PMDA's recent approaches for innovative medicines ～in order to deliver them sooner to People～

Masatoshi Narita, Ph.D.
Senior Executive Director
Pharmaceuticals and Medical Devices Agency

Today's Topics

1. Organization
2. Recent Approaches for Innovative Medicines
3. International Activities

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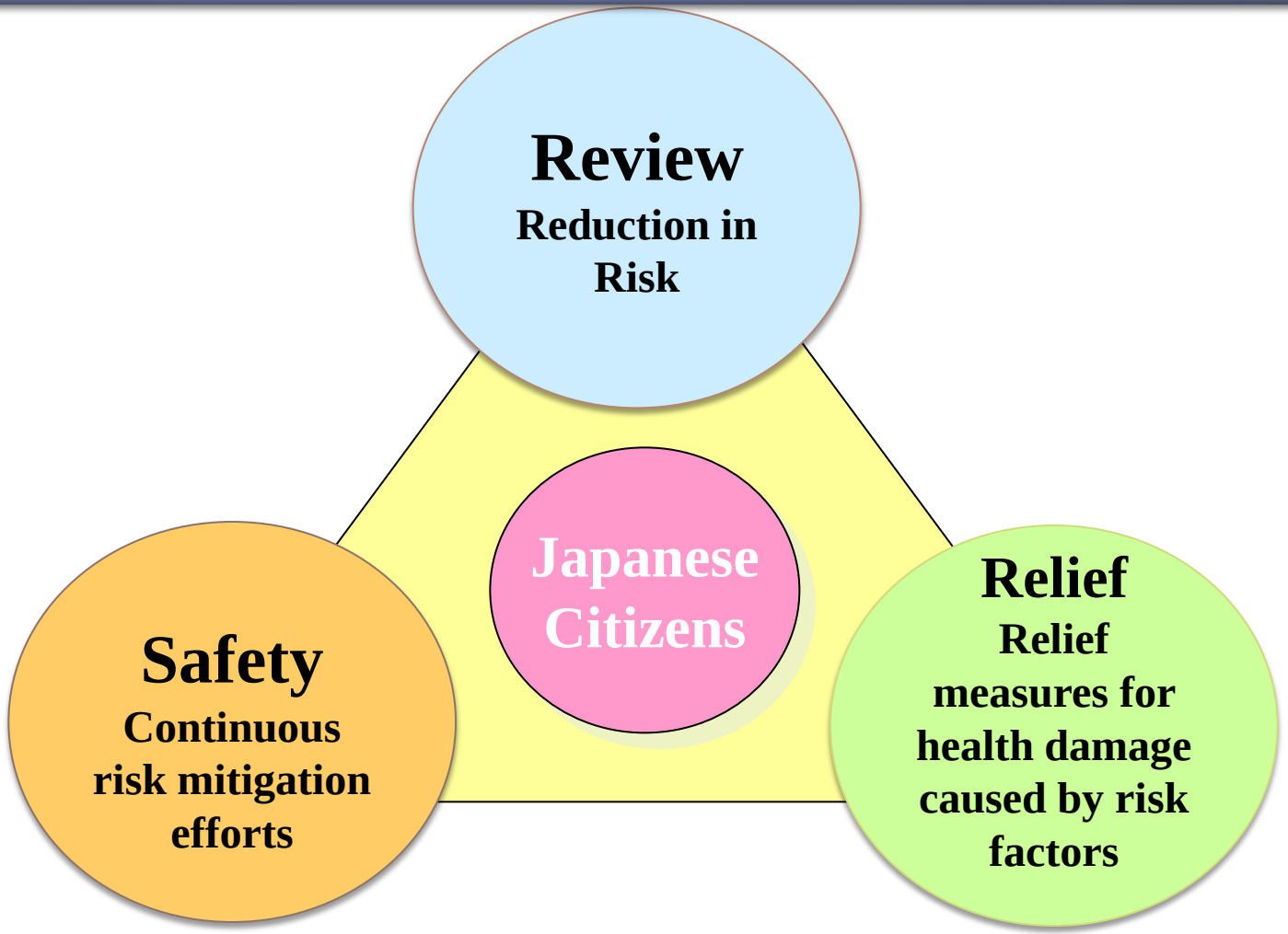
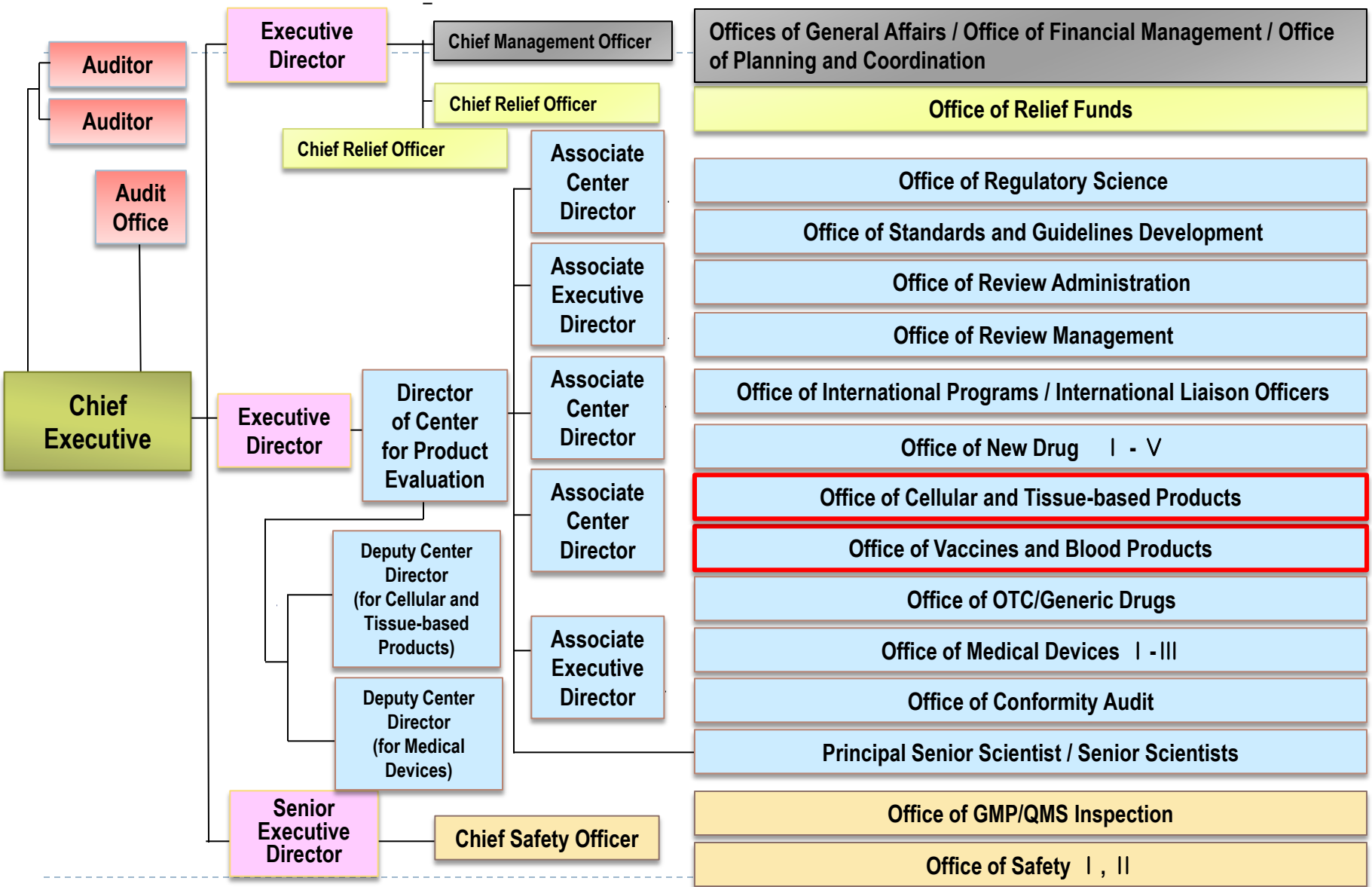
PMDA's Safety Triangle

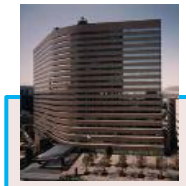
Unique Three-pillar System Securing Nation's Safety



Organization Chart of PMDA



Establishment of PMDA-WEST



PMDA(Tokyo)

PMDA-WEST (Osaka)

Pharmaceutical Affairs Consultation on R&D Strategy

GMP On-site Inspection

Collaboration

University, Research Institutions

Drug companies

Network to Support drug development

National Institute of Biomedical Innovation

Office of strategy to support drug development

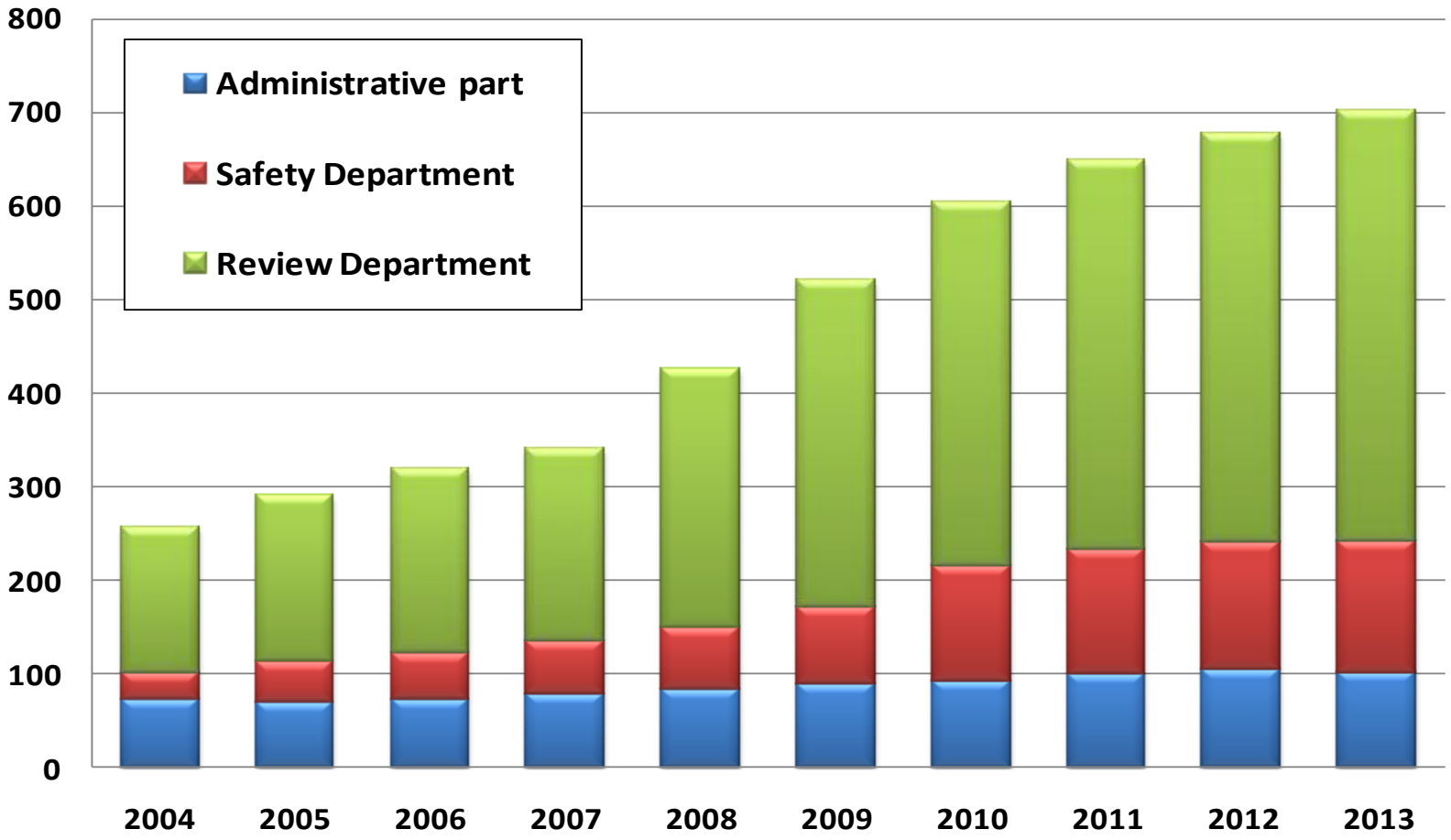
RIKEN

AIST

Research Institutes for drug development

University & Private research institutes

PMDA Staff Size



Review Time for New Drugs

Priority Review Products

	FY 2008	FY 2009	FY 2010	FY 2011	FY 2012	FY2012 Target
Total Review Time (Month)	15.4	11.9	9.2	6.5	6.1	9
Regulatory Review Time	7.3	3.6	4.9	4.2	3.9	6
Applicant's time	6.8	6.4	3.4	2.0	1.1	3

Standard Review Products

	FY 2008	FY 2009	FY 2010	FY 2011	FY 2012	FY 2012 Target
Total Review Time (Month)	22.0	19.2	14.7	11.5	10.3	12
Regulatory Review Time	11.3	10.5	7.6	6.3	6.0	9
Applicant's time	7.4	6.7	6.4	5.1	3.9	3

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Innovative Medicinal seeds from Academia in Japan

ACTEMRA® Injection (Tocilizumab (r-INN))

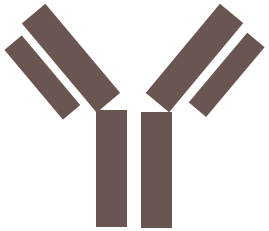
Approved in JAPAN; April 2005
(First marketing authorization)

- Target Identification / Target Validation

Professor Tadamitsu Kishimoto (Osaka University, Japan) identified IL-6 related to Castleman's disease (Blood 1989; 74:1360-1367)

- Extensive research & Development

ACTEMRA® (Tocilizumab) is a humanized monoclonal antibody targeting IL-6 receptor developed by Osaka University and Chugai Pharmaceutical Co., Ltd.

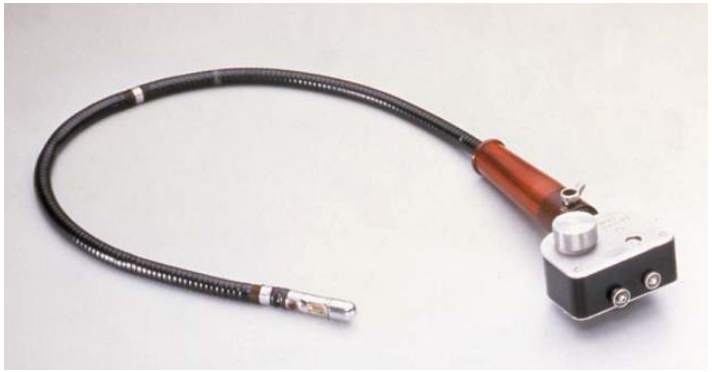


Innovative Medicinal seeds from Academia in Japan

1950 → 201X

University of Tokyo

OLYMPUS GASTROCAMERA GT-I



Copyright: The Japan Society of Mechanical Engineers.

University of Tsukuba

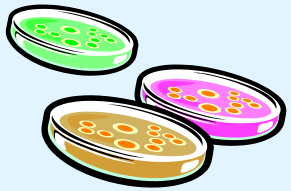
"HAL" (Hybrid Assistive Limb®)

Copyright: CYBERDYNE INC

Recent Approaches for Innovative Medicines

Basic Research

Seeds originated in Japan



Quality Study → Non-Clinical Trial → Clinical Trial → Review → Approval → Post-marketing

correspond better to the characteristics of innovative new medicines in all phases from seeds to practical use

Practical Use

Innovative Drugs and Medical Devices

- Science Board
- Pharmaceutical Affairs Consultation on R&D Strategy
- Promotion Program for Practical Use of Innovative Drugs, Medical Devices, and Regenerative Medicines
- Program of Collaborative Graduate Schools
- Improvement of Safety Measures

Science Board

Issues of PMDA

① Being required to conduct review and consultation understanding of the research activities in state-of-the-art technologies such as nucleic acid medicine, Companion diagnostics, artificial heart, cellular & tissue-based products, cancer vaccine etc.,.

② Being required to adequately conduct review and consultation in the state of the art technologies from early stage of development for prompt supplying of products among the medical work front,.

③ Requiring a cooperation with academia, to continuously train for reviewers to catch up accelerating innovative technologies and contribute practical use of state of the art technologies.

Establishment of the Science Board

(May 14th ,2012)

Science Board

Science Board

- Committee members: External experts from Academia
- Not involved in the Review Process of individual products

Committee

Recommendation on

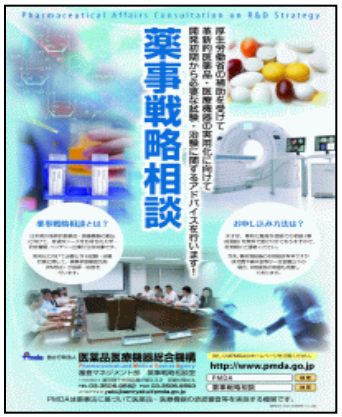
1. Review policy for innovative medical products
2. Development of guidelines
3. Regulatory Science Research
4. Personnel exchanges between PMDA and Academia
5. Election of External review experts
6. Improvements in the scientific aspects of review

Subcommittee

Deliberation on problems in each field
Collaboration with PMDA working team (RS research, guideline development, etc.)

Pharma-ceuticals	Medical Devices	Bio-based products	Cellular- & tissue-Based products

Pharmaceutical Affairs Consultation on R&D Strategy



Valley of Death

-Short of funds, Knowledge on Regulation and development strategy

Strategic Consultation

Basic Research

Pharmaceutical and Medical Devices candidates

Quality Study

Non-Clinical Study

Clinical Trial

Up to the level of POC studies

Practical Use

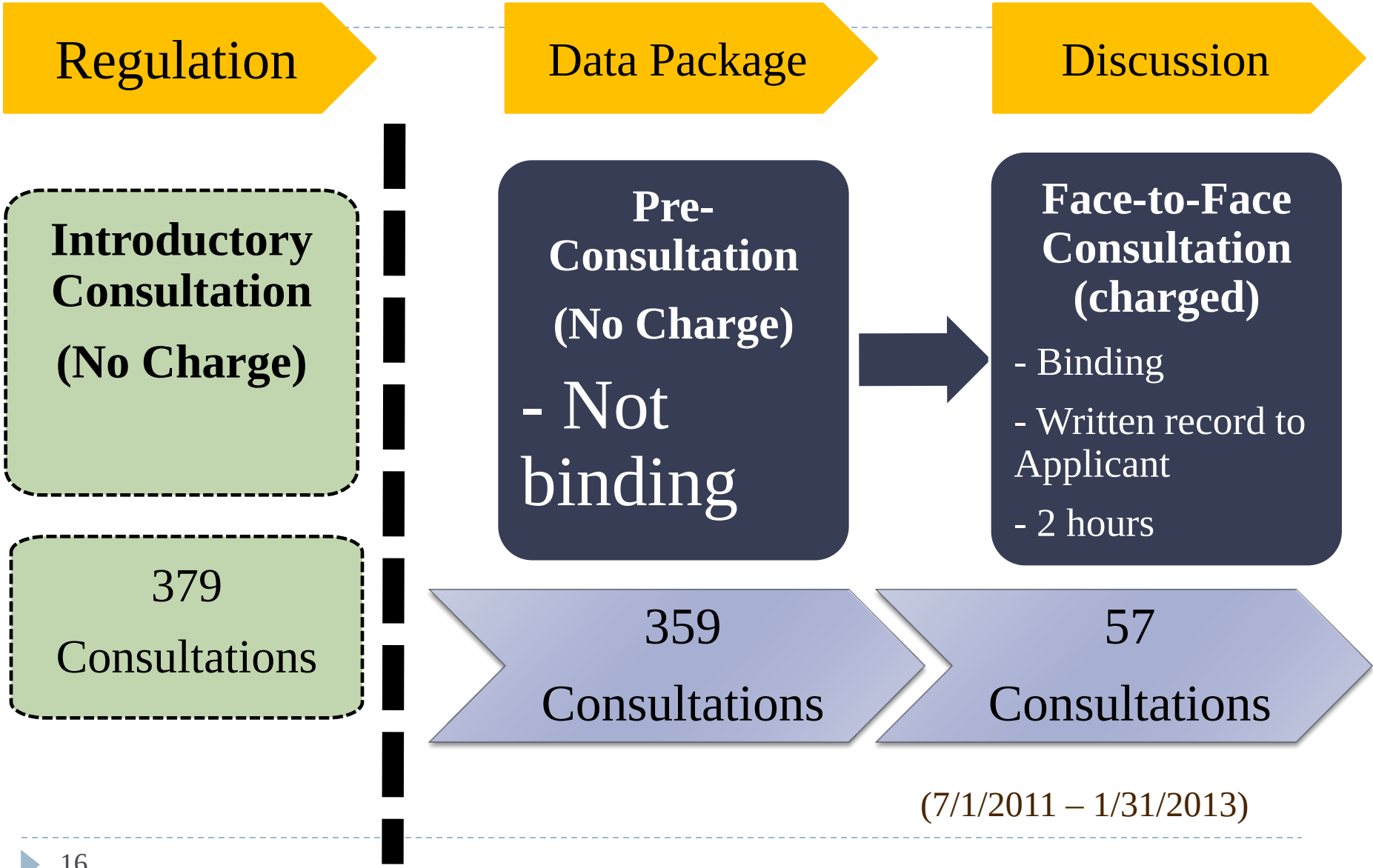
Innovative Products

* Further studies are handled by the Regular Consultation

Consultation on quality or toxicity study of biologics, cell-and tissue-based products

Consultation on endpoints or sample size of early clinical trial

Flow of R&D Strategy Consultation



(7/1/2011 – 1/31/2013)

Case of Face to Face consultation

Consulter	Product under development	Intended performance, Intended use, Indications
National Institute of Neuroscience, NCNP Department of Molecular Therapy Shin'ich Takeda	Morpholino oligos (Antisense)	Remedy for Duchenne muscular dystrophy (DMD)
United Centers for Advanced Research and Translational Medicine (ART), Tohoku University School of Medicine, Toshio Miyata	PAI-1Inhibitor （TM5509）	Improvement of disturbance of hematopoiesis on Cord blood transplantation
Center for iPS Cell Research and Application (CiRA), Kyoto University, Shinya Yamanaka	iPS Cell (Allo)	Starting Materials for cell & tissue products derived from iPS Cells
Sapporo Medical University, Osamu Honmou	Mesenchymal Stem Cell (Auto)	Improvement of neurological sign, activities of daily living disorders, and dysfunction associated with Stroke
CYBERDYNE INC.	ROBOT SUIT HAL (Hybrid Assistive Limb®) and Equipment for movement training used a subset of function on HAL	Devices for assistive movement with patients. Planed to introduce models which differ in intended use or indications.

Promotion Program for Practical Use of Innovative Drugs, Medical Devices, and Regenerative Medicines

human resource development

NIHS

pmda

Reviewers

Researchers

Universities and research institutions

Medical Institutions

Acquisition of innovative technologies
Speed up and improve product review

Outcome of researches

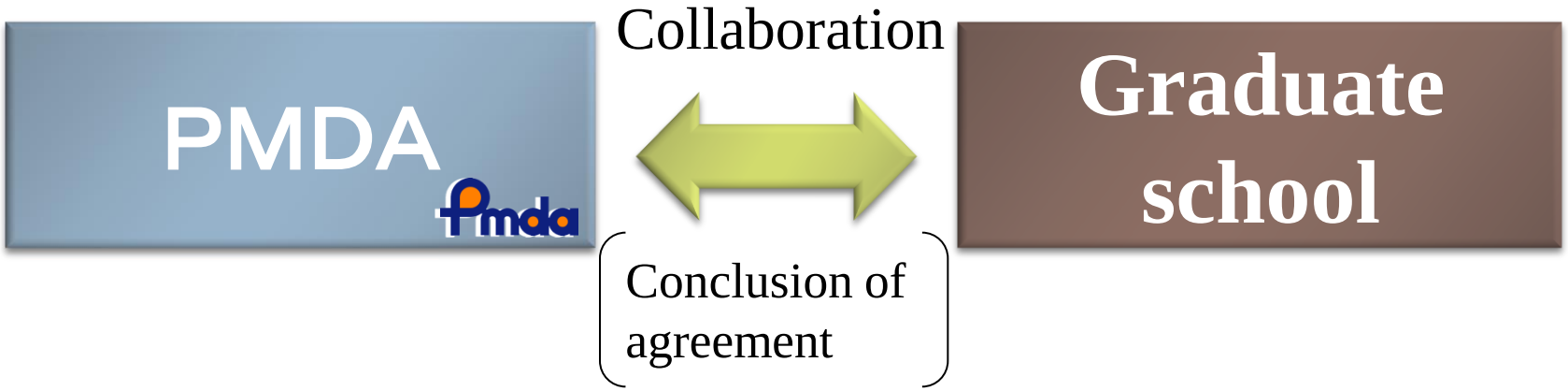
Fostering of Regulatory Scientist
Promotion of appropriate R & D

- **Develop standards and guidelines at early phase**
- **Facilitate practical application of innovative technologies**
- **Decrease drug /device lag**

Exchanging program in FY2012

- **Accepted 18 researchers from university, Research Institute etc.,**
- **Dispatched 30 PMDA staffs (including non-regular staff)to University, Research Institute etc..**

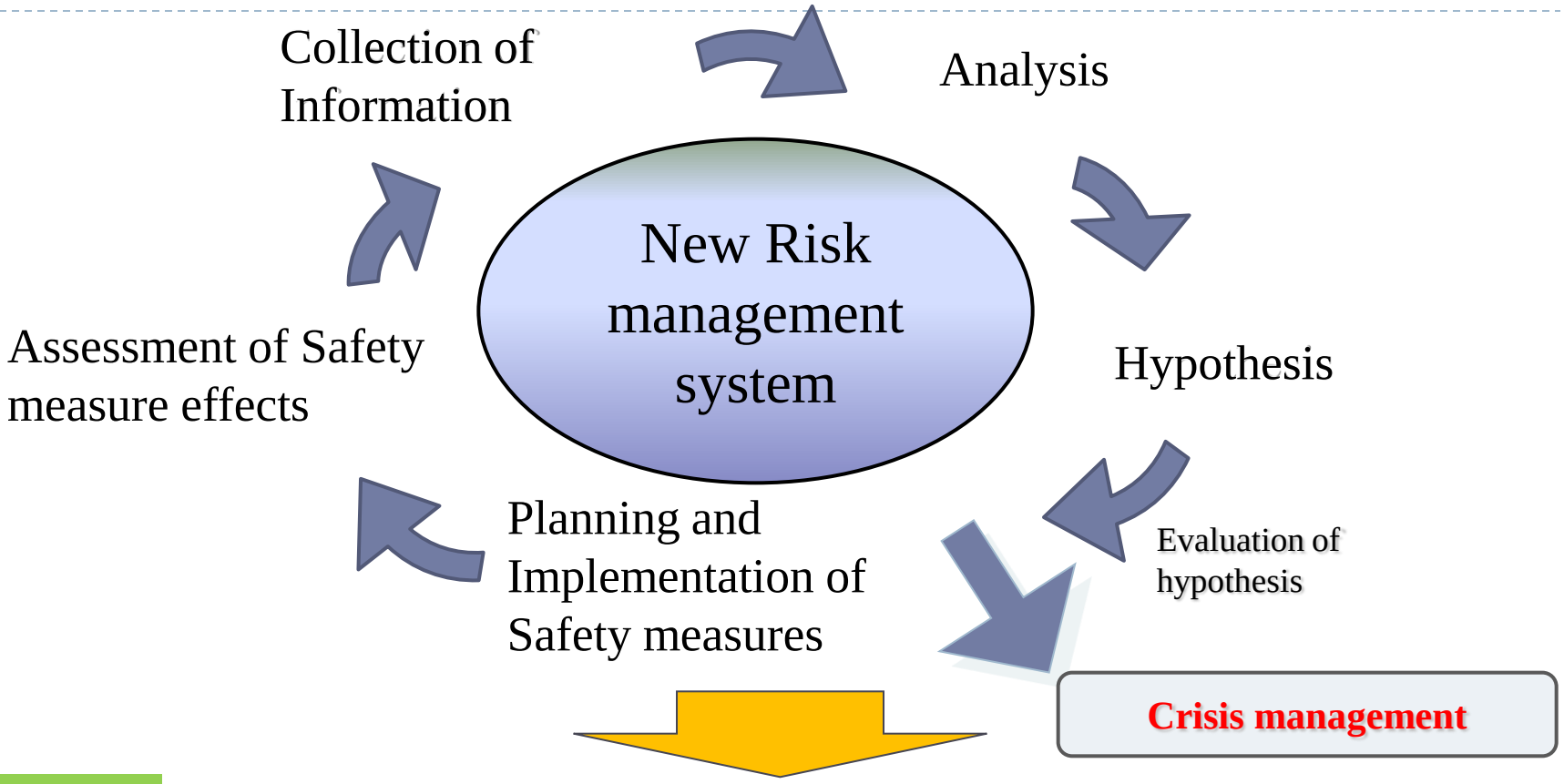
Program of Collaborative Graduate Schools



- PMDA Staffs
  - Engaging on education/research in the university as visiting professor etc.
  - Conducting the research and pursuing Ph.D. as graduate student
- Graduate school students
  - Learning about PMDA's operation in accordance with provided for research guidance and pursuing Ph.D.

Agreement with 17 Universities (as of June, 2013)

Improvement of Safety Measures



Goal

- Prevention of serious drug safety-related crisis from Japan
- Effective encouragement of proper drug use.
- Ensuring credibility to post-market safety management system.

Contents

1. Organization
2. Recent Approaches for Innovative Medicines
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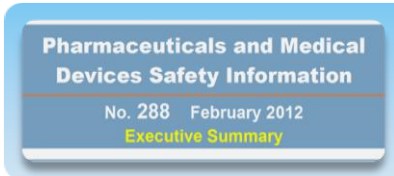
PMDA's International Activities

【PMDA International Vision: PMDA EPOCH】

1. Highest level of Excellence in Performance
2. Close Partnership with the Orient
3. Contribution to International Harmonization

Dissemination of Information

Review Report



Safety Information

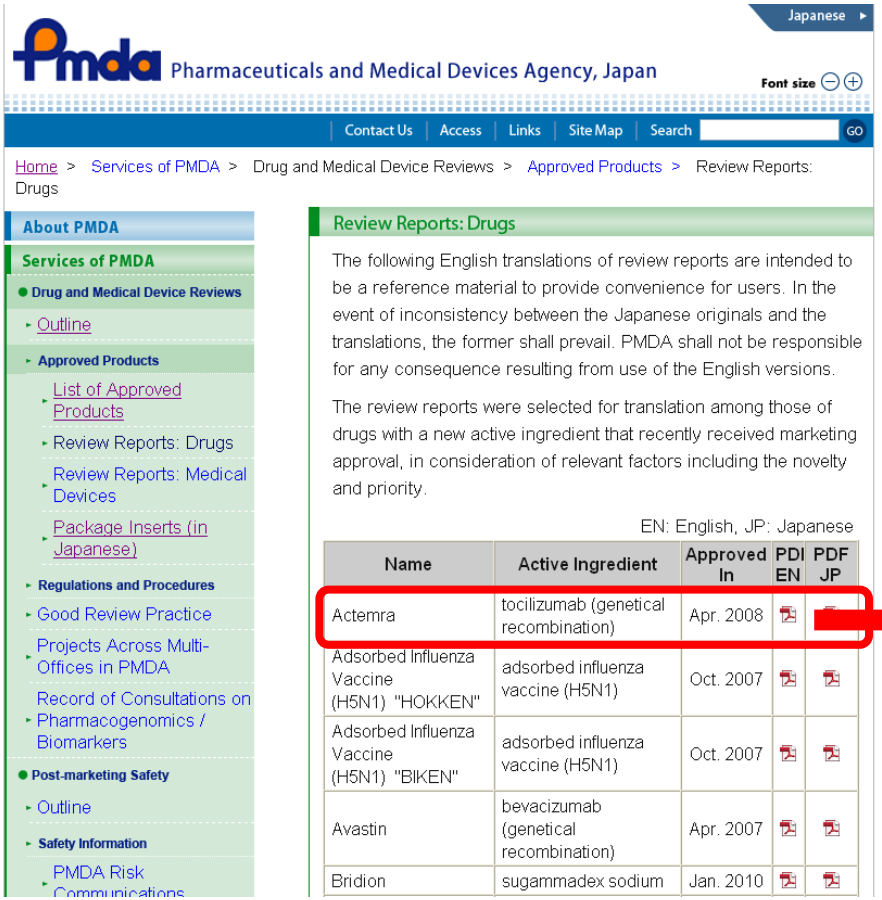
PMDA Updates

News Release

And more…

Review Reports

http://www.pmda.go.jp/english/service/drugs.html



Japanese

Pharmaceuticals and Medical Devices Agency, Japan

Font size

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Home > Services of PMDA > Drug and Medical Device Reviews > Approved Products > Review Reports: Drugs

About PMDA

Services of PMDA

- Drug and Medical Device Reviews
  - Outline
  - Approved Products
    - List of Approved Products
    - Review Reports: Drugs
    - Review Reports: Medical Devices
    - Package Inserts (in Japanese)
  - Regulations and Procedures
  - Good Review Practice
  - Projects Across Multi-Offices in PMDA
  - Record of Consultations on Pharmacogenomics / Biomarkers
- Post-marketing Safety
  - Outline
  - Safety Information
    - PMDA Risk Communications

Review Reports: Drugs

The following English translations of review reports are intended to be a reference material to provide convenience for users. In the event of inconsistency between the Japanese originals and the translations, the former shall prevail. PMDA shall not be responsible for any consequence resulting from use of the English versions.

The review reports were selected for translation among those of drugs with a new active ingredient that recently received marketing approval, in consideration of relevant factors including the novelty and priority.

EN: English, JP: Japanese

Name	Active Ingredient	Approved In	PDF EN	PDF JP
Actemra	tocilizumab (genetical recombination)	Apr. 2008		
Adsorbed Influenza Vaccine (H5N1) "HOKKEN"	adsorbed influenza vaccine (H5N1)	Oct. 2007		
Adsorbed Influenza Vaccine (H5N1) "BIKEN"	adsorbed influenza vaccine (H5N1)	Oct. 2007		
Avastin	bevacizumab (genetical recombination)	Apr. 2007		
Bridion	sugammadex sodium	Jan. 2010		

Review Report

January 22, 2008

Pharmaceuticals and Medical Devices Agency

The results of a regulatory review conducted by the Pharmaceuticals and Medical Devices Agency on the following pharmaceutical products submitted for registration are as follows.

[Brand name]	Actemra 80 mg for Intravenous Infusion Actemra 200 mg for Intravenous Infusion Actemra 400 mg for Intravenous Infusion
[Non-proprietary name]	Tocilizumab (Genetical Recombination)
[Name of applicant]	Chugai Pharmaceutical Co., Ltd.
[Date of application]	April 28, 2006
[Dosage form/Strength]	A concentrate for solution for intravenous infusion containing 80 mg, 200 mg, or 400 mg of Tocilizumab (Genetical Recombination) per vial
[Application classification]	Prescription drug (4) Drugs with new indications, (6) Drugs with new doses, (7) Other drugs

The First Indonesia-Japan Symposium

Date: February 13, 2013

Venue: Jakarta, Indonesia

Focus on: Pharmacovigilance and Good Distribution Practice

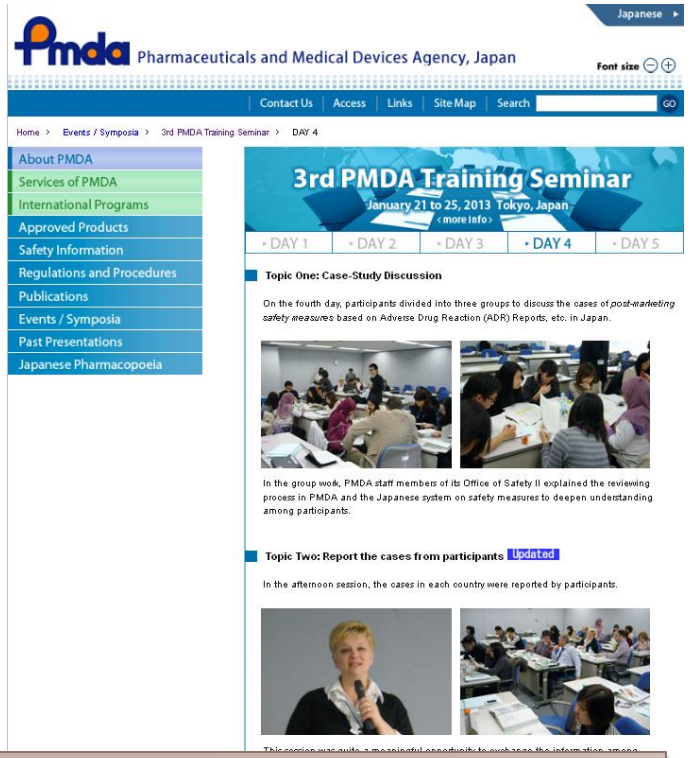
Scheduled Symposia

1st Thailand-Japan Symposium (Oct 24-25, 2013)
2nd Indonesia-Japan Symposium (under planning)

3rd PMDA Training Seminar (Regulators only)

2013 January 21-25: Post-Marketing Safety & Relief Services

Website: http://www.pmda.go.jp/english/events/3rd_pmda_training_seminar.html

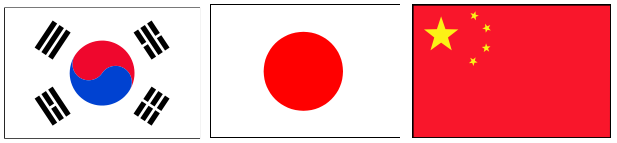


4th PMDA Training Seminar

Date: February 3-7, 2014 Theme: Review of generic drugs

Korea-Japan-China Symposium/Workshop

2007.04 The 1st Korea-Japan-China Minister of Health Meeting (Seoul)
2008.04 The 1st Korea-Japan-China Pharmaceutical affair bureau chief meeting (Tokyo)
The 1st East-Asia Regulatory Symposium
2008.11 The 2nd Korea-Japan-China Minister of Health Meeting (Beijing)
2009.11 The 3rd Korea-Japan-China Minister of Health Meeting (Tokyo)
2009.12 The 2nd Korea-Japan-China Pharmaceutical affair bureau chief meeting
Korea-Japan-China Clinical trial symposium
2010.09 The 3rd Working Group meeting (Seoul)
The 3rd Korea-Japan-China Pharmaceutical affair bureau chief meeting (Tokyo)
APEC-AHC Workshop
2010.09 APEC－LSIF（Sendai, Japan）
2010.11 The 4th Korea-Japan-China Pharmaceutical affair bureau chief meeting (Cheju Island , Korea)
2011.10 The 4th Working Group meeting (Tokyo)
The 4th Korea-Japan-China Pharmaceutical affair bureau chief meeting (Tokyo)
2011.11 Korea-Japan-China MRCT workshop (Tokyo)
2011.11 The 5th Korea-Japan-China Pharmaceutical affair bureau chief meeting (Chingtao, China)



Roadmap for the PMDA International Vision

Five Important Areas Where RMs are needed

1) Response to advanced science and technology

- Proactively provide information about the policies for review and scientific consultation of cutting-edge products and recommendation for relevant guideline developments.
- Introduce progressive analyzing and predictive methods.

2) Improvement of international operation basis

- Improve the organizational structure enabling wide range international activities and cultivate new internationally minded personnel* in a prompt manner.

*A personnel who has 1) good command of foreign languages, 2) an international human network, 3) abundant knowledge of his or her related area of expertise , 4) ability to make appropriate decisions under the given circumstances domestically and internationally, and 5) trustworthy international relations.

3) Dissemination of English information on regulatory review of medicinal products, especially publication of review reports in English

- Increase the number of English version of review reports (aiming to cover all the necessary review reports in English in the future).

4) Dissemination of information and international cooperation on safety measures

- Enhance exchanging information and establish a system to share evaluation reports with our overseas counterparts.
- Enrich the contents related to safely information in the English website.

5) Increase of the leverage of Japanese Pharmacopoeia (JP)

- Publish the newest JP version simultaneously in English and Japanese.
- Enhance cooperative relationship with the USP, EP, WHO and each Asian pharmacopeia.

Website: http://www.pmda.go.jp/english/international/pdf/PMDA_International_Vision/20130527_roadmap.pdf

Road map for the PMDA International Vision

Road map for the PMDA International Vision

1. Introduction

The Pharmaceuticals and Medical Devices Agency (PMDA), a Japanese Incorporated Administrative Agency, established its fundamental strategy on international affairs, "PMDA International Strategic Plan," in 2009 and specified the goals for its Second Mid-term Plan from FY 2009 to 2013. The PMDA International Strategic Plan defines three targets and five basic strategies, and the agency has pursued steady implementation of actual measures to achieve its targets.

[Three targets]

I. Strengthening of cooperation and building of collaborative relations with the United States (US), the European Union (EU), Asian countries, and relevant international organizations.
II. Proactive participation in international harmonization activities and further contributions to such activities
III. Improvement and strengthening of information provision to overseas countries

In November 2011, the PMDA released the "PMDA International Vision" that described concrete goals to be attained in 5-10 years. In the vision, the agency identified itself as one of the world's premier medical products regulatory agencies comparable to its American and European counterparts, and set three goals that it was committed to realizing.

(1) Secure the highest level of excellence in performance
(2) Maintain a close partnership with the orient
(3) Actively contribute to international harmonization

http://www.pmda.go.jp/english/international/pdf/PMDA_International_Vision/20130527_roadmap.pdf

Thank you for your attention !

http://www.pmda.go.jp/