

EMA/834865/2022

European Medicines Agency decision P/0449/2022

of 28 October 2022

on the agreement of a paediatric investigation plan and on the granting of a waiver for branaplam (EMA-002204-PIP02-20) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

European Medicines Agency decision

P/0449/2022

of 28 October 2022

on the agreement of a paediatric investigation plan and on the granting of a waiver for branaplam (EMA-002204-PIP02-20) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004¹,

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency²,

Having regard to the application submitted by Novartis Europharm Limited on 23 November 2020 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 9 September 2022, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 13 of said Regulation,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

- (1) The Paediatric Committee of the European Medicines Agency has given an opinion on the agreement of a paediatric investigation plan and on the granting of a waiver.
- (2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.
- (3) It is therefore appropriate to adopt a decision granting a waiver.

¹ OJ L 378, 27.12.2006, p.1, as amended.

² OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for branaplam, oral solution, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A waiver for branaplam, oral solution, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This decision is addressed to Novartis Europharm Limited Vista Building, Elm Park, Merrion Road 4 – Dublin, Ireland.

EMA/PDCO/576954/2022
Amsterdam, 9 September 2022

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a waiver

EMA-002204-PIP02-20

Scope of the application

Active substance(s):

Branaplam

Condition(s):

Treatment of Huntington's Disease

Pharmaceutical form(s):

Oral solution

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Novartis Europharm Limited

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Novartis Europharm Limited submitted for agreement to the European Medicines Agency on 23 November 2020 an application for a paediatric investigation plan for the above mentioned medicinal product under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 4 January 2021.

Supplementary information was provided by the applicant on 24 May 2022. The applicant proposed modifications to the paediatric investigation plan.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:
 - to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
 - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annexes and appendix.

Annex I

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

1.1. Condition:

Treatment of Huntington's disease

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- oral solution, oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

2. Paediatric investigation plan

2.1. Condition:

Treatment of Huntington's disease

2.1.1. Indication(s) targeted by the PIP

Treatment of juvenile Huntington's disease

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 2 years to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Oral solution

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Development of an age-appropriate oral solution
Non-clinical studies	Not applicable
Clinical studies	Study 2 Two-part double blind, controlled versus placebo study of pharmacokinetics, efficacy and safety of branaplam in paediatric patients from 12 years to less than 18 years of age (and young adults from 18 to less than 24 years of age) with juvenile Huntington's disease (HD) (CLMI070C12301). Study 3 Two-part, single arm study of pharmacokinetic, pharmacodynamic activity and safety of branaplam in paediatric patients from 2 years

	to less than 12 years of age with juvenile Huntington's disease (HD) (CLMI070C12204).
Extrapolation, modelling and simulation studies	<p>Study 4</p> <p>PBPK modelling and simulation study of branaplam to recommend a branaplam dose for patients with juvenile Huntington's Disease in studies 2 and 3 (CLMI070C12301 and CLMI070C12204).</p> <p>Study 5</p> <p>Pop PK/PD modelling and simulation study of branaplam to identify and confirm a branaplam dose for patients with juvenile Huntington's Disease patients in studies 2 and 3 (CLMI070C12301 and CLMI070C12204) and to support extrapolation of efficacy linking branaplam exposure to total huntingtin (tHTT) / mutant huntingtin (mHTT) levels.</p>
Other studies	Not applicable
Other measures	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By September 2028
Deferral for one or more measures contained in the paediatric investigation plan:	No