EMADOC-1700519818-2023410

# European Medicines Agency decision

EMEA-003489-PIP02-23

of 16 April 2025

on the agreement of a paediatric investigation plan and on the granting of a deferral for sonrotoclax 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

EMEA-003489-PIP02-23

of 16 April 2025

on the agreement of a paediatric investigation plan and on the granting of a deferral for sonrotoclax 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 BeiGene Ireland Limited on 11 September 2023 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 28 February 2025, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 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.

(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.

[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 sonrotoclax, 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 sonrotoclax, 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 BeiGene Ireland Limited, 10 Earlsfort Terrace, Dublin 2 D02 T380 - Co. Dublin, Ireland.

EMADOC-1700519818-1805664
Amsterdam, 28 February 2025

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

EMEA-003489-PIP02-23

## Scope of the application

**Active substance(s):**

Sonrotoclax

**Invented name and authorisation status:**

See Annex II

**Condition(s):**

Treatment of neuroblastoma

**Pharmaceutical form(s):**

Tablet

Age-appropriate dosage form

**Route(s) of administration:**

Oral use

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

BeiGene Ireland Limited

## Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, BeiGene Ireland Limited submitted for agreement to the European Medicines Agency on 11 September 2023 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 16 October 2023.

Supplementary information was provided by the applicant on 26 November 2024. 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.

   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 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

Not applicable

# 2. Paediatric investigation plan

## *2.1. Condition:*

Treatment of neuroblastoma

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

Treatment of relapsed/ refactory neuroblastoma

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

From birth to less than 18 years of age

### 2.1.3. Pharmaceutical form(s)

Tablet

Age-appropriate dosage form

### 2.1.4. Measures

| **Area** | **Description** |
|---|---|
| Quality-related studies | **Study 1 (same as in EMEA-003489-PIP01-23)**<br><br>Development of an age-appropriate formulation of sonrotoclax |
| Non-clinical studies | **Study 2 (same as in EMEA-003489-PIP01-23)**<br><br>Proof of concept study in *in vivo* (acute lymphoblastic leukaemia [ALL], acute myeloid leukaemia [AML], neuroblastoma [NB]) models to evaluate anti tumour activity of sonrotoclax combination with other active substances for which there is biological rational and no relevant existing clinical evidence |
| Clinical studies | **Study 3 (same as in EMEA-003489-PIP01-23)**<br><br>Open-label, single arm, two part (part 1: dose finding and part 2: expansion) study to evaluate pharmacokinetics, safety and activity, of sonrotoclax alone and in combination with azacitidine in relapsed/refractory AML (cohort 1), alone and in combination with dexamethasone in patients with relapsed/refractory ALL (cohort 2) and alone and in combination with topotecan in relapsed/refractory neuroblastoma (cohort 3), and in combination with potential other combinations beyond azacitidine (for AML) |

| | |
|---|---|
| | and steroids (for ALL) based on existing clinical data or data from PIP study 2 (cohort 4), in children from birth to less than 18 years of age.<br><br>**Study 4 (same as in EMEA-003489-PIP01-23)**<br><br>Open-label, randomised controlled trial to evaluate safety and efficacy of sonrotoclax in combination compared to standard of care in children with a condition identified based on results from PIP study 3.<br><br>Further key elements to be agreed with the PDCO to initiation of the study. |
| Modelling and simulation analyses | **Study 5 (same as study 5 in EMEA-003489-PIP01-23)**<br><br>Modelling and simulation analyses to evaluate and confirm the paediatric dose in children. |
| 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: | Yes |
| Date of completion of the paediatric investigation plan: | By December 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.**