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 paediatric investigation plan and grant a deferral and grant a waiver

MHRA-100299-PIP01-21

Scope of the Application

Active Substance(s)

Humanized monoclonal IgG1-based antibody

Condition(s)

Treatment of spinal muscular atrophy

Pharmaceutical Form(s)

Solution for injection

Route(s) of Administration

Subcutaneous use

Name / Corporate name of the PIP applicant

ROCHE PRODUCTS LIMITED

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, ROCHE PRODUCTS LIMITED submitted to the licensing authority on 23/01/2023 16:18 GMT an application for a Paediatric Investigation Plan

The procedure started on 15/06/2023 12:02 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 paediatric investigation plan and grant a deferral and grant a waiver

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-100299-PIP01-21

Of 14/08/2023 14:46 BST

On the adopted decision for Humanized monoclonal IgG1-based antibody (MHRA-100299-PIP01-21) 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 a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for Humanized monoclonal IgG1-based antibody , Solution for injection , Subcutaneous use .

This decision is addressed to ROCHE PRODUCTS LIMITED , 6 FALCON WAY, SHIRE PARK, WELWYN GARDEN CITY, United Kingdom, AL7 1TW

ANNEX I

1. Waiver

1.1 Condition:

Treatment of spinal muscular atrophy The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 months of age Pharmaceutical form(s): Solution for injection Route(s) of administration: Subcutaneous use Reason for granting waiver: on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of spinal muscular atrophy

2.2 Indication(s) targeted by the PIP:

Treatment of spinal muscular atrophy

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

The paediatric population from 2 months to less than 18 years of age

2.4 Pharmaceutical Form(s):

Solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 1 (BN42644) Two-part, double blind, randomised, placebo controlled study to evaluate pharmacokinetics (PK), pharmacodynamics (PD), safety, efficacy and tolerability of RO7204239 in combination with risdiplam in ambulant children from 2 years to less than 18 years of age (and adults) with spinal muscular atrophy (SMA). Study 2 (BN43504) Double blind, randomised, placebo controlled study to evaluate pharmacokinetics, pharmacodynamics, safety, efficacy and tolerability of RO7204239 in combination with risdiplam in non-ambulant children from 2 years to less than 18 years of age (and adults) with spinal muscular atrophy. Study 3 (BN44374) Open-label, uncontrolled, single-arm study to evaluate pharmacokinetics, pharmacodynamics, safety and tolerability of RO7204239 in combination with risdiplam in children from 2 months to less than 2 years with spinal muscular atrophy.
Extrapolation, Modeling & Simulation Studies	0	Not applicable.
Other Studies	0	Not applicable.

Other Measures	0	Not applicable.

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

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	31/08/2032
Deferral of one or more studies contained in the paediatric investigation plan:	Yes