24 May 2023

Dear UK Duchenne community,

Following your request to receive timely updates about the Roche and Sarepta research efforts in Duchenne muscular dystrophy (DMD), please find below an update on the regulatory filing and review of delandistrogene moxeparvovec (also known as SRP-9001) in the US.

What has been announced?
Following the U.S. Food and Drug Administration (FDA) advisory committee meeting, Sarepta had an informal telephone conference with the FDA to discuss the next regulatory steps for the accelerated Biologics License Application (BLA) review. The FDA has indicated that, subject to the completion of the review of delandistrogene moxeparvovec for the treatment of Duchenne muscular dystrophy, it is working toward potentially granting an accelerated approval, initially for use in Duchenne patients aged 4-5 years old.

EMBARK, the global, randomized, double-blind, placebo-controlled Phase 3 trial for delandistrogene moxeparvovec, is the proposed confirmatory study. The FDA has informed Sarepta that, if the trial meets its objectives, the Agency will consider a non-age restricted expansion of the delandistrogene moxeparvovec label. EMBARK is fully enrolled, with top-line results expected in the fourth quarter of 2023.

The FDA has also informed Sarepta that it requires additional time to complete the review, including final label negotiations and postmarketing commitment discussions, and has set a new PDUFA date (the goal date set by the FDA for announcing its decision) of June 22, 2023.

For further information, please see the Sarepta press release here[1].

What does this mean for patients in the UK?
Different health authorities act independently, following distinct processes with different filing requirements. Although their ultimate goals are the same for their geographical regions, they have distinct regulations and procedures, meaning there are differences in how medicines are approved.

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[1] https://investorrelations.sarepta.com/news-releases/news-release-details/sarepta-therapeutics-announces-update-regulatory-review-srp-9001

The MHRA is the health authority in the UK and is responsible for issuing Marketing Authorisations for medicines. We are engaging with the MHRA to determine the best possible route to achieving a potential future license for delandistrogene moxeparvovec. It is worth noting that in the UK, regulatory approval is only the first step. Further assessments of clinical and cost-effectiveness by the National Institute for Health and Care Excellence (NICE) and the Scottish Medicines Consortium (SMC) must also be undertaken before a medicine is available to patients. We will share updates in due course.

You are welcome to share this update with the UK community. If there are any questions about this update, please do not hesitate to contact medinfo.uk@roche.com.

Thank you very much.

Kind regards,
The Roche UK DMD team

Document number: M-GB-00012534
May 2023