

Notification of discontinuation of a paediatric development which is covered by an agreed PIP Decisionⁱ

Active substances(s): Losmapimod

Latest Decision number(s): P/0116/2024

Corresponding PIP number(s): EMEA-003448-PIP01-23

If the PIP has been submitted as part of a marketing authorisation application in order to comply with the requirements of Article 7 of the Paediatric Regulation (as a condition of the validation of the respective application) and a marketing authorisation was granted based on this application, then there is a legal obligation to complete that PIP. The same applies if there has been a successful post-authorisation application, where the PIP was included in order to comply with the requirements of Article 8 of the Paediatric Regulation.

Please confirm if any of the above applies:

Yes ☐ No ☒

If yes, it means that based on the Marketing Authorisation obtained at the end of that initial procedure or the successful post-authorisation application, as applicable, you are obliged to complete that PIP. That obligation cannot be cancelled by a unilateral decision, including by withdrawing the MA. Such PIP must be completed, unless it is modified in agreement with the PDCO by removing all outstanding PIP measures or granting a full product-specific waiver instead (upon relevant circumstances in accordance with the Paediatric Regulation). Non-completion of a binding PIP establishes noncompliance with the requirements of the Paediatric Regulation, which the European Medicines Agency has an obligation to report to the European Commission.

Please note that development of the medicinal product above in the following **condition(s)/indication(s)**:

Treatment of facioscapulohumeral muscular dystrophy

☒ has been discontinued

for the following reason(s): (tick all that apply)

- ☒ (possible) lack of efficacy in adults
- ☐ (possible) lack of efficacy in children
- ☐ (possible) unsatisfactory safety profile in adults
- ☐ (possible) unsatisfactory safety profile in children
- ☐ commercial reasons (please specify:)
- ☐ manufacturing / quality problems
- ☐ other regulatory action (please specify:)
- ☐ other reason (please specify:)

Please add a brief description (max 2000 characters) of the reason(s) for the discontinuation:

REACH was a Phase 3 multi-centre, randomized, double-blind, placebo-controlled trial designed to evaluate the efficacy and safety of losmapimod for the treatment of facioscapulohumeral muscular dystrophy (FSHD) in adults.

The trial enrolled 260 patients who were randomized 1:1 to receive either losmapimod, administered orally as a 15 mg tablet twice a day, or placebo over a 48-week treatment period. The primary endpoint was the absolute change from baseline in Reachable Workspace (RWS). Secondary endpoint measurements included Muscle Fat Infiltration (MFI) as measured by MRI, shoulder abductor strength as measured by hand-held dynamometry, Patient Global Impression of Change (PGIC), and the Neuro QoL Upper Extremity.

On 12 September 2024, Fulcrum Therapeutics announced its Phase 3 REACH trial did not achieve its primary endpoint of change from baseline in relative surface area (RSA) with losmapimod compared to placebo. In addition, secondary endpoints did not achieve nominal statistical significance. The safety and tolerability profile of losmapimod was considered consistent with previously reported studies. Therefore, development of losmapimod in FSHD is discontinued.

Date: 10/12/2024

Contact for inquiries from interested parties:

Telephone: +31 2024 54000

Email: info.nl@sanofi.com

ⁱ This form will be published to the corresponding decision available on the website of the European Medicines Agency.