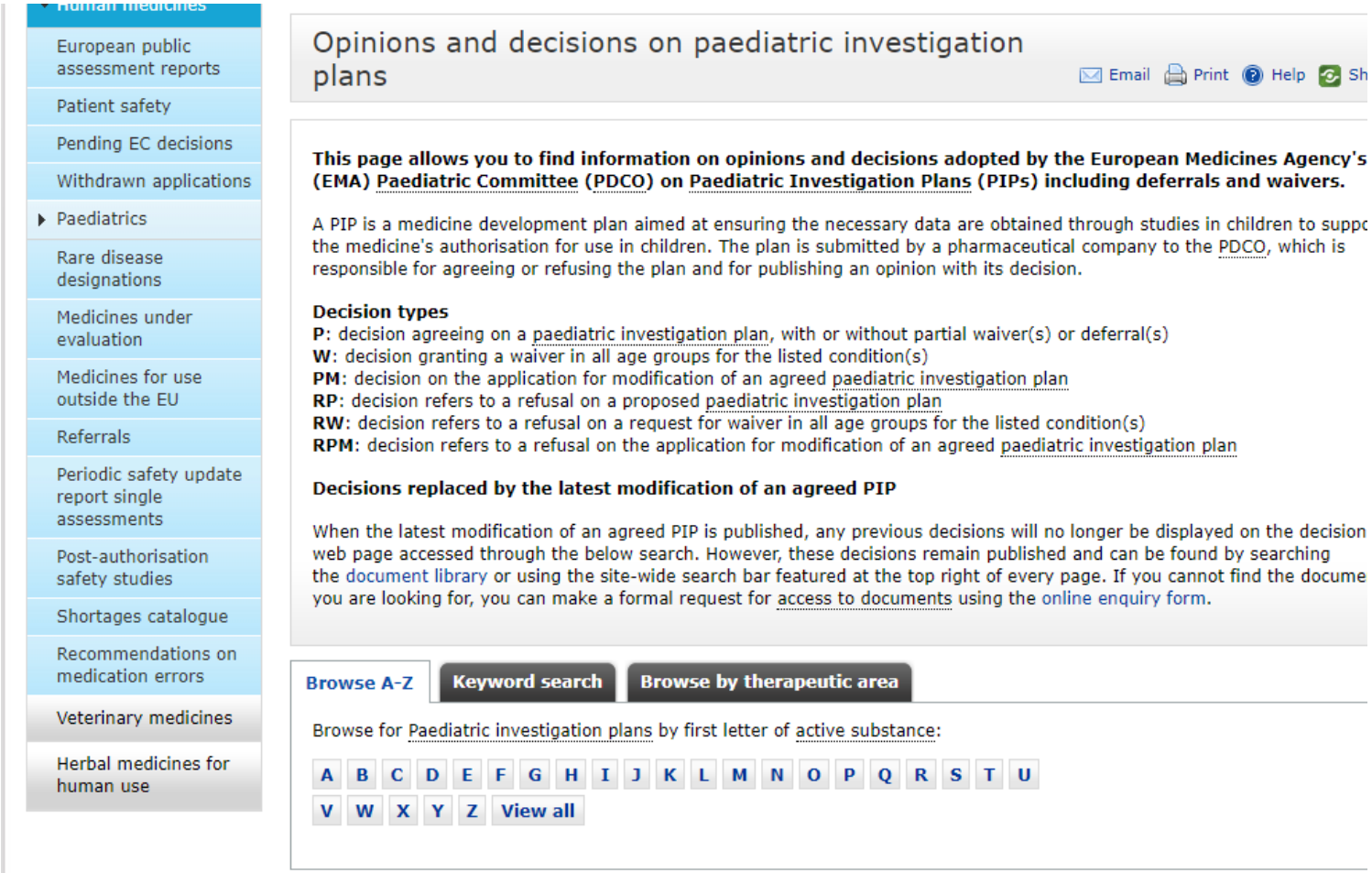
Transparency measures

EMA/EC multi-stakeholder workshop

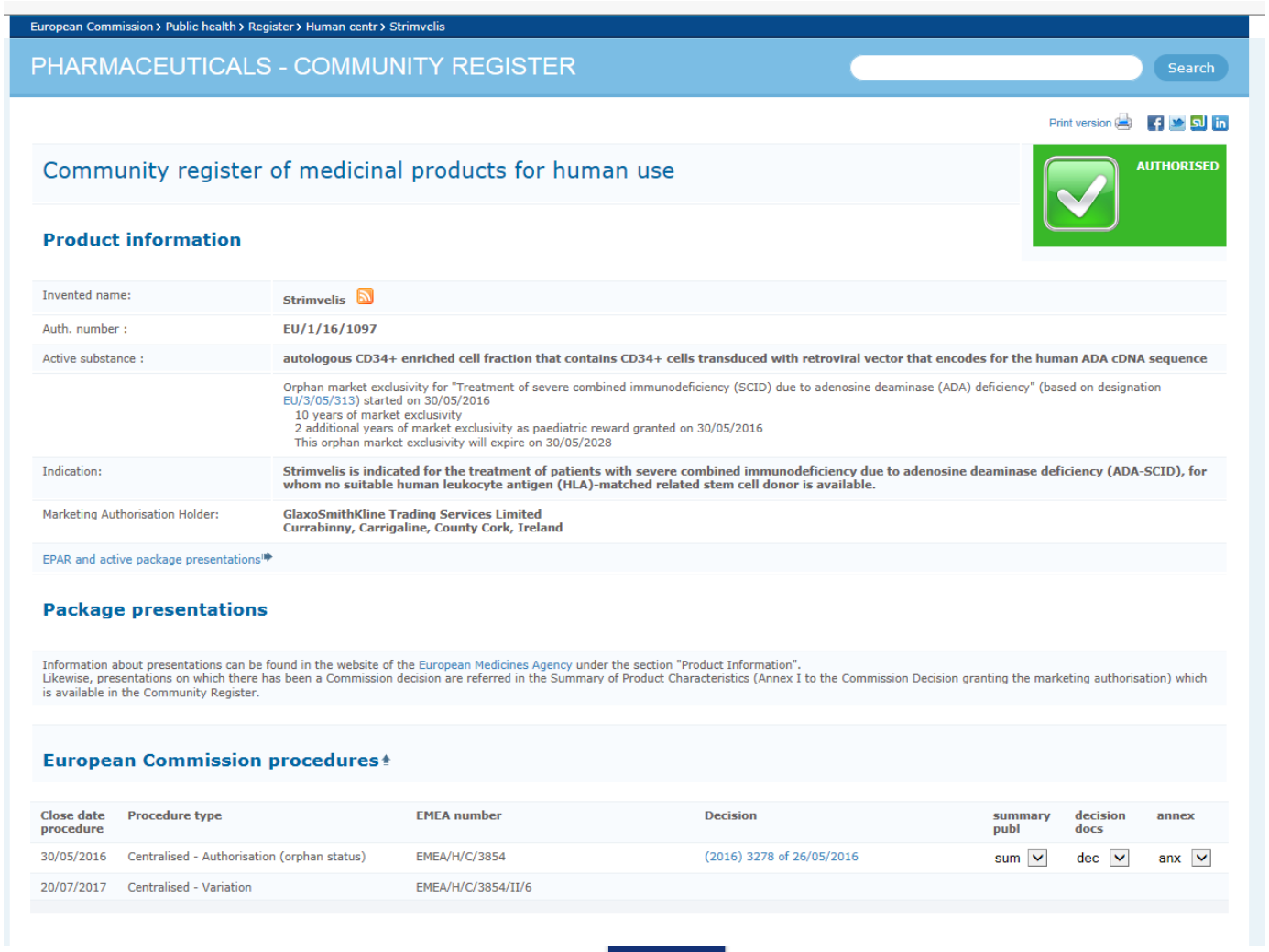
Paediatric Regulation

London, 20 March 2018

Information available- PIP decisions



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

AUTHORISED

Product information

Invented name:	**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	EMEA number	Decision	summary publ	decision docs	annex
30/05/2016	Centralised - Authorisation (orphan status)	EMEA/H/C/3854	(2016) 3278 of 26/05/2016	sum	dec	anx
20/07/2017	Centralised - Variation	EMEA/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

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.