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

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

• Scientific requirements for pharmacoepidemiological study data
  o Glossary used for pharmacoepidemiological studies intended for regulatory submission
  o Format of study protocols/reports intended for regulatory submission

• GCP renovation
  o ICH E8 General Considerations for Clinical Trials
  o ICH E6 Good Clinical Practices (and Annexes)

• Scientific standards for generic drugs
  o BE guideline for oral immediate release products
  o BE for medicines administered through other routes of admin
  o 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
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**

<table>
<thead>
<tr>
<th>Drug Development Process Informed by Patient Perspective</th>
<th>Potential ICH Guideline Topic</th>
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<tr>
<td><strong>Discovery/ Development:</strong></td>
<td>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:</td>
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<td>• What disease effects and treatment burdens <strong>matter most to patients</strong> that might be addressed by a medical therapy? (How) does this vary by subpopulation?</td>
<td>• 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.</td>
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<tr>
<td>• What would be the <strong>best way</strong> to measure these disease or treatment burdens/effects in a clinical trial?</td>
<td>• 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.</td>
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<td>• What would be the most <strong>appropriate endpoints</strong> to use in clinical trials (and robust enough to inform regulatory decision making)?</td>
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<td>• What is a <strong>clinically meaningful changes</strong> in an endpoint from a patient perspective?</td>
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<td>• How to define meaningful change in a patient <strong>over time</strong>?</td>
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### Drug Development Process Informed by Patient Perspective

**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?

### Potential ICH Guideline Topic

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.
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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

Any questions?

Further information

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

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