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-100918-PIP01-23

Scope of the Application

Active Substance(s)

BELIMUMAB

Condition(s)

Treatment of systemic sclerosis

Pharmaceutical Form(s)

Solution for injection, Powder for concentrate for solution for infusion

Route(s) of Administration

SUBCUTANEOUS USE; INTRAVENOUS USE

Name / Corporate name of the PIP applicant

GlaxoSmithKline UK Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, GlaxoSmithKline UK Limited submitted to the licensing authority on 10/03/2023 13:03 GMT an application for a Paediatric Investigation Plan

The procedure started on 10/07/2023 07:49 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-100918-PIP01-23

Of 15/04/2024 07:42 BST

On the adopted decision for BELIMUMAB (MHRA-100918-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 BELIMUMAB, Solution for injection, Powder for concentrate for solution for infusion , SUBCUTANEOUS USE; INTRAVENOUS USE .

This decision is addressed to GlaxoSmithKline UK Limited, 980 Great West Road, Brentford, UNITED KINGDOM, TW8 9GS

ANNEX I

1. Waiver

1.1 Condition:

Treatment of systemic sclerosis The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 8 years of age Pharmaceutical form(s): Solution for injection Powder for concentrate for solution for infusion Route(s) of administration: SUBCUTANEOUS USE INTRAVENOUS 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 systemic sclerosis

2.2 Indication(s) targeted by the PIP:

Treatment of systemic sclerosis Treatment of interstitial lung disease associated with systemic sclerosis

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

The paediatric population from 8 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Solution for injection Powder for concentrate for solution for infusion

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 the pharmacokinetics (PK) and pharmacodynamics (PD) of belimumab solution for injection, subcutaneous use, in children from 8 years to less than 18 years of age with juvenile onset systemic sclerosis and to provide PK/PD data to support the extrapolation of efficacy from the adult population.
Extrapolation, Modeling & Simulation Studies	2	Study 2 Modelling and simulation analyses to predict initial paediatric doses to be used in the paediatric clinical study (PIP Study 1). Extrapolation Plan Studies 1 and 2 are part of an extrapolation plan covering the paediatric population from 8 years 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:	30/09/2031

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