# Government response: Consultation on proposed statutory instrument for the Early Access to Medicines Scheme (EAMS)

**Executive summary**

The UK Early Access to Medicines Scheme (EAMS) is one of the ways through which a patient with a life threatening or seriously debilitating condition can gain access to a medicine before it has gained approval from a medicines regulatory authority. The MHRA launched a public consultation on the 6th August 2021 in order to seek views on proposed legislative changes to clarify the legal basis for EAMS. The consultation closed on the 17th September 2021. Comments and views expressed from a variety of stakeholders from across the UK were broadly positive and there was support for amending the Human Medicines Regulations 2012 to introduce specific EAMS provisions. Therefore, based on feedback, we have decided to lay a statutory instrument in early 2022. The statutory instrument will introduce the policy considered during the consultation and future guidance will be developed alongside the statutory instrument to provide the necessary interpretation and procedural support.

1. **Introduction**

The MHRA hosted a public consultation on a proposed statutory instrument for the Early Access to Medicines Scheme (EAMS) from the 6th of August to 17th September 2021. The consultation was carried out to obtain the views from patients, healthcare professionals, businesses involved in EAMS manufacture or supply, and the wider public.

The proposed legislative changes are designed to ensure that EAMS remains relevant and attractive following the UK’s exit from the European Union and that patients in the UK are able to access cutting edge therapies in advance of licensing decisions where they fulfil the EAMS criteria. In addition, proposals to simplify the supply of EAMS medicines and facilitate real-world data collection are intended to fulfil the goals to reduce regulatory burden and support the UK life sciences agenda in making the UK an attractive place to bring the development and introduction of innovative products.

The proposals included 5 key areas listed below:

* EAMS key principles of operation: describe the objectives of EAMS and clearly detail its principles of operation, as published in the current EAMS scientific opinion guidance
* Simplifying the supply of EAMS medicines: simplify and harmonise expectations for the manufacture, assembly and importation of EAMS medicines
* Create a supportive framework the collection of real-world data (RWD):  allow for RWD collection without the need for a clinical trial approval, as long as the MHRA has no concerns about the collection of that data
* Clarifying the liability for prescribers and patients: provide clarity around the use of EAMS medicines, in line with the General Medical Council (GMC) recommendations
* Pharmacovigilance (safety monitoring): In order to best support safe use and to reassure patients and healthcare professionals, introduce pharmacovigilance requirements in line with those currently expected through guidance.

The consultation asked three main questions:

* Question 1: Do you agree with the proposed inclusion of the principles of EAMS in the consultation? If not, please explain your reasoning.
* Question 2: Are there any concerns or comments with regards to the proposed provisions?
* Question 3: Are the proposed provisions comprehensive and do they strike the right balance? Are there additional provisions you would consider?

This consultation response will provide a summary of the feedback received from stakeholders and the Government’s response.

1. **Overview of consultation activity**

Overall, there were 59 complete responses which is considered to be a good number for this public consultation. 66% of responders were from organisations (69% of these organisations cover UK) and 34% were individuals (81% of were from England, 10% from Northern Ireland). Responders from organisations included industry trade associations, individual pharmaceutical companies, not for profit organisations involved in drug development, patient advocacy groups (8 patient groups in total, additionally 7% of responders also classified themselves as patients), regulatory / professional representation bodies and health delivery organisations. The variety of interests of the respondents provides a good diversity of views and is considered to be representative of all interested stakeholders.

During the consultation period, a dedicated patient engagement workshop was held to hear views directly on the proposals from a number of patient representatives from the MHRA’s Patient Group Consultative Forum (PGCF) (includes a broad and inclusive membership of organisations and individuals from across the whole of the UK).

1. **Summary of responses**

We have carefully reviewed and analysed each of the responses received. Comments and views expressed from a variety of stakeholders from across the UK were broadly positive and there was continued support for EAMS and support for amending the Human Medicines Regulations 2012 to introduce specific EAMS provisions. There were some comments from industry representatives regarding the lack of consultation on the legal text of the statutory instrument and the need to formally review the statutory instrument in case of potential unintended consequences. The legal text will reflect the policy as proposed in the consultation and there will be further engagement opportunities with relevant stakeholders in the creation of the detailed guidance which will help to ensure proper interpretation and implementation. There were many comments that covered EAMS procedural aspects and it is our intention that these will be considered in full during the development of the revised EAMS guidance. Guidance will be available in advance of the date of the regulations coming into force (28 days after the day on which they are made). Oral feedback from the dedicated patient group meeting was positive of the proposals and re-enforced the importance of the scheme. Further summary details of the formal responses to the consultation based on the questions posed are provided below.

**Summary graphs from the consultation responses**

*Do you agree with the proposed inclusion of the principles of EAMS in the Human Medicines Regulations? Yes/ No*



*Are there any concerns or comments with regards to the proposed provisions as described in the consultation document? Yes/ No*



*Are there additional provisions for EAMS that you would consider important? Yes/ No*

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*Do you think the proposals risk impacting people differently with reference to their [or could impact adversely on any of the] protected characteristics covered by the Public Sector Equality Duty set out in section 149 of the Equality Act 2010 or by section 75 of the Northern Ireland Act 1998? Yes/ No*

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***Experience with EAMS***

Responses to the consultation included responders who were active participants in EAMS programmes, those who routinely deployed EAMS products in the NHS, and organisations involved in the medicines development / the access pathway (e.g. Health Technology Assessment bodies, National Health Service).

The main themes of experiences with the current Scheme from responders who may have supplied, manufactured or received EAMS products are captured in the following reflections:

* Awareness of the scheme and championing availability of EAMS products for people affected by a condition
* EAMS is a critical element ensuring that the UK has a competitive and attractive market access environment for the launch of new products, more could be done to ensure that EAMS remains competitive
* EAMS is a critical element of the innovation life cycle
* Largely positive experiences of EAMS, but acknowledgment that the EAMS process does require a significant amount of resource
* The level of evidence needed to apply for an EAMS outweighs the potential benefit provided at this time
* Encourage the MHRA to go beyond this [the proposals in the consultation] and to consider additional considerations that could enhance the Scheme so it is a stronger and more attractive option for companies

***Question 1: Do you agree with the proposed inclusion of the principles of EAMS in the*** *Human Medicines Regulations 2012****as described above? If not, please explain your reasoning; Please provide any additional comments you may have***

***Consultation result: 88% YES, 12% No; 35 written responses***

Overall, there was broad agreement that introducing the core principles of EAMS onto a statutory basis would provide legal clarity which would benefit industry, patients and healthcare professionals.

There was agreement with the emphasis on reducing or removing existing administrative burdens, which was currently considered to be excessive in some areas. It was considered that the proposed changes would strengthen EAMS and bring about improvements to support access to EAMS products and therefore increase benefits to patients who have conditions that require additional effective treatments. However, it was noted that it is important to ensure that the proposed inclusions to the legislation do not add to the workload for both the NHS and industry for implementation of an EAMS scientific opinion.

The inclusion of a more supportive framework for real-world data collection was largely welcomed. It was noted that the generation of evidence is integral for the accurate assessment of innovative treatments and that real-world data might supplement clinical trial data. Removing barriers to this and creating greater opportunity to collect real-world data was considered an opportunity for drug developers and patients.

Some industry respondents stated that although they were supportive of the ambitions of the proposals in the consultation, they could not agree to the proposals without seeing the Statutory Instruments (SI’s) in order to ensure that the specific wording could be considered and any unintended consequences examined and avoided. The MHRA was asked to commit to holding a period of consultation on the draft legislative proposals, so that detailed feedback could be gathered and considered prior to them entering the legislative process. There were many comments that covered specific technical procedural aspects.

There were some comments that placing EAMS on a statutory footing will provide reassurance to the patients accessing treatments and it was noted that whilst the General Medical Council guidance sets out standards of good practice, it does not address the issue of legal liability.

There were responses that welcomed the changes, but that the proposals fail to address the fundamental issues that the industry faces in preparing for an EAMS, with regards to the level of evidence required in making the EAMS application, persistent gaps in funding, reimbursement and uptake post-market access.

***Government response***

We welcome the many useful comments and thank respondents for taking the time to carefully consider our proposals. The vast majority of comments were in the main positive about the inclusion of EAMS in the Human Medicines Regulations 2012. Where a negative view was expressed this was in the majority of cases because of a lack of opportunity to review the future legal text of the statutory instrument. The consultation was conducted on the policy proposals rather than the statutory instrument itself to ensure appropriate opportunity for responses received through the consultation to influence the overall policy direction. After careful review it is considered that the SI will introduce the policy as detailed in the consultation and there will be further engagement opportunities with relevant stakeholder in the creation of the detailed guidance which will help to ensure proper interpretation and implementation.

Issues relating to funding and other aspects outside of the remit of the MHRA and the Human Medicines Regulations 2012 cannot be addressed through this proposed statutory instrument which has a specific focus on the regulatory aspects of EAMS. However, these points may be considered in future operational guidance and/ or other wider policy initiatives.

***Question 2: Are there any concerns or comments with regards to the proposed provisions as listed above?***

***Consultation result: 54% Yes, 46% No, 36 written responses***

The proposed introduction of legislative provisions for EAMS must take account of the current and future context of medicines supply to Northern Ireland and additional requirements under the Northern Ireland Protocol. Northern Ireland must have access to the EAMS in the same way as any other region in the UK.

There were many comments supporting the objective to reduce complexity and clarify supply regulations that support the overall EAMS objectives, but it was noted the use of these flexibilities should not undermine the regulatory system that is in place to ensure safe, quality controlled manufacture and supply of medicines in line with existing standards and regulations (e.g. it was considered important to continue the ability to use clinical trial stock during EAMS).

There were numerous diverse comments on procedural aspects around data requirements for EAMS submissions and timelines. Requests were made to ensure clarity on the role of EAMS vs existing MHRA Guidance Note 14: The supply of unlicensed medicinal products (“specials”).

The proposal to provide a supportive framework for the collection of real-world data, and specifically enabling collection of evidence without the need for a Clinical Trial Authorisation was welcomed (though there were various opinions to the extent to which this was a barrier), particularly for cell and gene therapies for rare or ultra-rare diseases. For real-world data collection it was stated that it is important for sponsors and Health Care Professionals to understand that data can be collected as part of an EAMS programme but this data collection should remain voluntary. Guidance should be provided on Health Research Authority, local Ethics Committee approval and patient consent. There were also numerous comments on the practicalities of real-world data collection including what data is best collected, partnership working to align requirements, data ownership, ensuring patient confidentiality, burden of collecting data and what is the appropriate infrastructure. It was stated that patient trust is important in the collection of real-world data. Patients should be well informed of the benefits of their data, but it is essential that patients understand that they can decline to join a real-world data study without impacting their ability to join the EAMS? scheme.

There were comments supporting the reinforcement of the GMC’s guidance that the individual prescribing clinician is required to take full responsibility for his/her decision to treat a patient and there should be there should be equal, professional responsibility placed on the dispensing pharmacist.

The pharmacovigilance regulatory requirements proposals should not inadvertently and unnecessarily extend the current requirements, and lead to additional costs/administrative burden with no additional safety benefit. EAMS risk management plan? regulatory requirements should be proportionate and robust to consolidate existing requirements.

There were some additional policy comments including:

* The Independent review of EAMS report (2016) makes a number of other recommendations which are not covered, but which remain important and relevant if EAMS is to continue to be attractive in a global context
* The creation of the Innovative Licensing and Access Pathway (ILAP) provides an overarching policy environment under which individual schemes such as EAMS can be better supported and coordinated
* That the value of EAMS to companies and to patients could be better expressed
* There is opportunity to better streamline or reduce the overlap between the MHRA assessment of EAMS clinical data for a Scientific Opinion and the subsequent national Marketing Authorisation Application assessment
* Patient access to EAMS products has been continued following the product receiving a Marketing Authorisation but before a decision is made about NHS access, but there is currently no ‘framework’ or guidance under which these flexibilities could be utilised and enhanced.

***Government response***

Introducing a legislative basis for EAMS will not significantly change the scheme as it currently operates. We intend that the legislation will provide a clear framework for the Scheme rather than address every detail of the operation of EAMS. This will ensure that regulatory provisions for EAMS remain flexible and proportionate. As access pathways such as ILAP continue to develop and evolve, we will also continue to consider how EAMS and other access pathways can complement each other within the regulatory framework.

In implementing the proposals through a statutory instrument, EAMS guidance will be updated to explain in detail the implementation and operation of the system, this will address the comments and questions received on detailed procedural and operational aspects.

The guidance will also detail the framework around the collection of real-world data under the scheme. Patient trust is absolutely critical in the collection of patient data. Collection of real-world data during the scheme has great potential to improve the evidence base for new and innovative medicines as well as new indications for already approved medicines, however it will be important that patients receiving an EAMS product have the option to choose whether their data is used in this way. Therefore, the statutory instrument will be clear that consent must be obtained for collection of data under the scheme and refusal to give consent must not impact access to the EAMS product.

Pharmacovigilance requirements are an important part of the regulation of any medicine, including EAMS products, to gain understanding of the safety of medicines, and that any emerging risks associated with a medicine are detected. The pharmacovigilance requirements proposed to be introduced into legislation for EAMS are not expected to introduce any additional burden because they are based on the current requirements and expectations of the scheme. The pharmacovigilance requirements will be proportionate to the goal of ensuring that the safety profile of EAMS medicines can be sufficiently well monitored.

The provisions will consider the conditions required for the supply of EAMS medicines including the responsibilities placed on the EAMS scientific opinion holder and the prescriber.

We have considered our proposals in respect of the Northern Ireland Protocol and it is important to us that NI patients will continue to have access to EAMS products in the same way that they currently do under the Scheme. NI will continue to be covered by an EAMS Scientific Opinion before a marketing authorisation is approved.

***Question 3: Are there additional provisions you would consider?***

***Consultation result: 75% Yes, 25% No***

There were a variety of diverse comments which where relevant to the SI we will aim to address in enhanced guidance. These included (and not previously mentioned above):

* Reimbursement and funding of EAMS medicines (including links to the Innovation Medicines Fund and commercial attractiveness of the scheme)
* The need for enhanced and standardised communication to NHS trusts and prescribing healthcare professionals (in line with ABPI code of practice)
* Better signposting on how EAMS works
* Need for enhanced guidance and templates (including versus ILAP, periodic reporting, Treatment Protocols, standardising templates in the NHS, agreed minimum data set of information for the NHS)
* Proposals for further collaborative working between different organisations including the transition from managed access during the EAMS period to expanded access post-Marketing Authorisation but pre-Health Technology Assessment decision (NICE, Accelerated Access Collaborative - AAC)
* Need for public engagement on the topic of EAMS and patient friendly leaflet to explain the purpose of the data collection and the wider benefits it will have

***Government response***

We appreciate the wide range of suggestions of additional provisions for consideration. We agree that the current guidance for EAMS would benefit from being updated and intend to do so to accompany the legislation for EAMS. Comments that relate to the more procedural and operational aspects of EAMS will be addressed in the updated guidance. Other comments are acknowledged but do not require a legislative change and will be considered for the future development of EAMS policy. Opportunities to carry forward some of these suggestions will be actively considered once the statutory instrument has been made clarifying the legislative basis for EAMS.

Issues relating to funding of EAMS medicines cannot be addressed through this proposed statutory instrument which has a specific focus on the regulatory aspects of EAMS.

***We do not consider that our proposals risk impacting people differently with reference to their protected characteristics or where they live in; We welcome your views on this point (39 responses)***

***Government response***

The majority of responses received agreed that the proposals did not risk impacting people in reference to their protected characteristics of where they live or provided no further comment on this point.

1. **Section 2 of the Medicines and Medical Devices Act 2021**

The consultation was carried out in accordance with the requirement in section 2 of the Medicines and Medical Devices Act 2021. In making regulations under that section the Secretary of States overarching objective is to safeguard public health.

In considering this policy, and regulations that would be needed to give effect to it, the Secretary of State has had due regard to:

* the safety of medicines within the scope of this policy
* the availability of medicines within the scope of this policy
* whether the United Kingdom is likely to be seen as a favourable place in which to research the medicines within the scope of this policy, develop medicines within the scope of this policy or manufacture or supply medicines that come within the scope of this policy

We have assessed the EAMS proposals against these factors, as described in the consultation document, and evidence submitted in the consultation responses has not changed those assessments.

1. **Conclusion and next steps**

This consultation has provided valuable feedback on our proposals which have helped us develop and further refine how best to clarify the legal basis for EAMS. The overarching response has been supportive of the intent of the proposals and gives a clear indication that stakeholders are supportive of introducing a bespoke EAMS provision within the Human Medicines Regulations. We will now take steps to make a Statutory Instrument under the Medicines and Medical Devices Act 2021 to provide a legislative framework for EAMS.

We fully recognise that the guidance that accompanies the legislation will be critical for proper implementation and interpretation of the regulations. We will work with stakeholders through established connections in producing that guidance, with the intent to have guidance published when the statutory provisions come into force.

We wish to thank everyone who submitted a response to this consultation and who has engaged with us so far on the proposals for EAMS.