Medicines & Healthcare products Regulatory Agency

MHRA

MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

mhra.gov.uk

Information for NHS Medical Directors

DISCONTINUATION OF EAMS PROGRAMME FOR IDEBENONE (RAXONE)

Effective date: 3 months from 12th October 2020

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.

Santhera Pharmaceuticals
EAMS number 46555/0001

Summary of basis for discontinuation of EAMS:

Santhera Pharmaceuticals has taken a decision to discontinue the EAMS programme for idebenone (Raxone) to take effect in 3 months from 12th October 2020. The MHRA will withdraw the EAMS scientific opinion for idebenone, to coincide with the discontinuation of the EAMS programme.

Santhera has also decided to withdraw the application made to the European Medicines Agency for a conditional marketing authorisation of idebenone (under the trade name Puldysa) and all ongoing clinical development of idebenone in DMD is being halted.

The decision to discontinue the EAMS programme, and all ongoing clinical development of idebenone in DMD, is based on new evidence from a Phase III trial in DMD patients ("SIDEROS") that idebenone no longer offers promise to slow the decline in respiratory function, that had been suggested by a previous study ("DELOS"). The later study (SIDEROS) is a more definitive study in a larger number of patients and is considered to provide more reliable evidence of the presence or absence of benefit. There are no serious safety concerns with the medicine.

The award of an EAMS scientific opinion takes into consideration the degree of unmet need in the patient population. Where there is a high degree of need, an EAMS scientific opinion can be awarded in the presence of preliminary data provided robust confirmatory data are forthcoming, to inform a final conclusion on the balance of benefits and risks of a medicine. This is similar to the evaluation of a medicine for a conditional marketing authorisation. Those confirmatory data are now available for idebenone, the conclusion from which is that the prior promise of benefit is no longer sustained.