All Party Parliamentary Group on Access to Medicines and Medical Devices

Secretariat provided by RPP

Bridging the Gap: Strengthening Early Access to Innovative Medicines in England

In England, access to new medicines often remains inconsistent and inequitable, constrained by local capacity and a lack of national coordination. This briefing has been developed by the Access to Medicines and Medical Devices (AMMD) APPG following our roundtable discussion to set out practical recommendations to improve patient access to medicines approved by EAPs.

WHAT IS THE ISSUE?

Early access programmes (EAPs) are designed to bridge the gap between regulatory approval and NHS commissioning. For many patients, this early access granted by EAPs is lifesaving.

Although England has developed multiple early access mechanisms (including EAMS, ILAP, managed access agreements and the Innovative Medicines Fund), patient access remains inconsistent and often delayed due to limited coordination and implementation support.

Currently, there is no clear national framework governing the assessment, approval and delivery of early access programmes at trust level, including how trusts should operationalise EAPs, allocate resources and enable patient access. As a result, decisions are frequently made locally, leading to significant variation in implementation and access.

The primary barriers to effective delivery of early access medicines are often operational:

- **Workforce and service capacity**
- **Monitoring and adminstration**
- **Pharmacy and delivery constraints**
- **Lack of coordinated national support**

As a result of this, a system designed to enable access is not consistently delivery it in practice. Three underlying issues drive this gap:

- **Fragmentation** - No national framework or coordination
- **Unfunded delivery** - Operational costs not supported
- **Implementation burden** - Lack of standardisation and guidance

WHY THIS MATTERS

Clinical Impact

Delays in access can lead to irreversible disease progression, loss of function and reduced survival for patients with rare or rapidly progressing conditions.

Equity Impact

The current model creates systemic inequity, with patients experiencing different outcomes depending on where they are treated.

System Impact

Patient organisations act as system navigators, clinicians face operational burdens and opportunities for early intervention are missed.

Case study 1: Motor Neuron Disease

MND is a rapidly progressive and fatal condition, with six people diagnosed and dying each day. There is no cure.

Tofersen, the first licensed treatment in nearly 30 years, targets a rare genetic form (SOD1-MND) and can significantly slow disease progression.

Although provided for free through an early access route, delivery requires specialist procedures and monitoring that are not funded by the company.

Access is inconsistent and determined at trust level, creating a postcode lottery. Patients have lost function, had to travel abroad for treatment and in some cases died while waiting.

Case study 2: Duchenne muscular dystrophy

- A treatment, Givinostat, shown to slow disease progression was made available through an early access route, with the medicine provided free of charge.
- However, access was **delayed** by approximately **16 months** due to delivery barriers including pharmacy capacity, clinic time and monitoring requirements. Access varied significantly, with patient organisations forced to negotiate trust by trust.
- Of 24 specialist centres, 20 now provide treatment, often slowly, while four do not.
- Given the time-sensitive nature of eligibility, **patients risk losing the ability to walk** while waiting. This demonstrates a fundamental issue: access depends on local capacity rather than clinical need.

WHAT NEEDS TO CHANGE

1 Establish a national early access framework

DHSC and NHS England should introduce a clear national framework for assessing and delivering early access programmes, with defined expectations for trust participation.

2 Standardise and support local delivery

NHS England should deploy nationally endorsed toolkits and support earlier cross-sector collaboration to enable faster, more consistent implementation.

3 Fund the delivery of medicines

DHSC and NHS England should introduce a funding mechanism to cover administration, monitoring, pharmacy and staffing costs, ensuring local resource pressures do not block access.

4 Reform and align access pathways

DHSC, NICE and MHRA should ensure access pathways are coherent and integrated, including EAPs, EAMS, NICE processes and the Innovative Medicines Fund, with greater flexibility for rare and rapidly progressing conditions and use of real-world evidence.

5 Remove policy and adminstrative barriers

HM Treasury and HMRC, with DHSC, should clarify and reform VAT and other administrative requirements for free of charge medicines to avoid disincentivising industry participation.

6 Review EAPs efficacy for rare diseases

DHSC should extend the current evaluation of access pathways under Action 25 of the Rare Disease Action Plans to include EAPs, to identify best practice and support more consistent national delivery, particularly in relation to rare diseases.

International Best Practice - France's Early Access Model

France's early access framework, **Accès Précoce** (introduced in 2021), has enabled access to over **300 medicines** and thousands of patients, demonstrating timely and equitable access.

Its effectiveness is driven by three core features:

National, centralised decision making

Early access is coordinated at a national level, enabling rapid and consistent decisions across the system rather than reliance on individual trust capacity.

Funded delivery model

Early access is centrally funded, ensuring that administration, monitoring and service delivery costs do not act as barriers to patient access.

Integrated evidence generation

Real world data collection is embedded within early access, supporting evaluation and accelerating transition to routine commissioning.

A coordinated, funded and evidence driven model that the UK can learn from.