Regulatory Science Research in PMDA

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GAP between expectation and Reality

Concerns and Needs for medical services

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

Advancing Regulatory Science
- New approach on risk communication and management
- Objective evaluation tool for benefit/risk assessment
- Predictable model for efficacy/safety
- New study design and analytical tool

Ensure Social Balance

Medical Needs

Regulatory Science
- Traditional Science
Articles published by PMDA members

Number of articles published by PMDA members from 2005 to 2012, categorized by language: English and Japanese. The graph shows a steady increase in the number of articles published each year, with a significant rise in publications in English and a more fluctuating pattern in Japanese.
# 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 Global Clinical Trials: PMDA perspective</td>
<td>Clin Pharmacol Ther. 2013, in press</td>
</tr>
<tr>
<td>Characteristics of pharmacogenomics/biomarker-guided clinical trials for regulatory approval of anti-cancer drugs in Japan</td>
<td>J Human Genet 2013, advance online publication, 9 May 2013; doi:10.1038/jhg.2013.36</td>
</tr>
<tr>
<td>Improving clinical trial sampling 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>
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
Seeds of medical products discovered in Japan

Discovery in Basic research e.g.;

iPS

Basic Research

Pharmaceuticals & Medical Devices Agency

PMDA

Pharmaceutical Affair Consultation
Scientific Consultation
Review
Safety Measure

Offices of Review; Drugs, Biologics, Medical Devices

Offices of Safety

PMDA Science Board

Office of Review Innovation

Science Board

Board Member

Academia

Practical Use

Innovative Medical Products

e.g.; HAL

iPS-derived products

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

PMDA

Collaboration

Joint Graduate School agreement

Graduate School

- 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)

Hokkaido University
Teikyo University
Musashino University
Gifu University
Gifu Pharmaceutical University
Kyoto Pharmaceutical University
Shujitsu University
Okayama University
Kobe University
Osaka University
Nagoya University
Nagoya City University
University of Tsukuba
Chiba University
Yokohama City University
University of Shizuoka

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.


http://www.pmda.go.jp/regulatory/research.html
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

Outcome of research

Training in regulatory science

Effective research & development for regulatory approval

Learning a state-of-the-art technology

Improving a quality of review and other services in PMDA

Human Resource Exchange & Development

Reviewer

Researcher

Academia (University, Institute, Hospital)
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

1. e-Submission of study data
2. data Accumulation
3. Database

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
Products of science
(Substance, Knowledge, Information)

Regulatory Science Bridge

Data assessment
Balancing of various factors

Tools for data production

Drug A

Patients/Society

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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Thank you for your attention