EMA/255061/2024

# European Medicines Agency decision

P/0215/2024

of 13 June 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for human alpha-1 proteinase inhibitor, modified (SerpinPC) (EMEA-003463-PIP01-23), 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/0215/2024

of 13 June 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for human alpha-1 proteinase inhibitor, modified (SerpinPC) (EMEA-003463-PIP01-23), 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 726/2004[1],

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 Agency[2],

Having regard to the application submitted by ApcinteX Ltd on 31 May 2023 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 26 April 2024, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation 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 deferral 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 deferral.

(4) 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 human alpha-1 proteinase inhibitor, modified (SerpinPC), powder and solvent for solution for injection, subcutaneous 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 deferral for human alpha-1 proteinase inhibitor, modified (SerpinPC), powder and solvent for solution for injection, subcutaneous 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***

A waiver for human alpha-1 proteinase inhibitor, modified (SerpinPC), powder and solvent for solution for injection, subcutaneous 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 4***

This decision is addressed to ApcinteX Ltd, 3rd floor, 1 Ashley Road, Altrincham, WA14 2DT – Altrincham, United Kingdom.

EMA/PDCO/55018/2024 Corr[1]
Amsterdam, 26 April 2024

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

EMEA-003463-PIP01-23

## Scope of the application

**Active substance(s):**

Human alpha-1 proteinase inhibitor, modified (SerpinPC)

**Invented name and authorisation status:**

See Annex II

**Condition(s):**

Treatment of haemophilia B

**Pharmaceutical form(s):**

Powder and solvent for solution for injection

**Route(s) of administration:**

Subcutaneous use

**Name/corporate name of the PIP applicant:**

ApcinteX Ltd

## Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, ApcinteX Ltd submitted for agreement to the European Medicines Agency on 31 May 2023 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 14 August 2023.

[1] 14 May 2024

Supplementary information was provided by the applicant on 22 January. 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 deferral in accordance with Article 21 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)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population.

   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 haemophilia B

The waiver applies to:

- the paediatric population from birth to less than 6 months of age;
- powder and solvent for solution for injection, subcutaneous use;
- on the grounds that the specific medicinal product is likely to be ineffective.

# 2. Paediatric investigation plan

## *2.1. Condition:*

Treatment of haemophilia B

### 2.1.1. Indication(s) targeted by the PIP

Prophylaxis to prevent or reduce the frequency of bleeding episodes in children ≥ 2 years of age with severe haemophilia B (congenital factor IX deficiency) with or without factor IX inhibitors

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

From 6 months to less than 18 years of age

### 2.1.3. Pharmaceutical form(s)

Powder and solvent for solution for injection

### 2.1.4. Measures

| **Area** | **Description** |
|---|---|
| Quality-related studies | Not applicable |
| Non-clinical studies | Not applicable |
| Clinical studies | **Study 1 (AP-0102)**<br>Open-label, adaptive design trial to evaluate the activity and safety of SerpinPC in children aged 12 years to less than 18 years (and adults) with severe haemophilia A (with or without inhibitors) or moderately severe to severe haemophilia B (without inhibitors)<br>**Study 2 (AP-0103)**<br>Open-label trial to evaluate the activity and safety of SerpinPC in children aged 12 years to less than 18 years of age (and adults) with haemophilia B with inhibitors |

| | |
|---|---|
| | **Study 3 (AP-0106)**<br><br>Open-label extension study to investigate the long-term activity and safety of SerpinPC in subjects who completed Studies AP-0102, AP-0103 or AP-0107.<br><br>**Study 4 (AP-0107)**<br><br>Open-label single arm trial to evaluate the activity and safety of SerpinPC in children from 6 months to less than 12 years of age with severe haemophilia A (with or without inhibitors) or moderately severe to severe haemophilia B (with or without inhibitors) |
| Modelling and simulation analyses | **Study 5 (Not available)**<br><br>Population pharmacokinetic (PopPK) model to be used for paediatric exposure matching and sample design optimisation<br><br>**Study 6 (Not available)**<br><br>Population pharmacokinetic and pharmacodynamic (PopPKPD) model to be used to assess the similarity in pharmacodynamics between adolescent and adult patients |
| Other studies | Not applicable |
| Extrapolation plan | 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 June 2034 |
| Deferral for one or more measures contained in the paediatric investigation plan: | Yes |

# Annex II

# Information about the authorised medicinal product

***Information provided by the applicant:***

**The product is not authorised anywhere in the European Community.**