



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/530837/2023

European Medicines Agency decision P/0487/2023

of 30 November 2023

on the agreement of a paediatric investigation plan and on the granting of a deferral for govorestat (EMEA-003365-PIP02-23) 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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on the agreement of a paediatric investigation plan and on the granting of a deferral for govorestat (EMA-003365-PIP02-23) 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 726/2004¹,

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 Agency²,

Having regard to the application submitted by Applied Therapeutics, Inc on 7 August 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 10 November 2023, 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 govorestat, oral suspension, 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 govorestat, oral suspension, 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

This decision is addressed to Applied Therapeutics, Inc, 545 Fifth Avenue, 14th Floor, 10017 - New York, United States.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/476435/2023
Amsterdam, 10 November 2023

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

EMA-003365-PIP02-23

Scope of the application

Active substance(s):

Govorestat

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of galactosaemia

Pharmaceutical form(s):

Oral suspension

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Applied Therapeutics, Inc

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Applied Therapeutics, Inc submitted for agreement to the European Medicines Agency on 7 August 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 11 September 2023.



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 galactosaemia

2.1.1. Indication(s) targeted by the PIP

Treatment of classic galactosaemia

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)

Oral suspension

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Development of an age-appropriate oral liquid formulation
Non-clinical studies	Study 2 (576566) Definitive juvenile toxicity rat study Study 3 (identifier not yet available) Juvenile kidney toxicity rat study
Clinical studies	Study 4 (AT-007-1002) Double-blind, randomised, multiple dose, placebo-controlled trial to evaluate pharmacokinetics, safety, efficacy of govorestat as add-on to standard of care in children from 2 years to less than 18 years of age with classic galactosaemia. Study 5 (AT-007-1010) Double-blind, randomised, multiple dose, placebo-controlled trial to evaluate pharmacokinetics, safety, efficacy of govorestat as add-on to standard of care in children from birth to less than 2 years of age with classic galactosaemia.
Modelling and simulation studies	Study 6 (AT-007-1015) Modelling and simulation study to support dose determination in

	children from birth to less than 2 years of age with classic galactosaemia.
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:	No
Date of completion of the paediatric investigation plan:	By June 2028
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.