EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/FDA – Patient-Focused Drug Development ICH Reflection Paper

HCPWP/PCWP meeting

Presented by Milton Bonelli – 3rd March 2021
ICH Management Committee representative for EC/EMA

Agenda

1. ICH Background
2. Reflection papers
3. Patient Focused Drug Development
4. Next steps

ICH – Membership

Founding Members

Regulatory

- EC, Europe
- FDA, United States
- MHLW/PMDA, Japan

Industry

- EFPIA
- JPMA
- PhRMA

(non-founding) Members

Regulatory

- Health Canada, Canada
- Swissmedic, Switzerland
- ANVISA, Brazil
- HSA, Singapore
- MFDS, Republic of Korea
- NMPA, China
- TITCK, Turkey
- TFDA, Chinese Taipei

Industry

- BIO
- Global Self-Care Federation
- IGBA

Observers

Legislative or Administrative Authorities (17) and WHO

Regional Harmonisation Initiatives (RHIs)

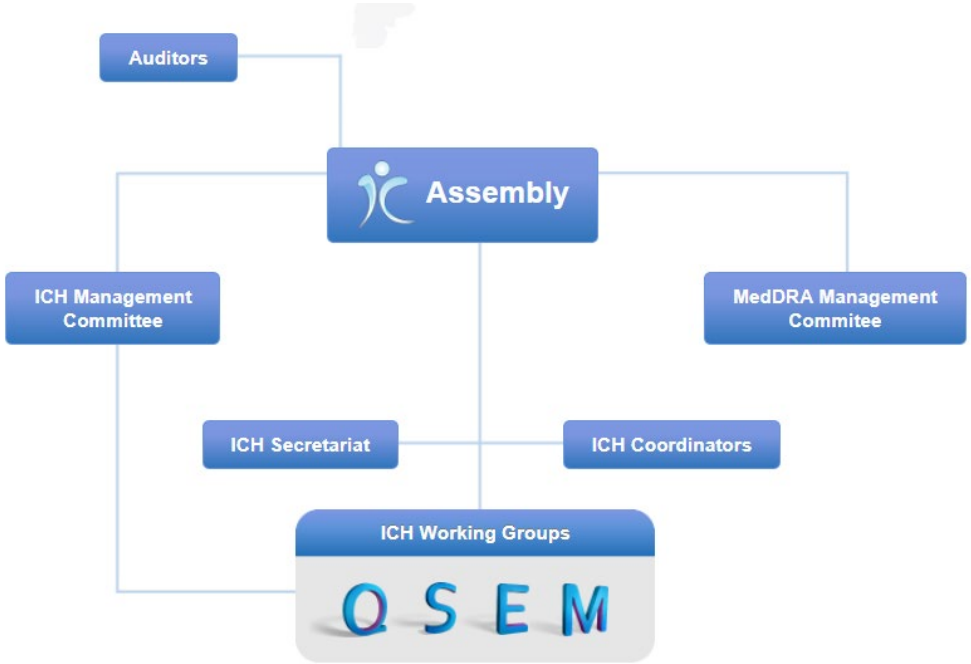
International Pharmaceutical Industry Organisation

- APIC; IFPMA

International Organisations regulated/affected by ICH Guideline(s)

- Bill & Melinda Gates Foundation
- CIOMS
- EDQM
- IPEC
- PIC/S
- USP

ICH – Structure



Now also Subcommittees:

1. New Topics
2. Implementation
3. Training

Agenda

1. ICH Background

2. Reflection papers

3. Patient Focused Drug Development

4. Next steps

ICH – From proposals to Guidelines

Strategic Discussions	Reflection Paper	New Topics
Intended to help ICH articulate a strategy and goals for future harmonization work		Will become a single GL effort/Q&A
Covers a domain area of ICH interest as high level principles	Covers a large area of interest including individual Guideline proposals	Covers a focused area with a concrete scope and specific objectives
Progress on rolling basis, decisions can happen at every bi-annual meeting		Yearly decisions at the June meeting

ICH – Reflection papers

- Scientific requirements for pharmacoepidemiological study data
  - Glossary used for pharmacoepidemiological studies intended for regulatory submission
  - Format of study protocols/reports intended for regulatory submission
- GCP renovation
  - ICH E8 General Considerations for Clinical Trials
  - ICH E6 Good Clinical Practices (and Annexes)
- Scientific standards for generic drugs
  - BE guideline for oral immediate release products
  - BE for medicines administered through other routes of admin
  - BE for complex formulation/products
- *Patient-Focused Drug Development* (PFDD)

Agenda

1. ICH Background

2. Reflection papers

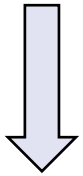
3. Patient Focused Drug Development

4. Next steps

Patient Focused Drug Development RP

1. Identifies key areas for further incorporation of the patient's perspective

2. Aims to improve the quality, relevance, safety and efficiency of drug development and inform regulatory decision making.



- Patient-meaningful COAs and endpoints
- Patient preference information for preference-sensitive Benefit/risk assessments

Patient Focused Drug Development – proposed actions 1/2

Drug Development Process Informed by Patient Perspective	Potential ICH Guideline Topic
Discovery/ Development: • What disease effects and treatment burdens matter most to patients that might be addressed by a medical therapy? (How) does this vary by subpopulation? • What would be the best way to measure these disease or treatment burdens/effects in a clinical trial? • What would be the most appropriate endpoints to use in clinical trials (and robust enough to inform regulatory decision making)? • What is a clinically meaningful changes in an endpoint from a patient perspective? • How to define meaningful change in a patient over time?	New ICH guideline addressing what to measure in a clinical trial, including refining the set (list) of important impacts and concepts from patients, to select, modify or develop clinical outcome assessments (COAs) that can demonstrate change, defining endpoints, and meaningful change. The scope of this guideline would include: • Qualitative and quantitative methods to identify disease/treatment impacts important to patients that would be candidate concepts for measurement with patient reported outcome (PRO) measures or other types of COAs or in quantitative assessments of the patient perspective. • The approach to organizing and structuring the content of the guideline document would undergo further consideration as this work advances under an ICH new topic proposal. One approach would be to develop the main document with an extensive focus on common considerations for all COAs and include annexes with considerations that may only apply to certain COA types such as observer reported (ObsRO), clinician reported (ClinRO), performance based (PerfO) measures, etc.

Patient Focused Drug Development – proposed actions 2/2

Drug Development Process Informed by Patient Perspective	Potential ICH Guideline Topic
Patient Preferences Informing Drug Development, Benefit-Risk Assessments, and Other Decisions: • What methods and approaches could be used to identify which treatment benefits would be most desirable to obtain and which risks would be most important to avoid, or to explore what patients might consider to be acceptable tradeoffs of increased expected harm(s) for a specified increase in expected benefit with a new medicinal product? • What are methodological considerations for sponsor conduct of patient preference studies to provide credible and reliable findings to support regulatory decision making?	New ICH guideline addressing methods for elicitation/ collection, analysis, reporting and application of qualitative or quantitative assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among the alternatives.

Agenda

1. ICH Background

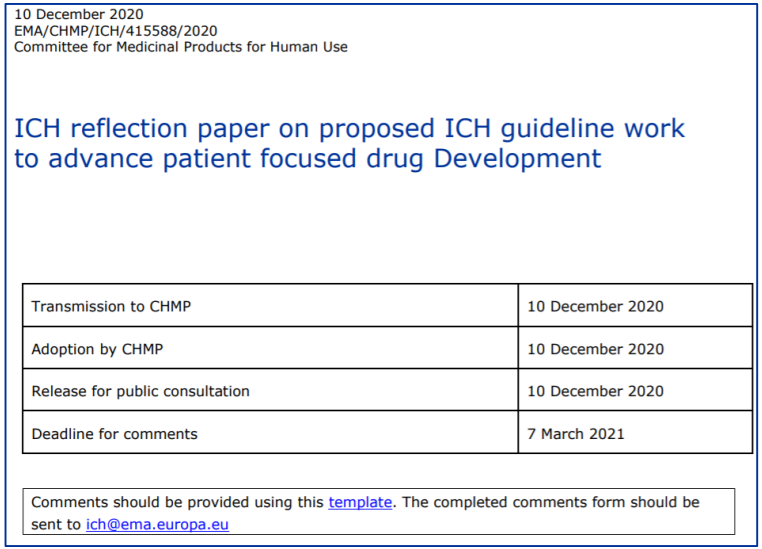
2. Reflection papers

3. Patient Focused Drug Development

4. Next steps

PFDD - Next Steps

- As endorsed by the ICH Assembly and CHMP, the PFDD Reflection Paper is now posted for public comments
- Comments will be considered for further steps (e.g., Update RP, proceed to guideline work)
- If/when the proposed guideline work is advanced, the concept paper 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

https://www.ema.europa.eu/en/documents/scientific-guideline/ich-reflection-paper-proposed-ich-guideline-work-advance-patient-focused-drug-development_en.pdf

Any questions?

Further information

[Insert relevant information sources or contact details as applicable.]

European Medicines Agency
30 Churchill Place • Canary Wharf • London E14 5EU • United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

Follow us on **@EMA_News**