EMA/508734/2014

European Medicines Agency decision

P/0246/2014

of 29 September 2014

on the agreement of a paediatric investigation plan and on the granting of a deferral for nanobody directed towards the fusion protein of human respiratory syncytial virus (ALX-0171) (EMEA-001553-PIP01-13) 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 Ablynx NV on 4 November 2013 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 15 August 2014, in accordance with Article 18 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for nanobody directed towards the fusion protein of human respiratory syncytial virus (ALX-0171), nebuliser solution, inhalation 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 nanobody directed towards the fusion protein of human respiratory syncytial virus (ALX-0171), nebuliser solution, inhalation 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 Ablynx NV, Technologiepark 21, 9052 - Zwijnaarde, Belgium.

Done at London, 29 September 2014

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)

EMA/PDCO/335111/2014

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

EMEA-001553-PIP01-13

Scope of the application

Active substance(s):

Nanobody directed towards the fusion protein of human respiratory syncytial virus (ALX-0171)

Condition(s):

Treatment of lower respiratory tract disease caused by human respiratory syncytial virus (RSV)

Pharmaceutical form(s):

Nebuliser solution

Route(s) of administration:

Inhalation use

Name/corporate name of the PIP applicant:

Ablynx NV

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Ablynx NV submitted for agreement to the European Medicines Agency on 4 November 2013 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 18 December 2013.

Supplementary information was provided by the applicant on 26 May 2014. The applicant proposed modifications to the paediatric investigation plan and requested a deferral and withdrew its request for a waiver.

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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 18 of said Regulation;
   - to grant a deferral in accordance with Article 21 of said Regulation.

   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 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.

London, 15 August 2014

On behalf of the Paediatric Committee
Dr Dirk Mentzer, Chairman
(Signature on file)

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 lower respiratory tract disease caused by human respiratory syncytial virus (RSV)

2.1.1. Indication(s) targeted by the PIP

Treatment of lower respiratory tract disease caused by human respiratory syncytial virus (RSV)

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)

Nebuliser solution

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1** Development of product- and age-specific nebuliser devices which take into consideration paediatric populations requiring or not (non)-invasive ventilation as well as immunocompromised subjects.
Non-clinical studies	2	**Study 2** PK study in juvenile animals. **Study 3** In vivo efficacy testing in a neonatal lamb model.
Clinical studies	9	**Study 4** Randomised, double-blind, placebo-controlled safety, tolerability and PK study in infants and toddlers hospitalised for RSV lower respiratory tract infection, aged 5 months to less than 24 months. **Study 5** Randomised, double-blind, placebo-controlled safety, tolerability and PK study in infants hospitalised for RSV lower respiratory tract infection, aged 28 days to less than 5 months.

		Study 6 Randomised, double-blind, placebo-controlled efficacy, safety and PK study in infants and children hospitalised for RSV lower respiratory tract infection, including high-risk subjects, aged 28 days to less than 3 years. **Study 7** Randomised, double-blind, placebo-controlled efficacy, safety and PK study in infants and children hospitalised for RSV lower respiratory tract infection and requiring invasive mechanical ventilation, including high-risk subjects, aged 28 days to less than 3 years. **Study 8** 2-year long-term safety follow-up study in subjects who completed studies 6 and 7. **Study 9** Randomised, double-blind, placebo-controlled efficacy and safety study in infants and children hospitalised for RSV lower respiratory tract infection, including high-risk subjects, aged 28 days to less than 3 years. **Study 10** Randomised, double-blind, placebo-controlled efficacy and safety study in infants and children at risk of hospitalisation for RSV lower respiratory tract infection, including high-risk subjects, aged 28 days to less than 3 years. **Study 11** Randomised, double-blind, placebo-controlled efficacy and safety study in preterm and term newborn infants aged less than 28days and infants with a history of prematurity, but currently aged 28 days to less than 3 months. **Study 12** 2-year long-term safety follow-up study in subjects who completed studies 9, 10, and 11.
Extrapolation, modelling and simulation studies	2	**Study 13** Modelling and simulation physiologically-based pharmacokinetic study for dose determination in all planned clinical studies. **Study 14** Extrapolation study to provide guidance on appropriate dosing regimens for immunocompromised subjects aged 0 to less than 18 years.
Other studies		Not applicable.
Other measures		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 September 2021
Deferral for one or more measures contained in the paediatric investigation plan:	Yes