Vamorolone Designated Promising Innovative Medicine (PIM) for treatment in Duchenne muscular dystrophy

Rockville, MD, 17 October 2019

In the UK the Early Access to Medicines Scheme (EAMS) is a regulatory path by the MRHA that aims to give patients with life threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorization when there is a clear unmet medical need. The initial step in this process is Promising Innovative Medicine (PIM) designation.

Co-founders of Duchenne UK Alex Johnson and Emily Crossley said, "We are delighted that MHRA has given PIM status to Vamorolone as a treatment for Duchenne Muscular Dystrophy. A PIM designation is the first step of a process that could allow patients earlier access to a new medicine. This is part of the Early Access to Medicines Scheme which Duchenne UK and Joining Jack lobbied for in 2014. We are pleased to see that the scheme may be used for Vamorolone."

UK foundations that have aided the development of vamorolone for DMD include Joining Jack, Duchenne Children's Trust, ActionDuchenne, Alex's Wish Foundation, and Duchenne Research Fund.

Vamorolone is a first-in-class drug that targets multiple biochemical pathways in DMD patient muscle simultaneously, and in initial open label studies has shown improvements of patient muscle function. A pivotal trial that may lead to drug approval is currently enrolling patients age 4 to 7 years at 6 sites in the United Kingdom (Newcastle University, Royal Hospital for Children [Glasgow], Alder Hey Children's Hospital [Liverpool], Leeds Teaching Hospital Trust, Great Ormond Street Institute of Child Health [London] and University Hospitals Birmingham). Information on the currently recruiting vamorolone clinical trial with contact information for UK recruitment sites can be found at clinicaltrials.gov.

About the UK Early Access to Medicines Scheme (EAMS)

The UK's industry-sponsored EAMS aims to give patients with life threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorization when there is a clear unmet medical need. The EAMS is a two-step process:

Step I is the Designation as a Promising Innovation Medicine (PIM). The PIM designation is an early indication that a medicinal product is a promising candidate for EAMS and gives reassurance that its clinical development is on track by having an early review of its data by the medicines regulator.

Step II is the Scientific Opinion by the Medicines and Healthcare products Regulatory Agency (MHRA, UK regulatory agency). The Scientific Opinion describes the benefits and risks of the medicine and supports the prescriber and patient to make a decision on using the medicine before its license is approved.