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Dear Colleagues

Re: Early Access to Medicines Scheme – Lumasiran in the treatment of primary hyperoxaluria Type 1 in all age groups

I am writing to advise you regarding NHS England's position on the recent positive Scientific Opinion for lumasiran published by the Medicines and Healthcare Regulatory products Agency (MHRA). It advises on where the technology will be commissioned by NHS England and under what circumstances. If your Trust does not meet the definition of a specialised centre and/or cannot meet the conditions within this letter and patients may benefit from the intervention then you should consider onward referral to a specialist metabolic disorders centre.

Following the publication of the positive Scientific Opinion on 14th July 2020, Trusts who provide specialised metabolic disorders services, as defined in Section 62 of The Manual (https://www.england.nhs.uk/publication/manual-for-prescribed-specialised-services-201718/), should use the EAMS application process to apply for access to lumasiran for eligible patients .

The Medicines and Healthcare Regulatory products Agency MHRA launched the Early Access to Medicines Scheme (EAMS) on 7th April 2014. The scheme is intended to enable patient access to medicines for treatment of life threatening or seriously debilitating conditions where there is an unmet need. The scheme offers a way by which unlicensed medicines can be made available to patients before approval of a license to benefit public health. It will also enable companies to gain additional knowledge and experience of these medicines in clinical use. (More information about the scheme can be found here: https://www.gov.uk/apply-for-the-early-access-to-medicines-scheme-eams)

Further to the designation of Promising Innovative Medicine (PIM) status for lumasiran in March 2020 (EAMS Step 1), MHRA issued a positive Scientific Opinion on 14th July 2020 which supports lumasiran:

> For the treatment of primary hyperoxaluria type 1 (PH1) in adults and children of all ages

A summary of the MHRA EAMS Public Assessment Report (PAR) is available here:

https://www.gov.uk/government/collections/early-access-to-medicines-scheme-eams-scientific-opinions

Following the publication of the positive Scientific Opinion on 14th July 2020, Trusts who provide specialist metabolic disorders services should use the EAMS application process to apply for access to lumasiran for eligible patients from this date.

For patients wishing to access lumasiran, Trusts must complete a Blueteq form to register each patient with NHS England once the application form has been submitted to Alnylam and the patient has been considered eligible for this EAMS. Further information regarding this can be requested from the dedicated EAMS email address: england.eams@nhs.net.

Patients must be informed that lumasiran is being made available ahead of a Marketing Authorisation decision through EAMS. Patients should be made aware that if lumasiran does not receive a Marketing Authorisation, or if NICE publishes negative guidance, or guidance that restricts the licensed population, then this will affect their continued treatment. They should also be made aware that if future recommendations are made restricting the length of the course of treatment then treatment provided during EAMS will count toward the recommended course length.

To register patients with Alnylam the treating clinician should email the Alnylam Early Access Program team (EAP@alnylam.com) who will arrange for the specialist vendor Clinigen to provide access to the Cliniport system. Clinigen will provide instructions for registering via Cliniport.

Once registered the patient details will be confirmed against the inclusion and exclusion criteria. For centres which have not been involved in the lumasiran clinical trial program, Alnylam can provide site training via Zoom.

Lumasiran is made available free of charge for EAMS patients during the EAMS period with associated activity costs covered by the appropriate commissioner. Following Marketing Authorisation of lumasiran (anticipated September 2020), free of charge supply under the EAMS arrangements will no longer be available for new patients. Free of charge supply will however continue for patients who commenced treatment during the EAMS period until reimbursement.

NICE are appraising the product as a priority. Should NICE issue positive Technology Appraisal guidance, this guidance will usually be implemented 30 days after final guidance is published.

Providers of specialised metabolic disorders services should engage with their local specialised commissioning team to consider the implications of

implementing this scheme.

I would be grateful if you could cascade this information to relevant clinical teams within your organisation to support the consistent adoption of the policy nationally.

Yours sincerely,

Tracey Williams
Principal Pharmacist