EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Progressing the concept of patient-centred development in practice

6th Industry Stakeholder Platform on R&D support – 4th June 2021

Nathalie Bere, Patient Engagement

Public and Stakeholders Engagement Department

An agency of the European Union

Ensuring the patient voice is incorporated throughout medicine development and associated evidence generation

EMA's Regulatory Science Strategy to 2025 and Joint EU Medicines Agencies Network strategy to 2025 - defines future direction of engagement - driving collaborative evidence generation to improve quality of regulatory evaluations and outcomes;

Includes two complementary elements:

1. Enhance generation and use of **patient experience data**

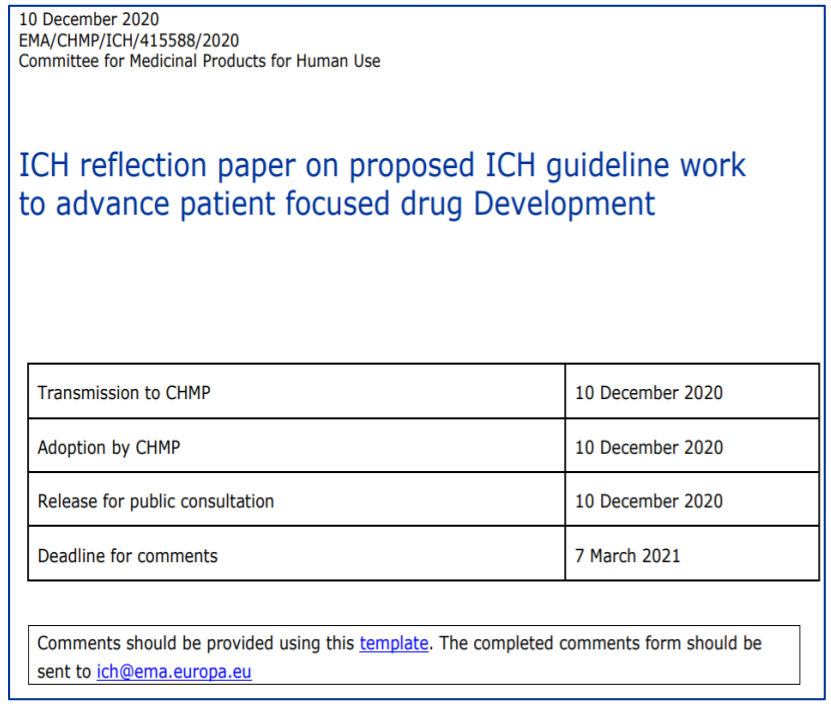
2. Expand methodologies for **patient engagement** during regulatory assessments

1. Enhance generation and use of **patient experience data**

ICH Reflection Paper on Patient Focused Drug Development

Proposes new ICH guidelines for harmonized approach to inclusion of patient's perspective in methodologically sound and sustainable way, to improve quality, relevance, safety and efficiency of drug development and to inform regulatory decision making.

- Public consultation concluded March 2021
- Substantial feedback received, currently being evaluated
- Input to be considered for next steps (update RP, guideline work)
- If proposed guideline agreed, should include plans for further public consultation and engagement



10 December 2020
EMA/CHMP/ICH/415588/2020
Committee for Medicinal Products for Human Use

ICH reflection paper on proposed ICH guideline work to advance patient focused drug Development

Transmission to CHMP	10 December 2020
Adoption by CHMP	10 December 2020
Release for public consultation	10 December 2020
Deadline for comments	7 March 2021

Comments should be provided using this template. The completed comments form should be sent to ich@ema.europa.eu

Proposed topics for ICH Guideline Development

1) Focus on informing the drug development process, patient-reported outcomes

- Use patient-reported outcomes (PROs) and other clinical outcome assessments (COAs)
- Define clinical meaningful changes in outcomes

2) Focus on the trade-offs between benefits and harms

- Quantify importance of benefits and harms for patients by measuring trade-offs
- Acknowledge that trade-offs may vary from patient to patient.

Patient experience data mapping

- Rationale: work with academics to support the development of guidance on collection and use of patient (experience) data for regulatory purposes:

- Mapping: research and analyse existing (qualitative and quantitative) methodologies, including:

  - Definitions
  - Experience and use
  - New methods (digital technologies)

- Make recommendations on most robust/relevant/feasible patient data collection methods for use within medicines development and assessment

- Contribute towards guidance development

Some other timely **initiatives**

- **FDA Patient Focused Drug Development guidance**
  https://www.fda.gov/drugs/developmentapproval-process-drugs/fda-patient-focused-drug-development-guidance-series-enhancing-incorporationpatients-voice-medical
- IMI **PREFER** project - Patient Preferences in benefit risk assessments during the drug life cycle:
  https://www.imi.europa.eu/projects-results/project-factsheets/prefer
- **SISAQOL-IMI** is to develop recommendations how to analyse and interpret data on health-related quality of life (HRQOL) and patient reported outcomes (PROs) in cancer clinical trials
  https://www.imi.europa.eu/projects-results/project-factsheets/sisaqol-imi
- IMI **PARADIGM** provides a unique framework to enable structured, effective, meaningful, ethical, innovative, and sustainable patient engagement (PE) demonstrating 'return on the engagement' for all.

2. Expand methodologies for **patient engagement** during regulatory assessments

Patient engagement during regulatory assessments

Continue involvement of individual patients along regulatory lifecycle:

- Scientific advice procedures
- SAG, Ad-hoc expert meetings
- CHMP oral explanations
- Review of documents

Early dialogue pilot:

- New CHMP pilot reaching out to patient organisations at the start of all orphan MAAs
- Gather important insights from patients from the beginning
- Facilitate further engagement as needed

Patient engagement during regulatory assessments

Patient engagement in crisis management

Engaging patients during COVID-19;

- Involvement in discussions, user-testing and document review
- Civil society in scientific committees and in EMA COVID-19 Taskforce
- Regular updates on COVID-19 pandemic to PCWP/HCPWP & Public stakeholder meetings
- Channel public health messages directly to patients, healthcare professionals and citizens to increase effectiveness and ensure EMA is trusted & regular source of reliable information

Development of Stakeholder Engagement Strategy

- EMA has established frameworks with its key stakeholder groups.
- Need an overarching stakeholder framework to streamline interaction and optimise oversight
- Foster implementation of EMAN 2025 and RSS Strategies and impact of EMA Extended Mandate on stakeholder engagement
- Rationalise Stakeholder engagement methodology;
  - Review format for stakeholder interactions and type of events/engagement
  - Look at new areas for further and earlier multi-stakeholder engagement
  - Update framework of interaction between EMA and patient/consumer organisations
  - Develop specific Action Plan for patient/consumer engagement

Thank you for your attention

Further information

Nathalie.Bere@ema.europa.eu

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000

Follow us on **@EMA_News**