MHRA
10 South Colonnade
Canary Wharf
London
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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-100363-PIP01-21

Scope of the Application

Active Substance(s)

Repagermanium

Condition(s)

Treatment of focal segmental glomerulosclerosis (FSGS)

Pharmaceutical Form(s)

Capsule, hard

Route(s) of Administration

Oral use

Name / Corporate name of the PIP applicant

Dimerix Bioscience Pty Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Dimerix Bioscience Pty Ltd submitted to the licensing authority on 14/12/2021 15:51 GMT an application for a Paediatric Investigation Plan

The procedure started on 01/02/2022 09:42 GMT

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-100363-PIP01-21

Of 24/04/2024 13:55 BST

On the adopted decision for Repagermanium (MHRA-100363-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 Repagermanium, Capsule, hard , Oral use .

This decision is addressed to Dimerix Bioscience Pty Ltd, 425 Smith Street, Victoria, Australia, 3065

ANNEX I

1. Waiver

1.1 Condition:

Treatment of focal segmental glomerulosclerosis (FSGS) The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 1 year of age Pharmaceutical form(s): Capsule, hard Route(s) of administration: Oral 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 focal segmental glomerulosclerosis (FSGS)

2.2 Indication(s) targeted by the PIP:

Treatment of focal segmental glomerulosclerosis in paediatric patients aged 1 year to less than 18 years who are concomitantly being treated with an angiotensin receptor blocker (ARB).

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

The paediatric population from 1 year to less than 18 years of age

2.4 Pharmaceutical Form(s):

Capsule, hard

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an age-appropriate capsule formulation.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	2	Study 2 (DMX-200-301) Double-blind, randomised, placebo-controlled trial to evaluate pharmacokinetics, efficacy and safety of repagermanium in paediatric patients from 12 years to less than 18 years of age (and adults) with focal segmental glomerulosclerosis. Study 3 (DMX-200-205) Open-label, single-arm trial to evaluate safety, tolerability, pharmacokinetics and activity of repagermanium in paediatric patients from 1 year to less than 12 years of age with focal segmental glomerulosclerosis.
Extrapolation, Modeling & Simulation Studies	3	Study 4 (DMX-200-301 PK modelling) Population PK modelling and simulation study to evaluate the use of repagermanium in paediatric patients from 1 year to less than 18 years of age with focal segmental glomerulosclerosis. Study 5 (DMX-200-301 PD modelling) Modelling and simulation study to extrapolate efficacy of repagermanium from adults and adolescents to paediatric patients from 1 year to less than 12 years of age with focal segmental glomerulosclerosis. Extrapolation

		plan Study 2 (DMX-200-301), Study 3, Study 4 (DMX-200-301 PK modelling) and Study 5 (DMX-200-301 PD modelling) are part of an extrapolation plan covering the paediatric population from 1 year to less than 12 years of age, as agreed by the Regulatory Agency.
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:	31/07/2031
Deferral of one or more studies contained in the paediatric investigation plan:	Yes