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SCIENCE MEDICINES HEALTH

ICH Patient-Focused Drug Development activities

EMA Multi-stakeholder workshop
Patient experience data in medicines development and regulatory decision-making

Presented by Milton Bonelli – 21 September 2022
ICH Management Committee representative for EC/EMA

ICH - Background

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- Unique harmonisation project, involving the Regulators and research-based Industries across the globe
- Started in 1990, reformed in 2015 to promote further global outreach
- Well-defined objectives:
    - To improve efficiency of new drug development and registration process
    - To promote public health, prevent duplication of clinical trials in humans and minimize the use of animal testing without compromising safety and effectiveness
- Accomplished through the development and implementation of harmonised Guidelines and standards

ICH – Membership

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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
- MHRA, UK
- NMPA, China
- SFDA, Saudi Arabia
- TITCK, Turkey
- TFDA, Chinese Taipei

Industry

- BIO
- Global Self-Care Federation
- IGBA

Observers

Legislative or Administrative Authorities (20) and WHO

Regional Harmonisation Initiatives (RHIs)

International Pharmaceutical Industry Organisations

- APIC; IFPMA

International Organisations regulated/affected by ICH Guideline(s)

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

ICH Articles of Association are public - https://www.ich.org/page/articles-procedures

ICH Patient-Focused Drug Development activities

ICH – From proposals to Guidelines

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Reflection papers

- Intended to help ICH articulate a strategy and goals for future harmonization work
- Covers a domain area of ICH interest as high level principles and outlines individual new ICH topic proposals
- Progress on rolling basis, decisions can happen at every bi-annual meeting

https://www.ich.org/page/reflection-papers

New Topic proposals

- Will become an ICH topic – i.e. a new guideline
- Covers a focused area with a concrete scope and specific objectives
- Yearly decisions (go/no-go) at the June meeting

Patient Focused Drug Development Reflection paper

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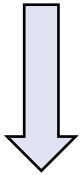
1. Identifies key areas for further incorporation of the patient's perspective

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



New Topic proposals

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

ICH Patient-Focused Drug Development activities

PFDD Reflection paper - process

- Co-sponsored EMA/FDA, supported by ICH parties in its first version
- Draft version underwent public consultation in 2021

Non-ICH stakeholders commenting on the first version included:

1. Private companies
2. Learned societies
3. Patient organizations
4. Individuals (patient/patient advocate/academia)
5. Industry associations

PFDD Reflection paper - process

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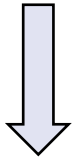
- Overall support for the initiative(s)
- Three types of comments:

1. Modifications proposed within the reflection paper, including mechanisms for engagement of non-ICH stakeholders in the ICH process
2. Recommendations for the development of the proposed guidance
3. Recommendations on the use of existing tools / research / initiatives.

Patient Focused Drug Development Reflection paper

- Reflection paper final version published 2021 with dedicated overview of comments document
- Agreement that Concept papers for any upcoming guideline in this space should include plans for public consultation and engagement similar to the approach being taken for ICH E6(R3)/GCP



Two types of stakeholder engagement foreseen:

1) regional public engagement approach held by ICH member organizations
2) meetings with the expert working group (EWG).

PFDD - Next Steps

- PFDD Reflection Paper is final, no changes foreseen
- Comments received will be considered for further steps of the ICH process (i.e. guideline work)
- Collaborate with FDA on drafting new topic proposal (ongoing)
- Advance overarching ICH discussions on public consultation/engagement processes

Thank you

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PFDD RP – proposed actions 1/2

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Drug Development Process Informed by Patient Perspective	Potential ICH Guideline Topic
• 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, and are the methods acceptable for patients? • What would be the most appropriate endpoints to use in clinical trials (and robust enough to inform regulatory decision making)? • What are 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 and define endpoints and meaningful change. The scope of this guideline would include: • Qualitative and quantitative methods to identify disease/treatment/preventive 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.

PFDD RP – proposed actions 2/2

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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.