Roche DMD Team: End of Year Duchenne Community Update

FAO: UK Duchenne Community
December 2022

Dear UK Duchenne Community,

As we approach the end of the year, in response to your request to receive regular updates, we would like to share a short summary of our activities surrounding delandistrogene moxeparvovec (SRP-9001) for the treatment of Duchenne muscular dystrophy (DMD), which Roche is developing in partnership with Sarepta.

As a reminder, in December 2019 Roche and Sarepta Therapeutics entered into a licensing agreement for delandistrogene moxeparvovec (SRP-9001). This agreement means that Roche is responsible for launching and commercialising delandistrogene moxeparvovec outside of the United States[1], whilst Sarepta Therapeutics is responsible for launching and commercialising delandistrogene moxeparvovec within the United States.

Over the last 12 months, with your continued contributions and collaborations, we advanced our research and achieved new milestones. We have presented key data from the delandistrogene moxeparvovec clinical development programme at major congresses worldwide, reached new clinical milestones and shared important regulatory updates.

We thank you for your collaboration with Roche, and look forward to continuing to work together in 2023.

Delandistrogene moxeparvovec ongoing clinical studies

Study 101[2]	**Study 102**[3]	**ENDEAVOR Study 103**[4]	**EMBARK Study 301**[5]
A Phase 1/2a study of delandistrogene moxeparvovec in four individuals with Duchenne aged between 4 and 7 years ClinicalTrials.gov NCT03375164	A Phase 2 placebo controlled study of delandistrogene moxeparvovec in individuals with Duchenne aged between 4 and 7 years who are ambulatory ClinicalTrials.gov NCT03769116	A Phase 1b study of delandistrogene moxeparvovec in a large population of 38 individuals ambulatory and non ambulatory with Duchenne over 3 years ClinicalTrials.gov NCT04626674	A Phase 3 double blind, placebo controlled study of delandistrogene moxeparvovec in 125 ambulatory individuals with Duchenne aged between 4 and 7 years ClinicalTrials.gov NCT05096221

Clinical milestones in 2022

- The first long-term, 4-year data for delandistrogene moxeparvovec have been presented.

[1] https://www.roche.com/media/releases/med-cor-2019-12-23

- The global Phase 3 study, EMBARK, which aims to assess the safety and efficacy of delandistrogene moxeparvovec in a large population of individuals with Duchenne, has now completed enrollment.

Key data releases at congresses in 2022

Study 101

- Long-term, 4-year safety and efficacy results were presented at the 2022 International Congress on Neuromuscular Diseases (ICNMD) (Brussels, Belgium, 5–9 July 2022).

Study 102

- First results from the second part of the trial (where patients initially treated with placebo [no drug] in the first part of the trial received delandistrogene moxeparvovec) were presented at the 2022 Muscular Dystrophy Association (MDA) Conference (Nashville, Tennessee and virtual, 13–16 March 2022).
- Full safety, biological and functional results for patients who have been treated with delandistrogene moxeparvovec up to 2 years post-infusion were presented at the 2022 27th International Annual Congress of the World Muscle Society (WMS) (Halifax, Canada, 11–15 October 2022).

ENDEAVOR (Study 103)

- One-year functional data from Cohort 1 (individuals with Duchenne aged between 4 and 7 years) were presented at ICNMD 2022. This is the first study evaluating the intended commercial process delandistrogene moxeparvovec material.

Integrated Analysis

- An integrated (combined) analysis of the safety and efficacy of delandistrogene moxeparvovec treatment across Study 101, Study 102 and ENDEAVOR was presented at ICNMD 2022.

EMBARK (Study 301)

- The study design of the pivotal Phase 3 study was presented at MDA 2022.

We continued to share our data at other major congresses.

Regulatory activities for delandistrogene moxeparvovec over the past year

- We are pleased to inform you that the U.S. Food and Drug Administration (FDA) has accepted the Biologics License Application (BLA) submitted by Sarepta Therapeutics for delandistrogene moxeparvovec for the treatment of ambulant individuals living with DMD. The FDA has granted the application a Priority Review and is expected to announce its decision on approval with a target goal date of May 29, 2023.[1]
- Roche acknowledges the urgent unmet need and is working with respective healthcare authorities to prepare for regulatory submission of delandistrogene moxeparvovec outside

of the US. We will keep you updated as we progress in these discussions with authorities including with the MHRA in the UK.

- 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.

Looking towards 2023

- The results from the first part of the Phase 3 study EMBARK are expected in the second half of 2023.
- Further studies are expected; additional details will be shared in due course.
- We look forward to engaging and collaborating with the Duchenne community in the coming year.

With kind regards,
The Roche UK DMD Team

References

1. *Sarepta Therapeutics. Sarepta Therapeutics Announces That U.S. FDA has Accepted for Filing and Granted Priority Review for the Biologics License Application for SRP-9001, Sarepta's Gene Therapy for the Treatment of Ambulant Individuals with Duchenne Muscular Dystrophy.* https://investorrelations.sarepta.com/news-releases/news-release-details/sarepta-therapeutics-announces-us-fda-has-accepted-filing-and?_ga=2.32279038.1586864239.1669653463-1704498055.1658248749 *(Accessed November 2022).*
2. *ClinicalTrials.gov NCT03375164 (Accessed November 2022).*
3. *ClinicalTrials.gov NCT03769116 (Accessed November 2022).*
4. *ClinicalTrials.gov NCT04626674 (Accessed November 2022).*
5. *ClinicalTrials.gov NCT05096221 (Accessed November 2022).*