Early Access to Medicines Scheme (EAMS) consultation – BIA draft response

Consultation description: The aim of this proposal is to ensure that EAMS remains an attractive option for patients, healthcare professionals and companies, so that cutting-edge therapies are available for patients where there is an unmet clinical need. We aim to make the legal basis for EAMS supply clear and minimise the burden on those supplying EAMS medicines and for those companies wishing to collect real-world data during the scheme. This will be delivered whilst continuing to ensure the safety of EAMS products through pharmacovigilance (safety monitoring), maximising patient access and benefit.

* Indicates where an answer from pre-specified list must be given.

EAMS Consultation (online form)	
Are you responding as an individual or on behalf of an organisation?*	On behalf of an organisation
What geographical location does your organisation cover?*	The BIA is the trade association for innovative life sciences in the UK
Name of Organisation	BioIndustry Association (BIA)
Relationship to EAMS*	Representing member organisations which obtained an EAMS scientific opinion or may be considering using EAMS
Do you agree with the proposed inclusion of the principles of EAMS in the Human Medicines Regulations? Please provide any additional comments you may have.	Yes, the BIA is supportive of this initiative. EAMS provides an important opportunity for patients with life threatening or seriously debilitating condition to access novel medicines prior to marketing authorisation. We believe that enabling early access to medicines should be at the heart of life sciences policy. The proposed inclusion of the principles of EAMS in the Human Medicines Regulations will provide a clearer legal basis and improve regulatory certainty for companies as regards the status of the scheme. Reducing the burden for EAMS medicines supply and simplifying the requirements for data collection would be welcome. The need for a clinical trial authorisation (CTA) application to collect real-world data has acted as a barrier in the past, but the proposals present new opportunities to do so, if proportionately implemented by the MHRA, and will help in the continued assessment of the risk/benefit profile and input into the broader authorisation strategy.
Are there any concerns or comments with regards to the proposed provisions as described in the	No, there are no concerns as such. We would like to provide our comments and suggestions to improve

consultation document? Please provide any additional comments you may have.	the proposals with greater clarity on the requirements. The policy objectives state that the "legislative changes are designed to ensure that the 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." We are fully supportive of this intention which should be effectively implemented to achieve the UK Government's life sciences industry policy agenda. We support the proposed flexible approach in section 3.2 and introducing "a simple administrative procedure" to permit the manufacture, assembly, and importation of EAMS medicines whether they are unlicensed or off-label. This would be welcome by companies which may only have a MIA(IMP), including many UK-based and non-UK companies, when Phase 3 trials have completed. The statement "provided that the proposed activity falls within its current scope" requires clarification when the scope of a MIA(IMP) can only be for a clinical trial. The proposed activity of supplying a medicine under EAMS obviously cannot be part of the scope of a procedure pertaining to a clinical trial. So, is the intention to allow the holder of a MIA(IMP) to use that licence for supply of an EAMS medicine? Will this be addressed in the proposed legislative changes to the Clinical Trials regulations? The proposal to provide a supportive framework for the collection of RWD in section 3.3, and specifically enabling collection of evidence without the need for a CTA is welcome. It would be helpful to provide further guidance on the data that can be collected under the scheme. We would also suggest that the MHRA seeks to align the real-world data framework applied in EAMS with other such frameworks in development by its partner organisations, including NICE and the Innovative Medicines Fund when that becomes operational with a view to facilitating adoption of such innovative treatments in the NHS. Related to this, early engagement with the scheme partners, including NICE, on the data collection plan for EAMS would be beneficial in ensuring that any

	data collected would support future HTA submissions. For example, data generated from EAMS could be complementary to (but not replacement of) those derived from the clinical development programme to support an informed assessment on the benefit/risk and therapeutic position of the product with a view of timely adoption in the NHS.
Are there additional provisions for EAMS that you would consider important? Please provide any additional comments you may have.	Yes, see below. To incentivise industry's participation in the EAMS, consideration should be given to developing a coherent framework for innovative methods of treatment to be made available and reimbursed for effective adoption in the NHS. In this regard, the scheme together with the recently created Innovative Licensing and Access Pathway (ILAP) as continuum of market access should be appropriately funded. We look forward to continuing the dialogue with Government and addressing the needs of SMEs to recover the costs of EAMS medicines provided free of charge until a NICE recommendation. The lack of funding has proved challenging for SMEs to use EAMS since it was launched in April 2014. There is an increasing need to enable continued access to and reimbursement of medicines between the point of marketing authorisation and NICE recommendation. This is particularly important as changes in the regulatory landscape may see breakthrough therapies approved earlier than has previously been possible (and these therapies may be candidates for EAMS). We would therefore suggest that the MHRA looks to work with the scheme partners to identify a way for EAMS and non-EAMS medicines to be made available following marketing authorisation, should there be a significant gap between approval and NICE recommendation. Progress has already been made in this area on enabling periods of data collection following a recommendation by NICE, such as through the Cancer Drugs Fund or Innovative Medicines Fund. Allowing periods of data collection and reimbursement pending a NICE decision would further support access to new, innovative medicines. Moreover, we would welcome further guidance on the role that EAMS can play in supporting rapid access to

	medicines through ILAP. We believe ILAP represents a positive step forward in bringing partner organisations together with life sciences companies to support earlier patient access to the most therapeutically impactful new medicines. Finally, there is a need to also improve communication of the availability of EAMS medicines to healthcare professionals to raise awareness and support patient access.
In Northern Ireland new policies must be screened under Section 75 of the Northern Ireland Act 1998, which places a statutory duty on public authorities, to mainstream equality in all its functions – so that equality of opportunity and good relations are central to policy making and service delivery. In addition, new or revised policies must be rural proofed in line with the Rural Needs Act (NI) 2016 which requires public authorities to have due regard to rural needs. We do not consider that our proposals risk impacting people differently with reference to their protected characteristics or where they live in NI. We welcome your views on this point.	We agree with MHRA's position. Equitable access to innovative methods of treatment within the UK by ensuring alignment of the policy to benefit patients ought to be considered within the proposal.
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.	No