Bridging the Gap: Delivering Early Access to Medicines Programmes Across the NHS

Access to Medicine and Medical Devices APPG report

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Speakers

Danny Beales MP (Chair, Moderator), **Katie Combes** (Policy and Public Affairs Lead, Duchenne UK), **Scott Purdon** (Chair, Charities Medicines Access Coalition, Head of Patient Advocacy, Myeloma UK), **Sian Guest** (Policy and Public Affairs Manager, MND Association), **Stella McKernan** (Senior Policy Advisor, MND Association)

Attendees

Iqbal Mohammed MP, Jacob Kellagher (Parliament Assistant), **Hannah Wiles** (Parliament Assistant), **Ellie Kirkland** (Healthcare Policy and Influencing Manager, Muscular Dystrophy UK), **Eleanor Carpenter** (Policy and Public Affairs Officer, Sarcoma), **Kirsten Spencer** (Metup UK), **Ryan McCullough** (Senior Public Affairs and Campaigns Manager, Alzheimer's Research UK), **Grace Lew** (Policy and Campaigns Officer, Duchenne UK), **Rowan Wathes** (Associate Director of Policy and Health Strategy, Parkinson's UK), **Rachel McEleny** (Public Affairs Manager, Genetic Alliance), **Harvey Jones** (Public Affairs Officer, Alzheimer's Research UK), **Sophia Corfield** (Government Affairs Manager, ABPI), **John Coughlan** (Head of International Government Affairs, Regeneron), **Charles Waller** (RPP), **Ciaran Johnston** (RPP), **Celia Yesil** (RPP), **Benjamin Phan** (RPP).

General Overview

The roundtable examined how early access programmes (EAPs), including EAMS, managed access and other routes, can better support patient access to innovative medicines, with a focus on addressing current barriers and system limitations. Participants emphasised that these programmes are most often life-changing and, in many cases, life-saving.

However, while EAPs are intended to bridge the gap between regulatory approval and routine NHS commissioning, in practice many patients face delays or exclusion due to staffing constraints, monitoring requirements and limited trust confidence. These challenges are particularly acute in rare and rapidly progressing conditions, where delays can lead to irreversible deterioration.

The discussion examined barriers preventing patients from accessing innovative medicines through EAPs, even where treatments are provided free of charge. Participants described a system characterised by inconsistency, local discretion and limited delivery capacity.

A central theme was the absence of a clear national mechanism in England, leaving access dependent on local priorities and capacity. Participants also highlighted the need for greater alignment across the wider access landscape. The discussion concluded with a call for stronger national leadership, improved implementation support and a more coordinated approach to early access.

Building on this discussion and evidence from patient groups, the APPG seeks to support NHS England and DHSC in strengthening early access programmes to deliver more consistent and equitable access to innovative medicines.

Key Findings

- The current system in England is fragmented and overly reliant on local trust level decisions, creating a postcode lottery in access.
- There is no clear, consistent national framework in England governing how trusts should assess, approve and operationalise early access programmes (EAPs).
- Although medicines in EAPs are often provided free of charge by manufacturers, delivery costs, staffing pressures, monitoring requirements, pharmacy capacity and administrative burden frequently prevent access.
- Patient organisations are often forced to act as de facto navigators and advocates, negotiating trust by trust to secure access for families, an unsustainable model that entrenches inequity.
- Current schemes such as the Early Access to Medicines Scheme (EAMS) were described as poorly suited to rare disease treatments, where evidence bases are smaller and disease progression is rapid.
- There are clear opportunities for improvement through practical, low-cost measures and learning from more coordinated models.

Importance of EAPS and testimonies, the value of EAPS

Participants emphasised that EAPs are often life changing and, in some cases, lifesaving, acting as a critical bridge between clinical innovation and routine NHS access, particularly for patients with rare or rapidly progressive conditions.

Katie Combes (Duchenne UK) illustrated this through her organisation's 16 month effort to secure access to a new Duchenne muscular dystrophy treatment. While not curative, she highlighted that the therapy slows progression and preserves mobility and independence, offering families valuable additional time and improved quality of life.

This was reinforced across conditions. Scott Purdon (CMAC), representing 33 charities, stressed that early access can mean the difference between decline and stability, citing a myeloma patient who, after exhausting multiple treatment lines, accessed a compassionate use therapy and is now doing well.

Stella McKernan and Sian Guest (MND Association) similarly highlighted the impact in SOD1 motor neurone disease, describing tofersen as the first licensed treatment in nearly 30 years with the potential to significantly slow progression. Sian Guest shared patient testimonies illustrating the urgency, including individuals travelling or relocating to access treatment and others experiencing irreversible decline while waiting, contrasted with a patient who regained function through a clinical trial.

Challenges to EAPs and barriers

Despite their value, participants described the early access landscape in England as fragmented, inconsistent and inequitable, with access driven more by local trust capacity than clinical need.

Katie Combes illustrated this through Duchenne UK's experience. Although the medicine was provided free of charge, trusts cited operational constraints, including pharmacy capacity, clinic time and monitoring, while some declined to participate entirely. Duchenne UK was forced to negotiate trust by trust: 20 of 24 centres now provide access, often slowly, while four still do not.

These issues were echoed across conditions. Scott Purdon highlighted poor signposting, inconsistent processes and confusion between access routes, noting that many patients require expert advocacy simply to navigate the system.

Stella McKernan and Sian Guest described similar challenges in a more acute context. While tofersen is free of charge, its delivery is unfunded and resource intensive, leading to uneven access, patient deterioration and capacity pressures within participating trusts.

Participants also identified weaknesses in national pathway design. EAMS was seen as poorly suited to rare and rapidly progressing conditions, while NICE methodologies were described as insufficiently flexible. Sophia Corfield (ABPI) added that VAT and administrative burdens can further discourage participation.

Kirsten Spencer (METUP UK) emphasised that these issues are consistent across disease areas and highlighted the need for greater support, guidance and safeguards to enable safe delivery of innovative treatments.

What is needed for improved EAPs

A central theme was the need for clearer national guidance and stronger coordination. Iqbal Mohamed MP framed this as a systems design issue, with participants agreeing that the absence of a national framework in England is a key driver of delay and inequity.

Participants highlighted the value of standardised implementation packages for trusts, including protocols, monitoring requirements, training and administrative templates to reduce duplication and enable faster, more consistent local decisions. Rowan Wathes (Parkinson's UK) reinforced that many barriers are operational rather than structural, pointing to the lack of shared service pathways and arguing that standardisation, combined with earlier cross sector collaboration, could provide a low cost, high impact solution.

Alongside local delivery, stronger national oversight was seen as essential. Rachel McEleny (Genetic Alliance UK) highlighted opportunities within the UK Rare Diseases Framework to strengthen coordination, including proposals for a national clinical lead. International examples were also cited, with John Coughlan (Regeneron) pointing to the French system as a model for more consistent, centralised decision making.

However, participants emphasised that structural reform must be matched by practical support. Even where medicines are provided free of charge, delivery costs, such as

administration, monitoring and follow up, remain a key barrier. Ryan McCullough (Alzheimer's Research UK) also highlighted wider system readiness challenges, including workforce, diagnostic and infrastructure gaps, alongside underspend in the Innovative Medicines Fund that could be used more strategically to support access and service readiness.

Participants also called for greater alignment across the access landscape. Mechanisms such as EAPs, EAMs and the Innovative Medicines Fund were seen as siloed, with a need for a more coherent end-to-end pathway and greater flexibility in evidence requirements for rare and rapidly progressing conditions, including increased use of real world data.

Policy Recommendations

1. Establish a national early access framework - DHSC and NHS England should implement a clear national framework for assessing and delivering EAPs, with defined expectations for trust participation to reduce variation.

2. Standardise and support local delivery - NHS England should deploy nationally endorsed toolkits (protocols, monitoring, consent and service specifications) 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 access pathways for rare and progressive conditions - NICE and MHRA should adapt EAMS and appraisal processes to better reflect rare diseases and treatments that slow progression, including greater use of real-world evidence.

5. Align the end-to-end access pathway - DHSC and NHS England should better integrate EAPs, EAMS, NICE processes and the Innovative Medicines Fund into a coherent pathway from early access to routine commissioning.

6. Remove policy and administrative barriers (including VAT) - 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.

7. Include EAPs in Rare Diseases Framework evaluation - DHSC and NHS England should extend the current evaluation of access pathways under Action 25 to include EAPs, to identify best practice and support more consistent national delivery.