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

MHRA-100824-PIP01-22

Scope of the Application

Active Substance(s)

perflubutane

Condition(s)

Diagnostic evaluation of focal hepatic lesions

Pharmaceutical Form(s)

Powder and solvent for dispersion for injection

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

GE Healthcare AS

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, GE Healthcare AS submitted to the licensing authority on 17/01/2023 11:55 GMT an application for a Paediatric Investigation Plan

The procedure started on 05/06/2023 15:59 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

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-100824-PIP01-22

Of 16/06/2023 16:15 BST

On the adopted decision for perflubutane (MHRA-100824-PIP01-22) 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 perflubutane, Powder and solvent for dispersion for injection , INTRAVENOUS USE .

This decision is addressed to GE Healthcare AS, Nycoveien 1, Oslo, NORWAY, NO-0485

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Diagnostic evaluation of focal hepatic lesions

2.2 Indication(s) targeted by the PIP:

Use in vascular phase and post vascular (Kupffer) phase for ultrasound imaging of focal hepatic lesions

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

The paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Powder and solvent for dispersion 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 Open-label, single arm trial to evaluate the comparability of contrast enhanced ultrasound (CEUS) patterns after intravenous administration of perflubutane in paediatric patients undergoing abdominal echography with previously reported patterns in adults.
Extrapolation, Modeling & Simulation Studies	2	Study 2 Modelling and simulation study to support dose recommendation of perflubutane for diagnostic evaluation of focal hepatic lesions in children from birth to less than 2 years of age with focal hepatic lesions. Extrapolation Plan Studies 1 and 2 are part of an extrapolation plan covering the paediatric population from birth to less than 18 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:	30/11/2026
Deferral of one or more studies contained in the paediatric investigation plan:	Yes