MHRA
10 South Colonnade
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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-101791-PIP01-25

Scope of the Application

Active Substance(s)

Bexicaserin hydrochloride

Condition(s)

Treatment of seizures and seizure disorders

Pharmaceutical Form(s)

Oral solution

Route(s) of Administration

ORAL USE, GASTROENTERAL USE

Name / Corporate name of the PIP applicant

Longboard Pharmaceuticals, Inc.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Longboard Pharmaceuticals, Inc. submitted to the licensing authority on 14/02/2025 15:14 GMT an application for a Paediatric Investigation Plan

The procedure started on 29/05/2025 17:08 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-101791-PIP01-25

Of 15/10/2025 08:16 BST

On the adopted decision for Bexicaserin (MHRA-101791-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 Paediatric Investigation Plan for Bexicaserin, Oral solution , GASTROENTERAL USE, ORAL USE .

This decision is addressed to Longboard Pharmaceuticals, Inc., 4275 Executive Square, Ste 950, La Jolla, UNITED STATES OF AMERICA, 92037

ANNEX I

1. Waiver

1.1 Condition:

Treatment of seizures and seizure disorders The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 1 year of age Pharmaceutical form(s): Oral solution Route(s) of administration: ORAL USE GASTROENTERAL 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 seizures and seizure disorders

2.2 Indication(s) targeted by the PIP:

The adjunctive treatment of seizures associated with developmental and epileptic encephalopathies (DEEs) for patients aged one year and older. The adjunctive treatment of seizures in children aged one year and older and adults with Dravet Syndrome.

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

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

2.4 Pharmaceutical Form(s):

Oral solution

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	1	Study 1 (LP-352-NC-0092) Definitive juvenile toxicity study in rats to support the evaluation of bexicaserin when used in children from 1 year of age.
Clinical Studies	5	Study 2 (LP352-201/PACIFIC) Randomised, double-blind, placebo-controlled, parallel group, dose-escalation study to evaluate the safety, tolerability, pharmacokinetics (PK), pharmacodynamics (PD), and efficacy of bexicaserin in the adjunctive treatment of seizures in children from 12 years to less than 18 years of age (and adults) with developmental and epileptic encephalopathies with continued seizure activity. Study 3 (LP352-202) Multicentre, open-label, long-term extension study to evaluate safety of bexicaserin in the adjunctive treatment of seizures in adolescents from 12 years to less than 18 years of age (and adults), with developmental and epileptic encephalopathies who completed Study 2. Study 4 (LP352-301) Randomised, double-blind, placebo-controlled, multicentre study to evaluate the efficacy, safety, and tolerability of bexicaserin

		in the adjunctive treatment of seizures in children from 1 year to less than 18 years of age (and adults) with developmental and epileptic encephalopathies with continued seizure activity. Study 5 (LP352-302) Randomised, double-blind, placebo-controlled, multicentre study designed to evaluate the efficacy, safety, and tolerability of bexicaserin in the adjunctive treatment of seizures in children 1 year to less than 18 years of age (and adults) with Dravet Syndrome with continued seizure activity. Study 6 (LP352-303) Open-label, long-term, study to evaluate the long-term safety and activity of bexicaserin in the adjunctive treatment of seizures in children from 1 year to less than 18 years of age (and adults) with developmental and epileptic encephalopathies with continued seizure activity and have completed either study 4 or 5 (LP352-301 or LP352-302).
Extrapolation, Modeling & Simulation Studies	2	Study 7 (FR-006) Modelling and simulation population pharmacokinetic study of bexicaserin to support dose selection and exposure response analysis in children from 2 years to less than 18 years of age (and adults) to evaluate the use of the product in the adjunctive treatment of seizures and seizure disorders in paediatric patients. Study 8 Modelling and simulation population pharmacokinetic study to support a dosing strategy for children from 1 year to less than 2 years of age in studies LP352-301 (Study 4), LP352-302 (Study 5) and LP352-303 (Study 6) and to confirm or modify the paediatric posology compared to the regimen used in clinical trials.
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:	30/06/2028
Deferral of one or more studies contained in the paediatric investigation plan:	Yes