EMADOC-1700519818-3379396

European Medicines Agency decision

EMA/PE/0000338563

of 14 August 2026

on the acceptance of a modification of an agreed paediatric investigation plan for omaveloxolone (Skyclarys) 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

EMA/PE/0000338563

of 14 August 2026

on the acceptance of a modification of an agreed paediatric investigation plan for omaveloxolone (Skyclarys) 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 European Medicines Agency's decision P/0204/2022 issued on 10 June 2022, the decision EMA/PE/0000246210 issued on 8 August 2025 and the decision EMA/PE/0000302591 issued on 20 March 2026,

Having regard to the application submitted by Biogen Netherlands B.V. on 23 March 2026 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 26 June 2026, in accordance with Article 22 of Regulation (EC) No 1901/2006,

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 acceptance of changes to the agreed paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[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

Changes to the agreed paediatric investigation plan for omaveloxolone (Skyclarys), are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

.

Article 2

This decision is addressed to Biogen Netherlands B.V., Prins Mauritslaan 13, 1171 LP Badhoevedorp, Netherlands.

EMADOC-1700519818-3046208

Amsterdam, 26 June 2026

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMA/PE/0000338563

Scope of the application

Active substance(s):

Omaveloxolone

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of Friedreich's ataxia

Pharmaceutical form(s):

Capsule, hard

Age appropriate oral capsule formulation

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Biogen Netherlands B.V.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Biogen Netherlands B.V. submitted to the European Medicines Agency on 23 March 2026 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0204/2022 issued on 10 June 2022, the decision EMA/PE/0000246210 issued on 8 August 2025 and the decision EMA/PE/0000302591 issued on 20 March 2026.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 28 April 2026.

Scope of the modification

Some measures of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

   The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the 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 Friedreich's ataxia

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- capsule, hard, age appropriate oral capsule formulation, oral 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 Friedreich's ataxia

2.1.1. Indication(s) targeted by the PIP

Treatment of Friedreich’s ataxia

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

From 2 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Capsule, hard

Age appropriate oral formulation

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1 (PED-FORM-DEV-1)** Development of an age appropriate oral capsule formulation
Non-clinical studies	Not applicable
Clinical studies	**Study 2 (408-C-1402 [Part 1])** Double-blind, randomised, placebo-controlled, dose ranging trial to evaluate pharmacokinetics, pharmacodynamics, safety and efficacy of omaveloxolone in adolescents from 16 years to less than 18 years of age (and adults) with genetically confirmed Friedreich's ataxia

	Study 3 (408-C-1402 [Part 2]) Double-blind, randomised, placebo controlled trial to evaluate safety and efficacy of omaveloxolone in adolescents from 16 years to less than 18 years of age (and adults) with genetically confirmed Friedreich's ataxia **Study 4 (408-C-2001)** Open-label, single arm trial to identify the appropriate dose and evaluate pharmacokinetics, pharmacodynamics, safety activity and acceptability/palatability of omaveloxolone in children from 2 years to less than 16 years of age with genetically confirmed Friedreich's ataxia **Study 6 (296FA301) (study added in procedure EMA/PE/0000246210)** Randomized, double-blind, placebo-controlled study to evaluate the efficacy, safety, PK, and PD of omaveloxolone in children from 2 years to less than 16 years of age with genetically confirmed Friedreich's ataxia, with a long term extension
Extrapolation, modelling and simulation studies	**Study 5** Modelling and simulation study to support the use of omaveloxolone in children from 2 years to less than 16 years of age with genetically confirmed Friedreich's ataxia
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 July 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:

Condition(s) and authorised indication(s)

1. Treatment of Friedreich's ataxia

Authorised indication(s):

- Skyclarys is indicated for the treatment of Friedreich's ataxia in adults and adolescents aged 16 years and older.
  - Invented name(s): Skyclarys
  - Authorised pharmaceutical form(s): Hard capsule
  - Authorised route(s) of administration: Oral use
  - Authorised via centralised procedure