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-102181-PIP01-25

Scope of the Application

Active Substance(s)

Tinlarebant

Condition(s)

Treatment of Stargardt disease

Pharmaceutical Form(s)

Tablet, Capsule, hard

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Voisin Consulting Life Sciences

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Voisin Consulting Life Sciences submitted to the licensing authority on 21/11/2025 11:18 GMT an application for a

The procedure started on 02/12/2025 14:09 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-102181-PIP01-25

Of 23/03/2026 08:16 GMT

On the adopted decision for Tinlarebant (MHRA-102181-PIP01-25) 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 for Tinlarebant, Tablet, Capsule, hard , ORAL USE .

This decision is addressed to Voisin Consulting Life Sciences, 64 Avenue Pierre Grenier, Boulogne Billancourt, FRANCE, 92100

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Stargardt disease The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 3 years of age Pharmaceutical form(s): Tablet Capsule, hard Route(s) of administration: ORAL 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 Stargardt disease

2.2 Indication(s) targeted by the PIP:

Treatment of autosomal recessive Stargardt Disease (STGD1)

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

The paediatric population from 3 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Tablet Capsule, hard

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 (LBS-008-CTDF) Development of an age-appropriate capsule (granule dissolution) formulation of tinlarebant for dosing of paediatric subjects from 3 years of age in study LBS-008-CT12 (PIP Study 4).
Non-Clinical Studies	0	Not applicable.
Clinical Studies	3	Study 2 (LBS-008-CT02) Open-label, dose-finding followed by 2-year extension study to evaluate safety and tolerability of tinlarebant in adolescents from 12 years to less than 18 years of age with Stargardt Disease. Study 3 (LBS-008-CT03) Multicentre, randomised, double-masked, placebo-controlled study to evaluate the safety and efficacy of tinlarebant in adolescents from 12 years to less than 18 years of age (and adults) with autosomal recessive Stargardt Disease (STGD1). Study 4 (LBS-008-CT12) Multicentre, randomised, double-masked, placebo-controlled, study to evaluate the safety, tolerability, and efficacy of tinlarebant in children from 3 years to less than 12 years of age with autosomal recessive Stargardt Disease (STGD1).
Extrapolation, Modeling & Simulation Studies	1	Study 5 (LBS-008-PedMS) Population PK/PD study to determine optimal dosage strength of

		tinlarebant for paediatric subjects in study LBS-008-CT12 (PIP study 4).
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:	30/06/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes