EMA/220354/2023

European Medicines Agency decision
P/0198/2023

of 22 May 2023

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for humanised VHH-type bispecific antibody against complement component 5 and serum albumin (ALXN1720), (EMEA-003302-PIP01-22) 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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granting of a waiver for humanised VHH-type bispecific antibody against complement component 5
and serum albumin (ALXN1720), (EMEA-003302-PIP01-22) in accordance with Regulation (EC)
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)
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 Alexion Europe SAS on 8 August 2022 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 2023, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said
Regulation and Article 13 of said Regulation,

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.

Has adopted this decision:

**Article 1**

A paediatric investigation plan for humanised VHH-type bispecific antibody against complement component 5 and serum albumin (ALXN1720), 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 humanised VHH-type bispecific antibody against complement component 5 and serum albumin (ALXN1720), 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 humanised VHH-type bispecific antibody against complement component 5 and serum albumin (ALXN1720), 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 Alexion Europe SAS, 103-105 rue Anatole France, 92300 - Levallois-Perret, France.
Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and a waiver
EMEA-003302-PIP01-22

Scope of the application

Active substance(s):
Humanised VHH-type bispecific antibody against complement component 5 and serum albumin (ALXN1720)

Invented name and authorisation status:
See Annex II

Condition(s):
Treatment of myasthenia gravis

Pharmaceutical form(s):
Solution for injection

Route(s) of administration:
Subcutaneous use

Name/corporate name of the PIP applicant:
Alexion Europe SAS

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Alexion Europe SAS submitted for agreement to the European Medicines Agency on 8 August 2022 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 12 September 2022.

Supplementary information was provided by the applicant on 23 January 2023. 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)(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 myasthenia gravis

The waiver applies to:

- the paediatric population from birth to less than 6 years of age;
- solution for injection, subcutaneous use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. **Condition:**

Treatment of myasthenia gravis

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

Treatment of acetylcholine receptor-antibody positive generalised myasthenia gravis

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

From 6 years to less than 18 years of age

2.1.3. **Pharmaceutical form(s)**

Solution for injection

2.1.4. **Measures**

<table>
<thead>
<tr>
<th>Area</th>
<th>Description</th>
</tr>
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<tbody>
<tr>
<td>Quality-related studies</td>
<td>Not applicable</td>
</tr>
<tr>
<td>Non-clinical studies</td>
<td>Not applicable</td>
</tr>
</tbody>
</table>
| Clinical studies                  | **Study 1**
|                                   | Open-label, single-arm study to evaluate pharmacokinetics (PK), pharmacodynamics (PD), safety and activity of ALXN1720 in paediatric patients from 6 years to less than 18 years of age with generalised myasthenia gravis (gMG) who express acetylcholine receptor-antibodies (AChR+) (ALXN1720-MG-30X) |
| Modelling and simulation studies  | **Study 2**
<p>|                                   | Modelling and simulation study using population target-mediated drug disposition (TMDD) to determine (adult) and paediatric doses. |</p>
<table>
<thead>
<tr>
<th>Other studies</th>
<th>Not applicable</th>
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<tr>
<td>Extrapolation plan</td>
<td>Studies 1 and 2 are part of an extrapolation plan covering the paediatric population from 6 years to less than 18 years of age, as agreed by the PDCO.</td>
</tr>
</tbody>
</table>

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 2030 |
| 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.