Medicines & Healthcare products Regulatory Agency

MHRA

Early Access to Medicines Scheme – Treatment protocol – Information on the pharmacovigilance system and requirements for reporting safety data

Introduction

The aim of the Early Access to Medicines Scheme (EAMS) is to provide earlier availability of promising new unlicensed medicines and medicines used outside their licence, to UK patients that have a high unmet clinical need. The medicinal products included in the scheme are those that are intended to treat, diagnose or prevent seriously debilitating or life-threatening conditions where there are no adequate treatment options.

The scientific opinion is based on assessment of the information supplied to the MHRA on the benefits and risks of the medicine. As such this is a scientific opinion and should not be regarded as a licensed indication or a future commitment by the MHRA to licence such a medicine, nor should it be regarded as an authorisation to sell or supply such a medicine. A positive scientific opinion is not a recommendation for use of the medicine and should not be interpreted as such. Under EAMS the risk and legal responsibility for prescribing the medicine remains with the physician, and the opinion and EAMS documentation published by the MHRA are intended only to inform physicians' decision making and not to recommend use. An EAMS scientific opinion does not affect the civil liability of the manufacturer or any physician in relation to the product.

As the safety profile of the EAMS medicine may not yet be fully established it is particularly important that any harmful or unintended responses to EAMS medicines are reported. More information about the scheme can be found here:
http://www.mhra.gov.uk/Howweregulate/Innovation/EarlyaccesstomedicinesschemeEAMS/index.htm

Physicians should enroll any patients receiving EAMS medicines in the drug registry put in place by the pharmaceutical company to enable systematic collection of information on adverse events. Suspected adverse drug reactions (ADRs) for any patients can also be reported directly to the MHRA via the Yellow card scheme at www.mhra.gov.uk/yellowcard. When reporting please provide as much information as possible, including information about medical history, any concomitant medication, onset, treatment dates, outcome and results of any test results or investigations.

The information below is intended for healthcare professionals and is provided by the pharmaceutical company that manufactures the EAMS medicine. It summarises the requirements for clinical monitoring and reporting of adverse events with medicines used under the scheme.

Healthcare professionals should also consult the relevant detailed information provided by the company.

Information on the Pharmacovigilance System:

Provision of lumasiran through the Early Access to Medicines Scheme (EAMS) is designed to provide early access to this medicine for the treatment of Primary Hyperoxaluria Type 1 (PH1) in adults and paediatrics of all ages.

All patients should be made aware of the benefits and risks associated with the treatment as outlined in the Treatment Protocol for Healthcare Professionals and Treatment Protocol for Patients. Patients or legal guardians will be required to sign an Informed Consent Form / Assent Form to acknowledge their understanding and consent to their information being shared with Alnylam.

The prescribing physician will request access to lumasiran from Alnylam for each individual patient by completing a Patient Access Form. Following confirmation of eligibility by Alnylam, physicians will work with Clinigen, Alnylam's designated third party, who will provide the necessary EAMS-related materials to the physician in the form of a physician pack. The physician pack includes:

- Patient Access Form
- Treatment Protocol for Health Care Professional (HCP)
- Treatment Protocol for Patients (Information for Patients)
- Adverse Event Reporting Guidelines
- Individual Safety Reporting Forms (for HCPs)
- Discontinuation Form
- Patient Consent / Assent Forms

In addition, healthcare professionals will receive training on the appropriate use and administration of lumasiran and the recognition and reporting of adverse events.

Patients will be instructed to report any AEs they experience to their physician or directly to the MHRA via the yellow card scheme www.mhra.gov.uk/yellowcard or call toll free at 0800 731 6789 (10am to 2pm Monday-Friday only).

Active Pharmacovigilance Reporting:
All HCPs, (physicians, pharmacists and nurses) involved with the EAMS programme will be directed to report the following (Pharmacovigilance Data) using the 'EAMS lumasiran adverse event report form' included in the physician's pack. The Adverse Event Reporting Guidelines document will indicate the submission timeline requirements for Adverse Events.

- Any adverse events (AEs) regardless of seriousness or causality (please note this includes any AEs listed in the Treatment Protocol)
- Overdose
- Drug misuse
- Drug abuse
- Lack of effect
- Medication error
- Occupational exposure
- Off label use
- Exposure during pregnancy or lactation

A unique patient identifier will be generated by Alnylam's designated third party, Clinigen, once an individual patient is accepted into EAMS. This unique identifier will take the format of LUMA-XXXX where LUMA stands for lumasiran. The unique identifier will be used to track and deliver drug supply to/from the hospital pharmacy and will also be used for monitoring safety information. This number will remain in a database of patients entered into EAMS.

In the event that a patient enrols in EAMS but does not receive treatment, the unique identifier will not be reused.

The prescribing physician is also requested to report if a patient discontinues treatment by completing an EAMS Discontinuation Form with the last date of treatment and the reason for discontinuation. If the reason for discontinuation is due to an adverse event, this should also be reported as per the procedures for reporting an AE/SAE.

For AEs that are considered AEs of Clinical Interest (AECI), Alnylam should be notified using the AE Report Form. Additional clinical and laboratory information may be collected.

For all injection site reactions (ISRs), the physician/HCP should submit additional information regarding each ISR in the AE Report Form (eg, symptom(s), injection-site location, follow-up actions taken, etc.).

Any AEs characterized as intermittent requires documentation of the start and stop of each incidence.

An assessment of severity should be made for each AE as well as an assessment of causality. All AEs should be reported regardless of causality assessment. All AEs should be followed up by the physician until resolution or stabilization of the event.

Contact Details:

EAMS Queries:
To request access to EAMS or EAMS related enquiries: EAP@alnylam.com

Pharmacovigilance Reporting: All reporting forms should be submitted to Clinigen at: drugsafety@clinigengroup.com

Alnylam Medical Information Hotline:
Toll: +44 162 88 78592
Toll-free: 08001412569
medinfo@alnylam.com