



# Transparency measures

**EMA/EC multi-stakeholder  
workshop**

**Paediatric Regulation**

**London, 20 March 2018**

# Information available- PIP decisions

- Human medicines
  - European public assessment reports
  - Patient safety
  - Pending EC decisions
  - Withdrawn applications
  - ▶ Paediatrics
  - Rare disease designations
  - Medicines under evaluation
  - Medicines for use outside the EU
  - Referrals
  - Periodic safety update report single assessments
  - Post-authorisation safety studies
  - Shortages catalogue
  - Recommendations on medication errors
- Veterinary medicines
- Herbal medicines for human use

## Opinions and decisions on paediatric investigation plans

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**This page allows you to find information on opinions and decisions adopted by the European Medicines Agency's (EMA) Paediatric Committee (PDCO) on Paediatric Investigation Plans (PIPs) including deferrals and waivers.**

A PIP is a medicine development plan aimed at ensuring the necessary data are obtained through studies in children to support the medicine's authorisation for use in children. The plan is submitted by a pharmaceutical company to the PDCO, which is responsible for agreeing or refusing the plan and for publishing an opinion with its decision.

### Decision types

**P:** decision agreeing on a paediatric investigation plan, with or without partial waiver(s) or deferral(s)

**W:** decision granting a waiver in all age groups for the listed condition(s)

**PM:** decision on the application for modification of an agreed paediatric investigation plan

**RP:** decision refers to a refusal on a proposed paediatric investigation plan

**RW:** decision refers to a refusal on a request for waiver in all age groups for the listed condition(s)

**RPM:** decision refers to a refusal on the application for modification of an agreed paediatric investigation plan

### Decisions replaced by the latest modification of an agreed PIP

When the latest modification of an agreed PIP is published, any previous decisions will no longer be displayed on the decision web page accessed through the below search. However, these decisions remain published and can be found by searching the [document library](#) or using the site-wide search bar featured at the top right of every page. If you cannot find the document you are looking for, you can make a formal request for [access to documents](#) using the [online enquiry form](#).

[Browse A-Z](#)

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[Browse by therapeutic area](#)

Browse for Paediatric investigation plans by first letter of active substance:






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# Information available - Community register

European Commission > Public health > Register > Human centr > Strimvelis


## PHARMACEUTICALS - COMMUNITY REGISTER

Search

Print version     

Community register of medicinal products for human use

**Product information**

**Strimvelis** 


Auth. number : **EU/1/16/1097**

Active substance : **autologous CD34+ enriched cell fraction that contains CD34+ cells transduced with retroviral vector that encodes for the human ADA cDNA sequence**

Orphan market exclusivity for "Treatment of severe combined immunodeficiency (SCID) due to adenosine deaminase (ADA) deficiency" (based on designation EU/3/05/313) started on 30/05/2016  
 10 years of market exclusivity  
 2 additional years of market exclusivity as paediatric reward granted on 30/05/2016  
 This orphan market exclusivity will expire on 30/05/2028


Indication: **Strimvelis is indicated for the treatment of patients with severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID), for whom no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available.**

Marketing Authorisation Holder: **GlaxoSmithKline Trading Services Limited  
 Currabinny, Carrigaline, County Cork, Ireland**

EPAR and active package presentations 

**Package presentations**

Information about presentations can be found in the website of the [European Medicines Agency](#) under the section "Product Information". Likewise, presentations on which there has been a Commission decision are referred in the Summary of Product Characteristics (Annex I to the Commission Decision granting the marketing authorisation) which is available in the Community Register.

**European Commission procedures **

Close date procedure	Procedure type	EMA number	Decision	summary publ	decision docs	annex
30/05/2016	Centralised - Authorisation (orphan status)	EMA/H/C/3854	(2016) 3278 of 26/05/2016	sum <input type="checkbox"/>	dec <input type="checkbox"/>	anx <input type="checkbox"/>
20/07/2017	Centralised - Variation	EMA/H/C/3854/II/6				

# What can be improved

- Indication of PIP in Community register
- PIP number and decision number
- Link to EMA website

# Clinical Trial Regulation

## Protocol:

- Description of the group /subgroup participating;
- Description of the inclusion/exclusion criteria;
- Justification for the gender and age allocation of subjects;
- If a specific gender or age group is excluded or underrepresented the reasons have to be given.

# Clinical Trial Regulation

Assessors:

- Specific paediatric expertise.

Ethics committees

- They will have to take into account the views of lay persons (in particular patients/patients' organisations).

# Clinical Trial Regulation

- Specific rules on paediatric trials;
- PIP to be considered in the assessment of the CT application;
- Increased transparency on trials (start date, end date, results);
- Clinical trial database.

# Clinical Trial Regulation

The Regulation will greatly improve the transparency of clinical trials

[http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general\\_content\\_000629.jsp&mid=WC0b01ac05808768df](http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000629.jsp&mid=WC0b01ac05808768df)





# Thank you for your attention!

***Disclaimer:*** The views and opinions expressed in these PowerPoint slides are those of the presenter; they do not necessarily reflect the opinion of the European Commission.