MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

agree a modification of an agreed paediatric investigation plan.

MHRA-100126-PIP01-21-M01

Scope of the Application

Active Substance(s)

Adeno-associated viral vector serotype 9 containing the human mini-dystrophin gene

Condition(s)

Duchenne Muscular Dystrophy

Pharmaceutical Form(s)

Solution for infusion

Route(s) of Administration

Intravenous use

Name / Corporate name of the PIP applicant

Pfizer Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Pfizer Limited submitted to the licensing authority on 14/05/2021 15:20 BST an application for a Modification

The procedure started on 30/07/2021 14:23 BST

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to agree a modification of an agreed paediatric investigation plan.

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-100126-PIP01-21-M01

Of 20/05/2022 09:51 BST

On the adopted decision for Adeno-associated viral vector serotype 9 containing the human mini-dystrophin gene (MHRA-100126-PIP01-21-M01) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan)

This decision applies to a Modification for Adeno-associated viral vector serotype 9 containing the human mini-dystrophin gene, Solution for infusion , Intravenous use .

This decision is addressed to Pfizer Limited, Ramsgate Road, Sandwich, United Kingdom, CT13 9NJ

ANNEX I

1. Waiver

1.1 Condition:

Not Applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Duchenne Muscular Dystrophy

2.2 Indication(s) targeted by the PIP:

Treatment of Duchenne Muscular Dystrophy

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

All subsets of the paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Solution for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	0
Non-Clinical Studies	0	0
Clinical Studies	4	Study 1 (C3391001) Open-label, single arm, single ascending dose study to evaluate safety and tolerability of PF-06939926 in ambulatory and non-ambulatory patients with DMD. Study 2 (C3391003) Randomized (2:1), double-blind, placebo-controlled study to evaluate safety and efficacy of PF-06939926 in ambulatory paediatric patients from 4 to less than 8 years of age with DMD. Study 3 (C3391002)Randomized (1:1), double-blind, placebo-controlled study to evaluate safety and efficacy of PF-06939926 in non-ambulatory population with DMD. Study 4 (C3391008) Single arm, open-label safety study of PF-06939926 in paediatric patients from birth to less than 4 years of age with DMD.
Extrapolation, Modeling & Simulation Studies	0	0
Other Studies	0	0
Other Measures	0	0

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	30/06/2036

Deferral of one or more studies contained in the paediatric investigation plan:	Yes