EMA/33649/2021

# European Medicines Agency decision

P/0017/2021

of 29 January 2021

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for verinurad / allopurinol (EMEA-002754-PIP01-19) 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.**

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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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the application submitted by AstraZeneca AB on 24 January 2020 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 11 December 2020, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

***Article 1***

A paediatric investigation plan for verinurad / allopurinol, capsule, hard, age appropriate oral formulation, 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 deferral for verinurad / allopurinol, capsule, hard, age appropriate oral formulation, 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***

A waiver for verinurad / allopurinol, capsule, hard, age appropriate oral formulation, 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 4***

This decision is addressed to AstraZeneca AB, SE-151 85 - Södertälje, Sweden.

EMA/PDCO/527714/2020
Amsterdam, 11 December 2020

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

EMEA-002754-PIP01-19

## Scope of the application

**Active substance(s):**

Verinurad / allopurinol

**Condition(s):**

Treatment of chronic kidney disease

**Pharmaceutical form(s):**

Capsule, hard

Age appropriate oral formulation

**Route(s) of administration:**

Oral use

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

AstraZeneca AB

## Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, AstraZeneca AB submitted for agreement to the European Medicines Agency on 24 January 2020 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 2 March 2020.

Supplementary information was provided by the applicant on 9 September 2020.

## 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 Norwegian Paediatric Committee member 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 annex 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 chronic kidney disease

The waiver applies to:

- the paediatric population from birth to less than 2 years;
- capsule, hard, age-appropriate oral formulation, oral use;
- on the grounds that the specific medicinal product is likely to be unsafe.

# 2. Paediatric investigation plan

## *2.1. Condition:*

Treatment of chronic kidney disease

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

Treatment of chronic kidney disease with hyperuricaemia and albuminuria

### 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)

Capsule, hard

Age appropriate oral formulation

### 2.1.4. Measures

| **Area** | **Number of measures** | **Description** |
|---|---|---|
| Quality-related studies | 1 | **Study 1**<br>Development of age-appropriate oral formulation suitable for children 2 to less than 12 years of age |
| Non-clinical studies | 0 | Not applicable |
| Clinical studies | 1 | **Study 2**<br>12-month, randomised, double-blind, placebo-controlled, pharmacokinetic (PK), efficacy and safety study in children and adolescents 2 years to less than 18 years with chronic kidney disease (CKD) stages 1 to 3, albuminuria and hyperuricaemia. |

| Area | Number of measures | Description |
|---|---|---|
| Extrapolation, modelling and simulation studies | 2 | **Study 3**<br>Population PK/PD model to predict verinurad and allopurinol exposures and reduction of serum UA and UACR in paediatric subjects.<br>**Study 4**<br>Extrapolation study to potentially extrapolate the UACR and eGFR slope data of verinurad from adults to children and adolescents (2 years to less than 18 years old) with CKD and hyperuricaemia. |
| Other studies | 0 | Not applicable. |
| Other measures | 0 | Not applicable. |

# 3. Follow-up, completion and deferral of PIP

| | |
|---|---|
| Concerns on potential long term safety/efficacy issues in relation to paediatric use: | Yes |
| Date of completion of the paediatric investigation plan: | By June 2030 |
| Deferral for one or more measures contained in the paediatric investigation plan: | Yes |