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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-101292-PIP01-23

Scope of the Application

Active Substance(s)

Complement Factor B Antisense Oligonucleotide (RO7434656)

Condition(s)

Treatment of primary immunoglobulin A nephropathy

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 17/01/2024 10:47 GMT an application for a Paediatric Investigation Plan

The procedure started on 06/08/2024 15:13 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.

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

gov.uk/mhra

Final Decision Letter

MHRA-101292-PIP01-23

Of 05/09/2024 08:56 BST

On the adopted decision for Complement Factor B Antisense Oligonucleotide (RO7434656) (MHRA-101292-PIP01-23) 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 Complement Factor B Antisense Oligonucleotide (RO7434656), 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 primary immunoglobulin A nephropathy (IgAN) The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Solution for injection Route(s) of administration: SUBCUTANEOUS USE Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of primary immunoglobulin A nephropathy (IgAN)

2.2 Indication(s) targeted by the PIP:

Treatment of primary immunoglobulin A nephropathy

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

The paediatric population from 2 years 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 Single-arm, open-label study to evaluate safety, tolerability, pharmacokinetics, pharmacodynamics and activity of RO7434656 in children from 2 years to less than 18 years of age with primary immunoglobulin A nephropathy.
Extrapolation, Modeling & Simulation Studies	3	Study 2 Modelling and simulation study to evaluate the use of RO7434656 in the treatment of primary immunoglobulin A nephropathy in children from 2 years to less than 18 years of age. Study 3 Analysis of existing data on efficacy, safety, pharmacokinetics and pharmacodynamics of RO7434656 in children and adolescents 2 years to less than 18 years of age with primary immunoglobulin A nephropathy. Extrapolation plan Studies 1, 2 and 3 are part of an extrapolation plan covering the paediatric population from 2 years to less than 18 years of age with primary immunoglobulin A nephropathy.
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:	30/11/2033
Deferral of one or more studies contained in the paediatric investigation plan:	Yes