EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

PCWP/HCPWP feedback from CHMP

Presented by: Fátima Ventura – Concha Prieto(CHMP)

March 2023

Summary (March 2020 – March 2023)

- CHMP early contact with patient organisations
  - Pilot phase
  - Current state
- HCP/Patients further input in the context of CHMP activities

CHMP early contact with patient organisations

January 2021
EMA/97615/20212021
Stakeholders & Communication Division

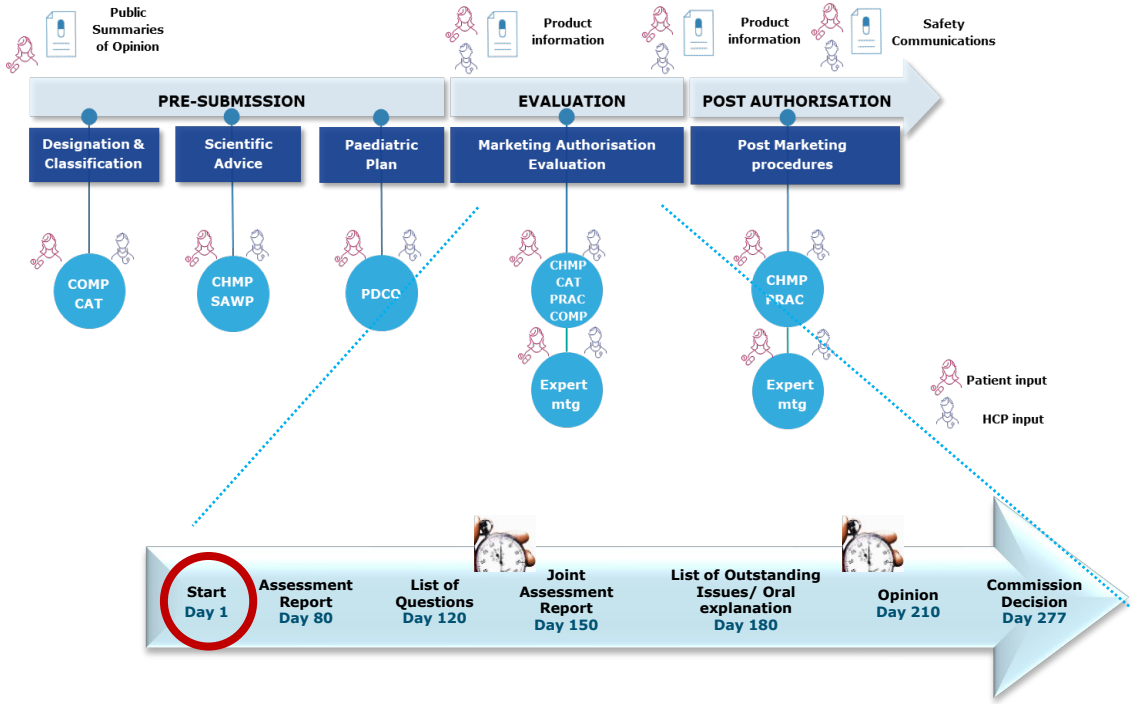
Pilot phase for CHMP early contact with patient / consumer organisations

https://www.ema.europa.eu/en/documents/other/pilot-phase-chmp-early-contact-patient/consumer-organisations_en.pdf

- Patients/representatives involved in EMA activities at various timepoints over (including CHMP evaluations)
- Requests for patient input generally late in the evaluation often once major objections have been identified (e.g. expert meeting, oral explanation)
- Late input - missed opportunities to properly incorporate patient perspectives

- **Aim**: make current engagement practices more efficient - proposed to establish contact with relevant patient / consumer organisations at the start of new medicines assessment
- **Contribution**: QoL, treatment options, unmet medical needs - CHMP well-aware of all aspects since beginning
- Expected to facilitate further interactions with patients as the procedure progresses.
- In line with CHMP work plan objective and EMA's Regulatory Science Strategy

CHMP early contact with patient organisations



- Relevant organisations contacted at start of orphan MAA's
- Patient organisations invited to share key aspects from their perspectives of living with the condition (3-4 weeks to respond) (in advance of first AR).
- Information shared with (Co-) Rapporteurs (and company for transparency) - Rapps decide if information provides added value, is useful for assessing the dossier, and if merits being included in AR.
- Value of patient input received during pilot assessed by short questionnaire

EUROPEAN MEDICINES AGENCY
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26 July 2022
EMA/665019/2022
Stakeholders & Communication Division

Pilot on early dialogue with patient organisations for orphan marketing authorisation applications: Outcome Report

https://www.ema.europa.eu/en/documents/report/pilot-early-dialogue-patient-organisations-orphan-marketing-authorisation-applications-outcome_en.pdf

Pilot outcome summary

- 37 procedures over 17 months (2021-2022)
- Rapporteurs were positive and input received reflected usefulness and benefit of reaching out to patient organisations at start of assessment of MAA's.
- Patients provided new insights that contributed to the D80 assessment report.
- 41% of cases contributed to the development of the first assessment report
- Information from patients related to *daily impacts*, *treatment options*, *perspectives and perceptions of adverse effects*, *what constitutes important improvements and desired benefits for new treatments* have proven to be insightful / helpful
- Pilot now a new methodology to be continued and extended to medicines of potential significant impact.

Reflecting patient perspective in the CHMP assessment report

23 June 2022
EMA/CHMP/597782/2022
Committee for Medicinal Products for Human Use (CHMP)

CHMP day 120 list of questions

Overview and list of questions

Patient's engagement

Being engaged in the EMA pilot "*CHMP early contact with patient organisations*", the EMA contacted relevant patient organisations for Fabry disease during the first round of this procedure. The aim of the pilot is to enable patients to share their experience, concerns and needs related to their condition with the Rapporteurs/CHMP so that these can be considered in a timely manner during the assessment process, where appropriate.

The information in this section was received from the patients' organisations relating to Fabry disease; their feedback has been considered during the assessment of this procedure.

Fabry disease is a life-threatening, complex multi-organ disease. In addition to the life-threatening aspects of the disease, there are many symptoms that severely affect the patients´ wellbeing and quality of life on a daily basis (such as constant pain, GI symptoms or fatigue). There are several ERT

Reflecting patient perspective in the CHMP assessment report

Amsterdam, 10 November 2022
EMA/CHMP/762284/2022
Committee for Medicinal Products for Human Use (CHMP)

CHMP Day 180 second list of outstanding issues

2.1.6. Patient's engagement

Being engaged in the EMA pilot "*CHMP early contact with patient organisations*", the EMA contacted relevant patient organisations for Pompe disease during the first round of this procedure. The aim of the pilot is to enable patients to share their experiences, concerns and needs related to their condition with the Rapporteurs/CHMP so that these can be considered in a timely manner during the assessment process, where appropriate.

The information in this section was received from the patients' organisations relating to Pompe disease; their feedback has been considered during the assessment of this procedure.

All patients expressed the need to be able to adjust the dose of their enzyme replacement therapy until the optimum levels are reached (personalised dosing).

No limits in terms of manufacturing capacities should restrict the ability to use higher doses (Genzyme had experienced tensions on supply due to higher demand than expected back in 2008, but since then, no biosimilar has been introduced on the market, the price has not changed, and not all member states agree to cover higher doses).

Most patients expect that a new treatment could stabilise the disease more than existing ones; some recovery would, of course, be welcomed, but experience with alglucosidase alfa might limit this expectation.

With miglustat, diarrhoea is reported the day the product is taken, which can exacerbate this symptom for people with Pompe disease suffering from GI disorders. These episodes can be controlled (no carbohydrate products ingested the day before, and some medications can also help).

As most patients are taking alglucosidase alfa already, the administration of the miglustat and cipaglucosidase alfa combination poses no problem. However, the switch might require returning to the hospital for a short time for those receiving infusions at home, which could be a concern during the Covid-19 pandemic.

Home infusions are not applied in all member states or regions. Otherwise, when applied, all patients

Beyond the pilot phase

- The early contact methodology has now become a regular part of CHMP's contact with stakeholders.

- Now include all indications and not only rare diseases.

- Also eligible healthcare professional organisations are planned to be consulted as well shortly.

Data of early patients contact after the pilot (since 2022)

Month/year	Type of procedure	# responses from PCO
September 2022	3 orphans	1 response
October 2022	3 orphans 2 non orphans	4 responses
1 December 2022	3 orphans 2 non orphans	3 responses and 1 use of previous response for same indication
28 December 2022	1 orphan 1 non orphan	2 responses
24 January 2023	3 orphans 3 non orphans	ongoing

Interaction between CHMP and **Patients' representatives**

Participation in CHMP activities: 2020-2022

- **Contributing for decision on recomendations**

Number interactions	CHMP Activity
2020 – 42 (22 meetings) **2021 – 25 (14 meetings)** **2022 – 33 (15 meetings)**	**Scientific Advisory Groups/ Ad hoc Expert Groups** (neurology, oncology, haematology, viral disease)
2020 – 102; 2021 – 90	**Scientific advice, protocol assistance**
2020 – 10 (6 procedures - Hopeveus, Dapavirine, Arikayce, Gamifant, Fintepla, Sogroya**)** **2021 – 7 (5 procedures -** Evrysdi, Zolgensma, Ozawade, Raylumis, Tecentriq**)** **2022 – 2 (1 procedure -** Miplyffa**)**	**Oral explanations**

Interaction between CHMP and **HCP representatives**

Participation in SAG and Ad-hoc Experts Groups – 2020– 2022

- **Contributing for decision on recomendations**

Number interactions	CHMP Activity
2020 – 40 (18 meetings) 2021 – 25 (12 meetings) 2022 – methodology changed	**Scientific Advisory Groups/ Ad hoc Expert Groups** (psychiatry, neurology, oncology, haematology, immunology, and respiratory diseases)
2020 – 1 2021 – 4 2022 – methodology changed	**Scientific advice, protocol assistance**

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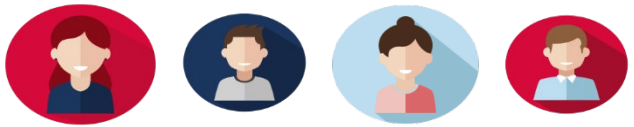

MINISTERIO DE SANIDAD, SERVICIOS SOCIALES E IGUALDAD
agencia española de medicamentos y productos sanitarios

¡Muchas gracias por tu interés!

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Any questions?

Further information

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