currently, key stakeholders typically use national reimbursement listing to imply "access" (Table 2).

Table 2: How key stakeholders describe access

JCA	Aims to enable "faster access to medicines",[2] which is understood as going through national health technology assessment processes
EFPIA Patient W.A.I.T. Survey	"Availability" means the inclusion of a centrally approved medicine in the public reimbursement list of a country[1]

The search did not identify studies specifically tracking actual patient access, ie, timing of patient access following national reimbursement.

Value drivers/metrics that influence patient access

- Yet patient access is more than national reimbursement. There are many reasons why the implementation of evidence-based interventions succeed or fail within a complex healthcare environment. Multiple layers of decision-making and varying degrees of health system readiness can collectively slow down the approval and distribution processes for a healthcare technology.
- Subnational payers are often overlooked, yet they are lead decision-makers with responsibility for strategic resource and workforce planning, change management, and implementation activities.
- Specific requirements for some medicines can lead to challenges that must be planned for to ensure fully enabled access for patients within markets. These illustrate the healthcare system implementation challenges that can delay patient access even with national market access and reimbursement in place. NOACs and ATMPs are examples of innovations that require careful consideration when being implemented into the healthcare system (Table 3).

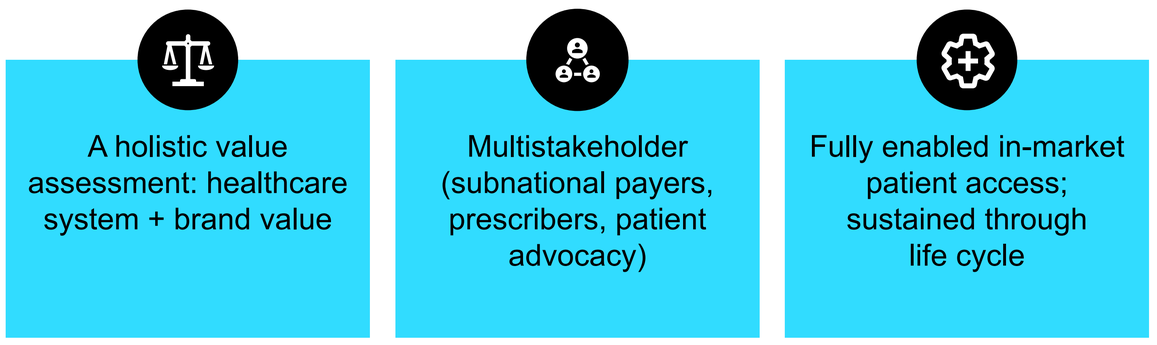
Table 3: Health system considerations for patient access to NOACs and ATMPs

NOACs	Delays in uptake of NOACs over vitamin K agonists, such as warfarin, were largely due to lack of consideration in the service pathway that the introduction of NOACs would bring, such as the removal of physician remuneration for monitoring warfarin.[3]
ATMPs	Learnings from the lag in uptake of NOACs can enable better assessment of how innovative ATMPs can be implemented into the healthcare system. With multiple ATMPs expected given high ~~in~~ research and investment, and the significant patient need for pioneering treatments, it is essential to address the healthcare implementation challenges as seen with NOACs, to prevent delay in fully-enabled patient access.

Conclusions

- Patient access is often understood only to mean national reimbursement, without considering a holistic assessment for drugs moving through the system to reach intended patients.
- There is a need for strategies and execution for national market and patient access, including early planning for barriers and levers to prioritize and operationalize medicines in complex and stretched healthcare systems.
- More work is needed among multidisciplinary stakeholders to ensure that real-world patient access is discussed, measured, and improved in meaningful, pragmatic ways (Figure 2).
- The question remains: how will we know time to access initiatives are successful if stakeholders are defining and measuring different things than fully enabled patient access in markets?

Figure 2: Key factors to consider for fully enabled in-market access



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