Regulatory Science Research in PMDA

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GAP between expectation and Reality

Concerns and Needs for medical services

New study design and analytical tool
Predictable model for efficacy/safety

Advancing Regulatory Science

Traditional Science

Current Issues
SAE after approval, Lower success rate, Drug/Device lag, Insufficient risk communication, Uncertainty for decision

New approach on risk communication and management
Objective evaluation tool for benefit/risk assessment

Ensure Social Balance

Medical Needs

Regulatory Science
Traditional Science
Regulatory Science Bridge to a Valley of Death

Table: PMDA’s Articles published in the journal (2013)

<table>
<thead>
<tr>
<th>Title</th>
<th>Journal</th>
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<tbody>
<tr>
<td>Regulatory challenges in the review of data from <strong>Global Clinical Trials</strong>: PMDA perspective</td>
<td>Clin Pharmacol Ther. 2013, in press</td>
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<td>Characteristics of <strong>pharmacogenomics/biomarker</strong>-guided clinical trials for regulatory approval of <strong>anti-cancer drugs</strong> in Japan</td>
<td>J Human Genet 2013, advance online publication, 9 May 2013; doi:10.1038/jhg.2013.36</td>
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<td>Improving clinical trial <strong>sampling</strong> for future research - an international approach: outcomes and next steps from the DIA future use sampling workshop 2011</td>
<td>Pharmacogenomics. 14(1):103-12, 2013.</td>
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</table>
PERSPECTIVES

OPINION

Pharmacogenetics in the evaluation of new drugs: a multiregional regulatory perspective

Marc Maliepaard, Charity Nofziger, Marisa Papaluca, Issam Zineh, Yoshiaki Uyama, Krishna Prasad, Christian Grimstein, Michael Pacanowski, Falk Ehmann, Silvia Dossena and Markus Paulmichl

PMDA EMA FDA

NATURE REVIEWS | DRUG DISCOVERY VOLUME 12 | FEBRUARY 2013 | 103
PMDA’s initiatives to advance Regulatory Science
PMDA Science Board

Seeds of medical products discovered in Japan

Discovery in Basic research e.g.;
iPS

PMDA

Pharmaceutical Affair Consultation  Scientific Consultation  Review  Safety Measure

Offices of Review; Drugs, Biologics, Medical Devices
Offices of Safety

Office of Review Innovation

Science Board

Board Member

Academia

PMDA Science Board

Practical Use

Innovative Medical Products

e.g.; HAL

iPS-derived products

Pharmaceuticals & Medical Devices Agency

12th Kitasato-Harvard Symposium, Tokyo
May 14th 2013
Collaborative Graduate School Program

- **PMDA Staff**
  - Visiting Professor (Lecture in regulatory science)
  - Graduate student (Ph.D. program); Research in University

- **University student**
  - Graduate student (Ph.D. program); Research in PMDA
Agreement with 17 University
(As of end of March 2013)

http://www.pmda.go.jp/regulatory/graduate_school.html
Regulatory Science Research in PMDA


3. Current situation and challenges in the evaluation of drugs used in the elderly.

4. Evaluation of the effects of ethnic factors on the efficacy and safety of the drug based on global clinical trial data.

5. Cross-product evaluation of differences on approved doses between Japan and US/EU based on clinical trial data including PK/PD data

6. Effects on pharmacovigilance of post marketing surveillance targeting all cases

7. A study of risk-based approach on document-based GCP conformity inspection
Regulatory Science Research &
Human Resource Exchange Program
(for developing innovative drug, device, cell & tissue products for practical use)

- Proactive establishment of the guideline and standards
- Promoting development using innovative techniques

Outcomes of research:
- Improving quality of review and other services in PMDA
- Learning a state-of-the-art technology

Pharmaceuticals & Medical Devices Agency

Reviewer

Researcher

Academia (University, Institute, Hospital)

Training in regulatory science
Effective research & development for regulatory approval
Research funds for developing innovative drug, device, cell & tissue products for practical use
Future workflow of review & consultation in PMDA
Future workflow of Review & Consultation in PMDA

- Dealing with state-of-the-art technology
  - iPS cell-based product
- Utilization of innovative methods
  - Pharmacometirics (Modeling & Simulation)
Advanced workflow of review/consultation using innovative assessment techniques

- e-Submission of study data
- data Accumulation
- NDA etc.

Evaluation / Analysis by PMDA
- Innovative Assessment Methods
  - Comprehensive analysis of stratified data
  - Active utilization of Modeling & Simulation
    - Disease model
    - Objective B/R assessment
    - Identifying AE-related factors etc.
- Giving additional scientific value to submitted data

Sophisticated Review & Consultation
- Effective & High Quality Review & Consultation
  - More evidence-based
  - More transparent

Advancing Regulatory Science
- Effective and successful development
- More scientific regulatory decision
- Epoch-making proposal leading the world

Practical use of Innovative Medical Products

Pharmaceuticals & Medical Devices Agency

12th Kitasato-Harvard Symposium, Tokyo
May 14th 2013
Regulatory Science Bridge

Stronger & More Complete Regulatory Science Bridge will help us in the future drug developments

Information

- HOMEPAGE (English)

- Regulatory Science Page
  http://www.pmda.go.jp/regulatory/index.html

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  uyama-yoshiaki@pmda.go.jp

Thank you for your attention