Medicines & Healthcare products Regulatory Agency

MHRA

Early Access to Medicines Scientific Opinion – Annex to Public Assessment Report FIRST RENEWAL	
Product	**Avalglucosidase alfa**
EAMS indication	**Treatment of late-onset Pompe disease (LOPD) in patients with symptoms and who have received alglucosidase alfa (Myozyme) for at least 2 years.** **Treatment of infantile-onset Pompe disease (IOPD) in patients at least 1 year old who have symptoms and have received alglucosidase alfa (Myozyme) for at least 6 months.**
Company	**Aventis Pharma Ltd t/a Sanofi**
EAMS number	**04425/0004**
EAMS Scientific Opinion first renewal date	**24 March 2022** **The renewal is effective from 24 March 2022 and is valid for 12 months**

WITHDRAWN

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 MHRA scientific opinion provides benefit and risk information to physicians who may wish to prescribe the EAMS 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/ethical_guidance/14327.asp

Background

An EAMS scientific opinion was granted by the MHRA on 05 March 2021 for Avalglucosidase alfa in the treatment of late-onset Pompe disease (LOPD) in symptomatic patients who have received Pompe disease ERT with alglucosidase alfa (Myozyme) for ≥ 2 years and in the treatment of infantile-onset Pompe disease (IOPD) in symptomatic patients ≥ 1 year old who have received Pompe disease ERT with alglucosidase alfa (Myozyme) for ≥ 6 months.

EAMS Scientific Opinion First Renewal

In February 2022 the Company submitted a request for a renewal of the EAMS Scientific Opinion for Avalglucosidase alfa for a further 12 months from March 2022.

There are no significant changes to the previous knowledge of safety and efficacy resulting from information gained since the scientific opinion was granted.

The product continues to meet the EAMS criteria and no changes are required to the treatment protocols.

The benefit risk balance for the product remains positive and the EAMS scientific opinion can be renewed.

The EAMS scientific opinion is renewed from 24 March 2022 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.

WITHDRAWN