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-100027-PIP01-21

Scope of the Application

Active Substance(s)

benzylamine derivative of benzofuran

Condition(s)

Treatment of paroxysmal nocturnal haemoglobinuria (PNH)

Pharmaceutical Form(s)

Age appropriate oral solid dosage forms; Tablet

Route(s) of Administration

Oral use

Name / Corporate name of the PIP applicant

BioCryst Ireland, Ltd.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, BioCryst Ireland, Ltd. submitted to the licensing authority on 22/03/2021 19:38 GMT an application for a Paediatric Investigation Plan

The procedure started on 09/06/2021 08:46 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-100027-PIP01-21

Of 04/05/2022 15:50 BST

On the adopted decision for benzylamine derivative of benzofuran (MHRA-100027-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 benzylamine derivative of benzofuran, Age appropriate oral solid dosage forms; Tablet , Oral use .

This decision is addressed to BioCryst Ireland, Ltd., Block 4, Harcourt Centre, Harcourt Road, Dublin 2, Dublin, United Kingdom, D02HW77

ANNEX I

1. Waiver

1.1 Condition:

Treatment of paroxysmal nocturnal haemoglobinuria (PNH) The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Tablet; Age appropriate oral solid dosage form 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 paroxysmal nocturnal haemoglobinuria (PNH)

2.2 Indication(s) targeted by the PIP:

Treatment of paroxysmal nocturnal haemoglobinuria (PNH)

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

The paediatric population from 2 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Tablet; Age appropriate oral solid dosage form

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an age appropriate oral solid dosage form.
Non-Clinical Studies	0	Not applicable
Clinical Studies	1	Study 2 (BCX9930-301) Open label single arm study to evaluate pharmacokinetics, pharmacodynamics, safety and activity of BCX9930 in children from 2 years to less than 18 years of age for the treatment of paroxysmal nocturnal haemoglobinuria (PNH).
Extrapolation, Modeling & Simulation Studies	2	Study 3 (BCX9930-PPK1) Modelling and simulations study to derive dosing of BCX9930 in children from 2 years to less than 18 years of age for the treatment of PNH. Study 4 (BCX9930-EXP1) Extrapolation study to support the dose selection of BCX9930 in children from 2 years to less than 18 years of age for the treatment of PNH.
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:	Yes
Date of completion of the paediatric investigation plan:	31/03/2027
Deferral of one or more studies contained in the paediatric investigation plan:	Yes