Navigating the complexities of early managed access programmes

An essential part of the market access continuum

pwc

Introduction

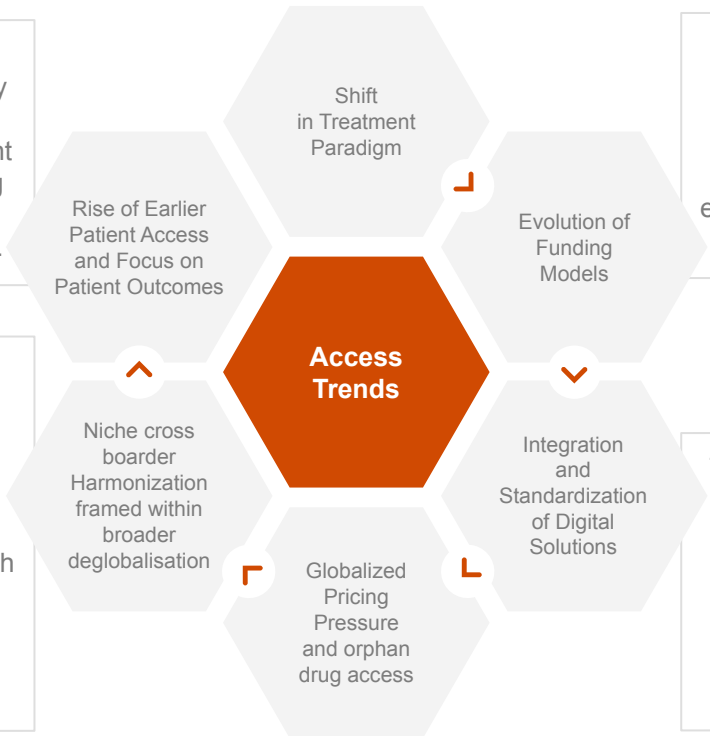
This whitepaper examines how early managed access (or pre-authorisation) programmes are emerging as an integral part of the biopharmaceutical industry's overall access strategy, and how they can shape your go-to-market and hospital engagement strategy based on emerging insights before launch, and on the maturity of the market you are entering.

Setting the scene: What are the global macro access trends?

The post-COVID global access environment is being shaped by payers and governments trying to find a delicate balance between financing highly specialised and targeted innovations, investing in expanded access, and addressing endemic diseases that affect our society at large. This challenge is further complicated by the need to encourage innovation in less attractive areas such as antibiotics, which are crucial in the fight against antimicrobial resistance. Within this complex landscape, several key trends are emerging across the continuum of early access (pre-authorization), awareness, affordability, and access (post-authorization).

Scientific advancements like ATMPs are shifting healthcare from symptomatic to curative and preventive models, impacting therapy options and infrastructure. Accumulated total approvals of 19 CGTs by the EMA and 36 by the FDA, 2 of which in 2024.

Patient-centered care is driving a shift towards early access to innovative therapies, prioritising patient preferences and improving treatment satisfaction and outcomes.



Healthcare funding is adapting in order to accommodate durable solutions, moving towards outcome-based models to ensure equitable access and encourage research.

Joint clinical assessments and harmonized Health Technology Assessment (HTA) processes, demonstrating increasing alignment between payers and regulatory systems, with emerging initiatives like Project Orbis in oncology and the CDC/Technology Foundation in Africa.

Technology is reshaping the way healthcare is delivered, with DiGAs playing a key role by offering personalised support, improving adherence, and reducing costs.

Legislative measures like the Inflation Reduction Act are addressing pricing pressures, requiring a global reassessment of pricing strategies. The IRA will also reduce the duration of economic reward for orphan drugs that introduce additional indications. Germany reduced the cap on orphan drugs from EUR 50M to 30M before formal AMNOG HTA assessment is initiated.

When navigating the global access landscape, it becomes evident that managed access programs are on the rise as they play a pivotal role in addressing high unmet medical needs whilst fostering equitable access to innovative treatments.

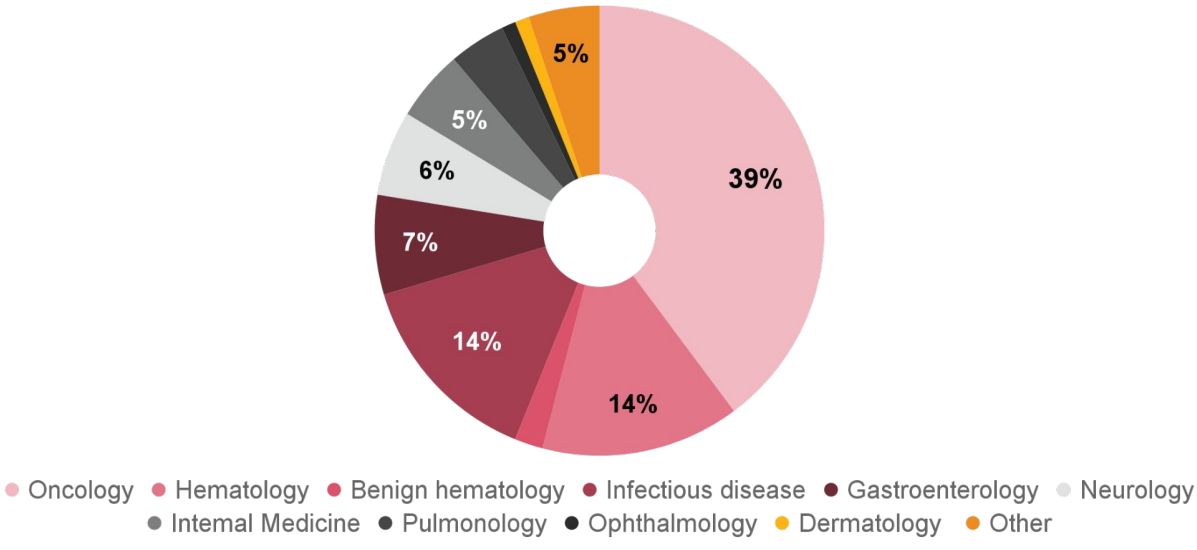
The current trend in the managed access landscape

In the evolving landscape of the biopharmaceutical industry, early access programs are playing a pivotal role in the access strategies of organisations during drug development. With a strategic focus on addressing high unmet medical needs across therapeutic niches, these programs are increasingly recognised as essential components of the access continuum.

The number of publications focusing on early managed access (EA) pathways has increased by 15% YoY, highlighting a growing interest in providing patients access to investigational medicines (53% of publications were related to oncology and haematology, reflecting a high unmet need in these areas) outside of the clinical protocol.

However, there are considerable geographical disparities in expanded access programs, with Europe and the Americas accounting for 87.4% of all publications, while Africa accounts for only 0.6%, revealing a pressing issue regarding international collaboration and equity in access. [1]

Distribution of disease areas covered by Early Access (EA)



[1] Source: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC10193319/

Key insights

523

# of unique therapeutics

+15%

YoY growth of new EA publications

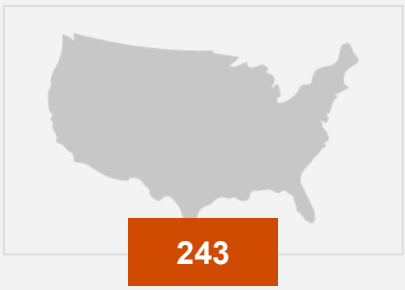
413

Average number of patients per EA program

The vast majority of EA programs were first **launched in a single country** and later scaled internationally

Countries with the most EA protocols published

1 USA



243

2 Italy

183

3 France

90

The strategic incorporation of early managed access programs is increasingly recognised as crucial within the evolving access landscape and can be a foundational in your access strategy. PwC looked at the legislative landscapes in 13 different markets spanning from East (Japan, South Korea) to West (Canada, USA), revealing significant heterogeneity and varying complexity in mechanisms for early and managed access for patients. By shedding light on the diverse nature of early access pathways, legislative frameworks and funding channels, PwC's whitepaper aims to provide stakeholders with actionable guidance for organisational preparedness amid pipeline reviews and the evolving access horizon.

What are managed access programmes and why do they exist?

PwC will use the term "Managed Access" as an overarching term describing a broad variety of paths to access, such as the Early Access to Medicines Scheme in the UK, Special Access Programs in Canada, “Therapeutic Use” in South Korea, and Expanded Access in the USA. These paths are essential for providing early access to promising new medicines, especially for patients grappling with chronic, severely debilitating, or life-threatening conditions in areas of high unmet medical needs. The primary beneficiaries of these programs are patients who fall outside clinical trial protocols or have no commercial access to an approved drug or need to bridge the gap to reimbursement (e.g., Belgium has “medical need programmes”), thus requiring post-authorization managed access.

There are a number of intangible benefits for a broad spectrum of stakeholders in the process, including pharmaceutical companies (as drug developers), healthcare professionals, regulatory authorities, patient organisations, specialised CROs, etc. It is important to proactively collaborate to ensure that the design of your managed access programme provides investigational medicines to patients while effectively managing expectations and addressing ethical considerations.

Complexity and heterogeneity in managed access programmes

The terminologies used in the context of managed access are diverse and interchangeable, reflecting nuances in programme design, regulatory environments, and geographies. After reviewing 13 markets, we observed over 33 different types of managed access channels from a legislative perspective. In most markets, the legislation describes two distinct paths: single-patient via "Named Patient Programs" (NPP) vs. patient cohorts in "Compassionate Use Programs" (CUP).

In some markets such as Canada, we also observed a distinction between acute and chronic treatments, and in one market, five different access paths are described.

Named Patient Programmes (NPP):

Also known as Named Patient Supply or Compassionate Use for single patients. Common in Europe, focused on individual patient access.

Compassionate Use Programmes (CUP):

Known in the U.S. as Expanded Access Programmes, they are used for groups of patients. Similar to NPP, but for cohorts.

Early Access Programmes (EAP):

In the UK, there are drugs designated as Promising Innovative Medicines (PIM) and specials, for example.

Special Access Programmes

Used in jurisdictions such as Canada, which has multiple programmes for access.

Special Access Schemes:

Used in some jurisdictions like Australia for non-approved drugs under special circumstances, similar to NPP and CUP.

The implementation and management of programmes such as NPP and CUP entail understanding various regulatory intricacies, ethical dilemmas, and logistical hurdles. These programmes require defining eligibility criteria, ensuring the availability of investigational products, and communicating with stakeholders while maintaining rigorous data collection protocols. Within the European Union, for instance, CUP initiatives demand a clearly definition of eligible patient cohorts, access to investigational treatments, and adherence to local regulatory requirements to avoid interference with concurrent clinical trials.

Moreover, impending marketing authorisations further highlight the complexity of navigating regulatory landscapes. Transparency, equitable access, and ethical considerations remain central across both NPPs and CUPs, prompting reflection on cost implications, resource allocation during supply shortages, and equitable patient selection processes.

Alignment for Effective Managed Access Programs

We believe it is important to balance the following four dimensions when your organisation is integrating early managed access programmes - these dimensions can be applied at a therapeutic area level and cascaded to a country level.

Managed access programmes must align with **corporate strategy**, emphasizing long-term commercial goals, **patient-centricity**, and ethical principles, while integrating seamlessly with ongoing clinical trials to optimise resource use and maintain scientific rigor.

Understanding global and local treatment guidelines and **assessing unmet medical** needs is crucial to determine early access strategies. Navigating **diverse** regulatory **landscapes** requires adherence to legal and ethical requirements, ensuring regulatory compliance and facilitating market entry. **Considering product attributes** beyond it's therapeutic indication is essential, as complex manufacturing processes and high costs may limit availability outside clinical protocols.

Risks encompass reputational ones, ethical distribution practices, compliance with regulations, internal alignment within the organisation, safety and liability considerations, programme traceability, potential abuse, and supply continuity.

The viability of early access programmes hinges on **internal funding preparation**, navigating diverse funding channels and pricing dynamics, fostering good will with patient organisations and local communities, collecting clinically effective real-world evidence, and aligning with long-term commercial goals, pricing strategies, and programme termination transitions.

Strategic alignment

Corporate and therapeutic area strategy: managed access programmes must resonate with the company's overarching strategy, emphasising alignment with goals such as long-term commercial launch intentions, access sequence (International Reference Pricing (IRP) considerations, orphan drug reimbursement), market positioning, patient advocacy, and product lifecycle management. By integrating managed access initiatives into their strategic framework, organisations can optimise resources and leverage synergies to maximise impact.

Patient-Centricity and Ethics: central to managed access programme alignment is the commitment to patient-centricity and ethical considerations. Programmes must prioritise patient needs, guaranteeing equitable access, informed consent, and the transparent communication of safety data. By embedding these principles into programme design, governance and execution, organisations will foster trust, enhance patient satisfaction, and uphold ethical integrity throughout their managed access programme lifecycle.

Clinical Trial Integration: integration with ongoing clinical trials is imperative to avoid conflicts and ensure coherence between early managed access programmes and randomised controlled trial (RCT) protocols. This alignment encompasses compatibility with trial parameters, including exclusion/inclusion criteria and timing, to mitigate disruptions and uphold scientific rigour.

Feasibility

Treatment guidance and unmet medical need: understanding the global and local treatment guidance is essential to establish the level of the unmet medical need, which is a key eligibility criterion for early access decisions, as physicians and regulators will weigh up the benefit/risk for the target populations.

Regulatory compliance and eligibility: navigating diverse regulatory landscapes across different jurisdictions requires adherence to specific legal and ethical requirements. Alignment with regulatory guidelines is paramount, and requires a comprehensive knowledge of regional regulations governing managed access approval, drug importation, protocols, and exit strategies. Such alignment ensures regulatory compliance while facilitating seamless market entry and sustained operational effectiveness.

Product attributes: beyond the therapeutic indication, you need to consider the technology that you are using, for example AMTP and radio-ligand. High manufacturing costs, low volume batch production, complex hospital care delivery and individually tailored solutions may not be conducive to providing access outside of your clinical protocol.

Managing termination and programme transition: programme termination typically occurs when a drug becomes commercially available. However, the criteria for termination vary by country, comprising factors such as marketing authorisation, commercial availability, and reimbursement policies. Transitioning to commercial availability requires ceasing the inclusion of new patients and determining when existing patients should discontinue treatment. This decision is influenced by the drug's marketing authorisation date, its launch strategy, and its availability in each country.

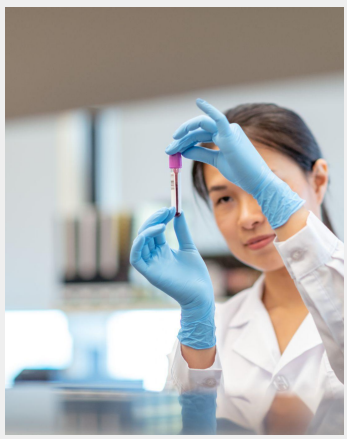
Viability

Funding channels and pricing strategy: we advise preparing internal funding pools to ensure free early access. Understanding diverse funding channels and pricing dynamics is critical for sustainable early managed access and managing the transition to reimbursement at commercial launch. Strategic pricing considerations are paramount: if seeking to charge for early access, which is possible in certain markets, the pricing can serve as a benchmark for future reimbursement negotiations in other territories, necessitating a careful evaluation to optimise long-term market positioning and financial sustainability.

Patient organisations and treatment community: a fundamental intangible benefit of managed access programmes is developing good will within the community. Thus, it is important to understand regional and local patient reach beyond the epidemiology data.

Clinical effectiveness data: RWE is an important component of your body of evidence as you develop a payer value proposition, therefore it is important to understand what data can legally be collected and through which mechanisms in each market. With the implementation of the Joint HTA in Europe in 2025, early effectiveness data could be invalable for population, intervention, comparator and outcomes (PICO) simulations.

Long-term commercial horizon: ensuring an in-depth understanding of your commercial intentions, pricing strategies, and reimbursement possibilities, as well as comprehending your supply commitments. Initiating an early-managed access programme in certain markets may require committing to a full New Drug Application (NDA) or Biologics License Application (BLA), which may result in navigating the reimbursement process in an unattractive market.



Risk

Risks encompass reputational ones, ethical distribution practices, compliance with regulations, internal alignment within the organisation, safety and liability considerations, programme traceability, potential abuse, and supply continuity.

Reputational: poorly managed programmes will have a significant reputational impact in niche therapeutic areas. We recommend that you follow the above considerations for your preparations to mitigate your potential exposure.

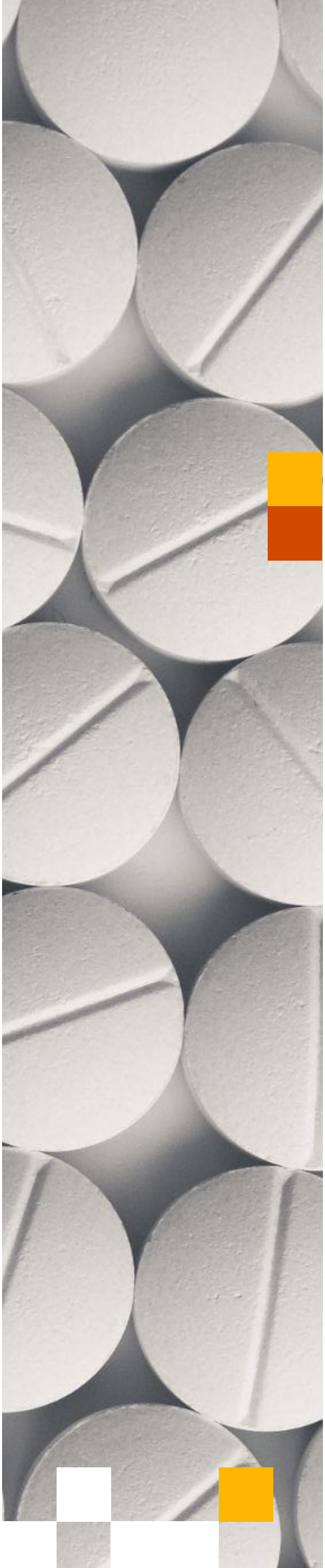
Regulatory compliance: ensure you know the local regulatory environment, that your supply route is GMP compliant, and audit local partners. Also be prepared to provide updates to stakeholders such as health authorities and HCPs as new data emerges.

Monitoring and safety: implement a comprehensive system for monitoring the safety of the medicine within the managed access context. This involves gathering and analysing adverse event reports from healthcare providers and patients, spotting potential safety concerns, and taking steps to mitigate risks.

Programme abuse: certain markets pose a significant risk, as materials can enter the black market. Therefore, it is imperative to manage the supply chain rigorously and ensure robust traceability measures are in place, such as serialisation.

Resource planning: these programmes require time and effort from numerous stakeholders. Defining and assigning specific roles and responsibilities among key departments (Medical, Regulatory, and Quality Assurance) and implementing agreements or internal procedural guidelines to facilitate inter-departmental collaboration is important.

Supply continuity for both clinical and managed access programmes: forecast demand and supply for drugs with small batch releases in the rare disease space, to mitigate supply risks and ensure continuity of supply.



Conclusion

In summary, successful alignment and understanding of the above elements requires a holistic approach integrating corporate strategy, regulatory compliance, patient-centricity, clinical trial integration, transition planning, and pricing strategy. By harmonising all these elements, organisations can optimise the effectiveness of their managed access programmes, uphold ethical standards, and deliver value to patients while navigating complex healthcare landscapes.

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