EMA/898536/2022

# European Medicines Agency decision

P/0510/2022

of 2 December 2022

on the agreement of a paediatric investigation plan and on the granting of a waiver for emactuzumab (EMEA-003172-PIP01-21) 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 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 SynOx Therapeutics Limited on 20 December 2021 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 14 October 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 emactuzumab, concentrate for solution for infusion, intravenous 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 emactuzumab, concentrate for solution for infusion, intravenous 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 Synox Therapeutics Limited, 25-28 North Wall Quay, D01 H104 - Dublin 1, Dublin, Ireland.

EMA/PDCO/643519/2022
Amsterdam, 14 October 2022

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

EMEA-003172-PIP01-21

## Scope of the application

**Active substance(s):**

Emactuzumab

**Invented name and authorisation status:**

See Annex II

**Condition(s):**

Treatment of tenosynovial giant cell tumour, local and diffuse type.

**Pharmaceutical form(s):**

Concentrate for solution for infusion

**Route(s) of administration:**

Intravenous use

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

SynOx Therapeutics Limited

## Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, SynOx Therapeutics Limited submitted for agreement to the European Medicines Agency on 20 December 2021 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 31 January 2022.

Supplementary information was provided by the applicant on 4 July 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)(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 tenosynovial giant cell tumour, local and diffuse type.

The waiver applies to:

- the paediatric population from birth to less than 12 years of age;
- concentrate for solution for infusion, intravenous use;
- on the grounds that the specific medicinal product is likely to be unsafe.

# 2. Paediatric investigation plan

## *2.1. Condition:*

Treatment of tenosynovial giant cell tumour, local and diffuse type.

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

Treatment of tenosynovial giant cell tumour in patients for whom surgical resection would be associated with potentially worsening functional limitations, high risk of early recurrence, or any other morbidity associated with surgery.

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

From 12 years to less than 18 years of age.

### 2.1.3. Pharmaceutical form(s)

Concentrate for solution for infusion

### 2.1.4. Measures

| **Area** | **Description** |
|---|---|
| Quality-related studies | Not applicable |
| Non-clinical studies | Not applicable |
| Clinical studies | **Study 1 (SNX-301-020)**<br><br>Open-label multicentre study to assess the safety, pharmacokinetics (PK) and antitumour activity of emactuzumab and to provide PK/pharmacodynamic (PD) data to support the extrapolation of efficacy from adults to adolescents from 12 years to less than 18 years of age with tenosynovial giant cell tumour as part of the randomised, double-blind study aimed to assess the safety and efficacy of emactuzumab compared to placebo in adults with tenosynovial giant cell tumour. |

| | |
|---|---|
| Modelling and simulation studies | **Study 2**<br><br>Modelling and simulation study, to evaluate the use of emactuzumab in adolescents from 12 years to less than 18 years of age with tenosynovial giant cell tumour and to support the extrapolation of efficacy from adults with tenosynovial giant cell tumour. |
| Other studies | Not applicable |
| Extrapolation plan | Study 1 (SNX-301-020) and study 2 are part of the extrapolation plan of efficacy data from adults to adolescents from 12 years to less than 18 years of age with tenosynovial giant cell tumour. |

# 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 October 2024 |
| Deferral for one or more measures contained in the paediatric investigation plan: | No |

# Annex II

# Information about the authorised medicinal product

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

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