

To:

Head of Paediatric Medicines
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Notification of discontinuation of a paediatric development which is covered by an agreed PIP Decision

Actives substances(s): PF-06252616

Invented name: domagrozumab

Latest Decision number(s): 1) P/0108/2016 2) P/0327/2017 3) P/ 4)
P/

Corresponding PIP number(s): 1) EMEA-001763-PIP01-15 2) EMEA-001763-PIP01-15-M01
3) EMEA- 4) EMEA-

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

Duchenne Muscular Dystrophy

- ☐ has been discontinued
- ☒ has been suspended/put on long-term hold (with possible re-start at a later time)

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:) (e.g. suspension, revocation of M.A.)
- ☐ other reason (please specify:)

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

Discontinuation of the domagrozumab PIP is a consequence of the results of the primary analysis of study B5161002. Study B5161002 did not meet its primary efficacy endpoint, which was to demonstrate a difference in the mean change from baseline in 4 Stair Climb (in seconds) following one year of treatment with domagrozumab as compared to placebo in patients with DMD. Further evaluation of the totality of evidence including secondary endpoints did not support a significant treatment effect. The decision was made after a thorough review of data available at the time of the primary analysis, which evaluated all study participants after one year of treatment, as well as those participants who were in the trial beyond one year. Studies B5161002 and B5161004 (long term follow up) were not terminated for safety reasons

Name and signature of the PIP contact point: Signature on file

Date: 16th January 2019

Contact for inquiries from interested parties: Pfizer Ltd

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