Medicines & Healthcare products Regulatory Agency

MHRA

SECOND RENEWAL of Early Access to Medicines Scientific Opinion – ANNEX to Public Assessment Report. The renewal is effective from 21st June 2019 and is valid for 12 months.

Raxone 150 mg film-coated tablets (idebenone)
As treatment for slowing the decline of respiratory function in patients with Duchenne Muscular Dystrophy (DMD) from the age of 10 years who are currently not taking glucocorticoids. The decline of respiratory function must be confirmed by repeated measurements of pulmonary function prior to initiation of treatment (see section 5.1). Raxone can be used in patients previously treated with glucocorticoids or in patients in whom glucocorticoid treatment is not tolerated or is considered inadvisable (see section 4.4).
Footnote:
DMD patients who are receiving therapeutic doses of glucocorticoid for the treatment of DMD are not eligible to receive Raxone through EAMS; however, patients receiving no more than physiological doses of glucocorticoid as replacement therapy for adrenal suppression due to prior longstanding therapeutic doses of glucocorticoid, can be enrolled to receive Raxone through EAMS.

Santhera Pharmaceuticals
EAMS number 46555/0001

Introduction

The aim of the Early Access to Medicines Scheme (EAMS) is to provide earlier availability of promising new unlicensed medicines to UK patients that have a high unmet clinical need. The MHRA scientific opinion provides benefit and risk information to doctors who may wish to prescribe the unlicensed medicine under their own responsibility. More information about the scheme can be found here: http://www.mhra.gov.uk/Howweregulate/Innovation/EarlyaccesstomedicinesschemeEAMS/index.htm

The scientific opinion is based on the information supplied to the MHRA on the benefits and risks of a promising new medicine. As such this is a scientific opinion and should not be regarded as a medicine licensed by the MHRA or a future commitment by the MHRA to license such a medicine. The General Medical Council's guidance on prescribing unlicensed medicines can be found here: https://www.gmc-uk.org/guidance/28349.asp

Background

An EAMS scientific opinion was granted by the MHRA on 21st June 2017 for Raxone as treatment for slowing the decline of respiratory function in patients with DMD from the age of 10 years who are currently not taking glucocorticoids.

In June 2018 the Licensing Authority, on the recommendation of the UK Commission on Human Medicines (CHM), renewed the EAMS scientific opinion for a further 12 months.

https://www.gov.uk/government/publications/early-access-to-medicines-scheme-eams-scientific-opinion-raxone-to-treat-the-decline-of-respiratory-function-in-patients-with-duchenne-muscular-dys

EAMS Scientific Opinion Second Renewal

In May 2019, CHM considered the Company's request for a renewal of the EAMS scientific opinion for Raxone for a further twelve months from June 2019. The basis of the request was to continue making idebenone available to DMD patients with the highest need, while a conditional marketing authorisation application to the European Medicines Agency for idebenone (under the invented name Puldysa) to treat respiratory function decline in DMD is underway.

A conditional marketing authorisation can be granted where "*In the interests of public health, applicants may be granted a conditional marketing authorisation where the benefit of immediate availability outweighs the risk of less comprehensive data than is normally required*" and where it is likely that confirmatory data will be obtained.

CHM noted that no new safety issues have been identified in the EAMS reporting period. To support the request to renew the EAMS scientific opinion, the Company submitted a limited amount of new efficacy data in a patient population consistent with the EAMS indication (the SYROS study). The new data support the potential for clinically relevant slowing of respiratory function decline when Raxone treatment is continued for longer periods than 12 months.

CHM concluded that benefit-risk remains positive on the basis of the available data which are still limited but will be expanded to a more comprehensive dataset in the ensuing confirmatory studies that are planned and in progress. Likewise, confirmatory evidence will be necessary to inform a decision on progression from conditional to full marketing authorisation.

CHM, in making its recommendation to renew the EAMS, noted common ground in the aims of access to a medicine through EAMS and access through a conditional marketing authorisation.

The EAMS scientific opinion is renewed from 21st June 2019 for a further twelve months. The medicine will continue to be subject to the compulsory EAMS reporting requirements, with periodic reporting of adverse event data. The Company is also obliged to inform the MHRA of any alteration in benefit-risk.