



Early Access to Medicines Scientific Opinion – Annex to Public Assessment Report THIRD RENEWAL	
Product	Idebenone (Raxone / Puldysa) 150 mg film-coated tablets
EAMS indication	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). <i>Footnote:</i> <i>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.</i>
Company	Santhera Pharmaceuticals (Deutschland) GmbH
EAMS number	46555/0001
EAMS Scientific Opinion third renewal date	21 st June 2020 The renewal is effective from 21 st June 2020 and is valid for 12 months.

Introduction

The aim of the Early Access to Medicines Scheme (EAMS) is to provide earlier availability of promising new unlicensed medicines to UK patients where there is 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 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 medicine licensed by the MHRA or a future commitment by the MHRA to license 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 a 'special' 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.

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 (idebenone) 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, and subsequently in June 2019, the Licensing Authority renewed the EAMS scientific opinion for a further 12 months on the recommendation of the UK Commission on Human Medicines (CHM).

<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 Third Renewal

In June 2020, CHM considered the Company's request for a renewal of the EAMS scientific opinion for Idebenone (Raxone) for a further twelve months from June 2020. 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 to treat respiratory function decline in DMD is under evaluation. The Company has applied for a conditional marketing authorisation (CMA) for idebenone (Puldysa) under a new licence. This is because a CMA cannot be awarded for Raxone under its existing product licence. Puldysa and Raxone contain the same active substance, idebenone, have an identical pharmaceutical composition and are both presented as 150 mg film-coated tablets.

A CMA may be granted for a medicine where "*the benefit of immediate availability outweighs the risk of less comprehensive data than is normally required*" and where it is likely that comprehensive, confirmatory data will be obtained to inform progression to a full marketing authorisation.

The European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) is expected to issue an opinion on whether to grant a CMA for Puldysa towards the end of 2020.

Should a CMA be granted, the EAMS scientific opinion will expire in the same way it would for a full marketing authorisation. Patients may be enrolled into the Raxone EAMS programme up to the date on which the European Commission issues its decision on the CMA for Puldysa.

CHM noted that no new safety issues have been identified in the EAMS reporting period.

CHM concluded that the EAMS scientific opinion should be renewed.

The EAMS scientific opinion is renewed from 21st June 2020 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.