

# Japan PMDA and CDISC Standards

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*Strength through Collaboration*

# Outline

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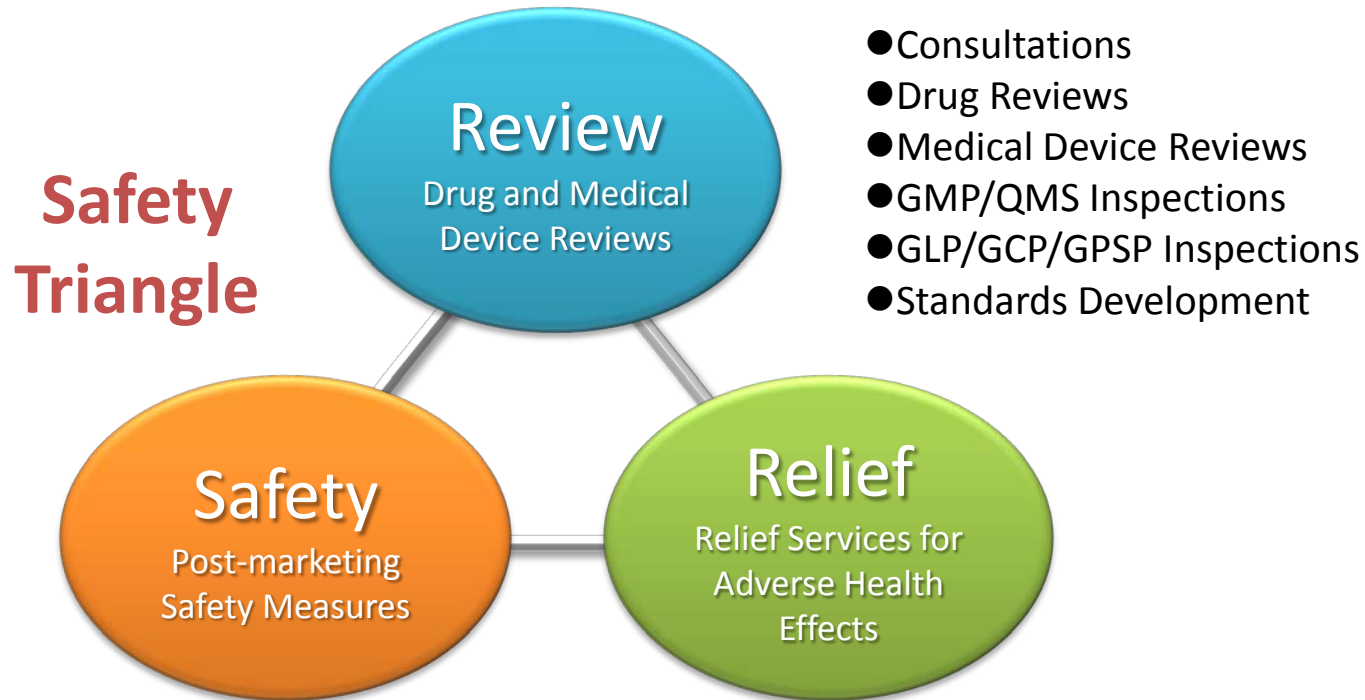
- New drug review process in Japan
- Task force for advanced review and consultation with electronic data
- PMDA and CDISC standards

# What we do in PMDA

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- **PMDA** (Pharmaceuticals and Medical Devices Agency), established in 2004, is **Japanese regulatory agency**, working together with Ministry of Health, Labour and Welfare.
- Our obligation is to protect the public health by **assuring safety, efficacy and quality of pharmaceuticals and medical devices**.

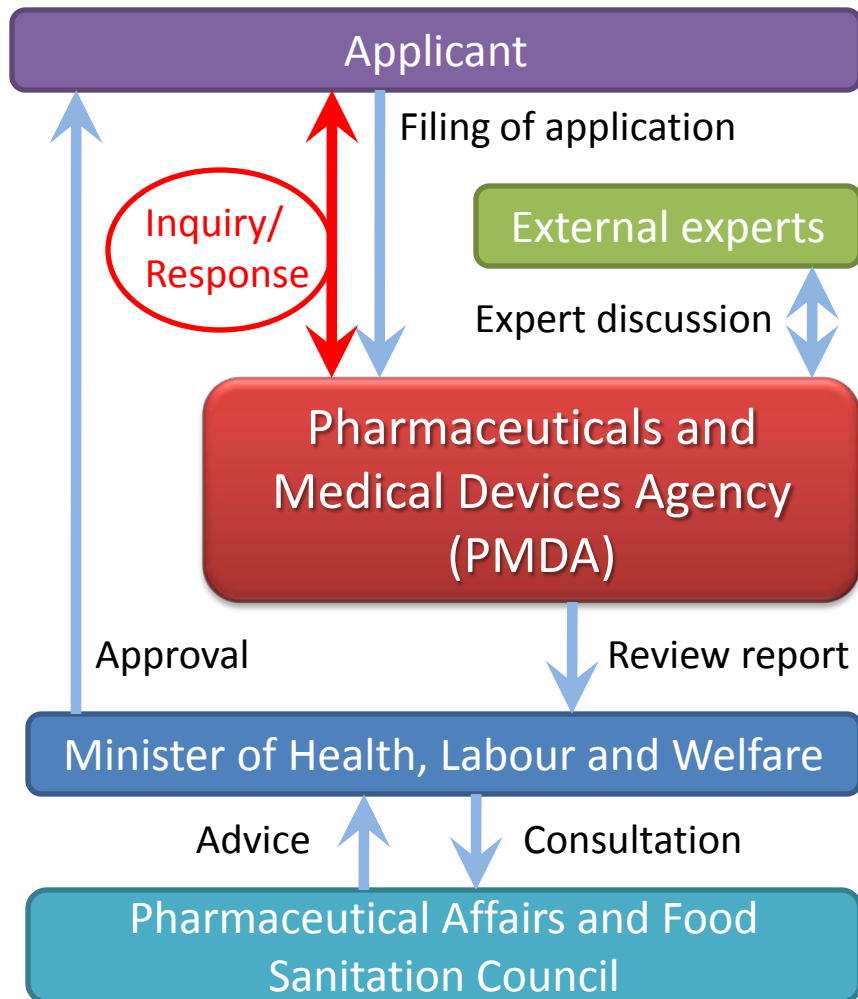
# What we do in PMDA



- Information Collection/Organization
- Research and Analyses
- Consultations
- Information Services

- Relief for Adverse Drug Reactions
- Relief for Infections Acquired through Biological Products
- Health Allowances for SMON Patients
- Health Allowances for HIV-Positive and AIDS Patients
- Relief for Individuals Affected by Hepatitis C through Specified Products

# New drug review process in Japan



- So far, patient level clinical trial data are not required in new drug application in Japan.
- Since PMDA does not have data to analyze, applicants must re-analyze the data to answer the inquiries from PMDA during the new drug review.
- Exchange of the inquiries and responses may be very frequent.

# Japan Revitalization Strategy -JAPAN is BACK-

Cabinet decision; June 14, 2013

## Three action plans

- Industry Revitalization Plan
- Strategic Market Creation Plan
  - Theme 1: Extending the nation's "healthy life expectancy"
- Strategy of Global Outreach



## Envisioned Society:

The society where people are able to live a healthy life and get old by enhancing effective preventive care services and health management  
**Providing the world's most advanced necessary medical care by activating medicine-related industries**

## Specific Strategy

## Strengthening the PMDA

The assessment lag "0" for pharmaceuticals and medical devices by accelerating the assessment process **by strengthening the PMDA system**

# Health and Medical Care Strategy

Agreement of Chief Cabinet Secretary, Minister of Health, Labour and Welfare and other concerned Ministers; June 14, 2013

## Three Basic Plan

- Achievement of a healthy, long-lived society
- Contribution to economic growth
- Global contribution

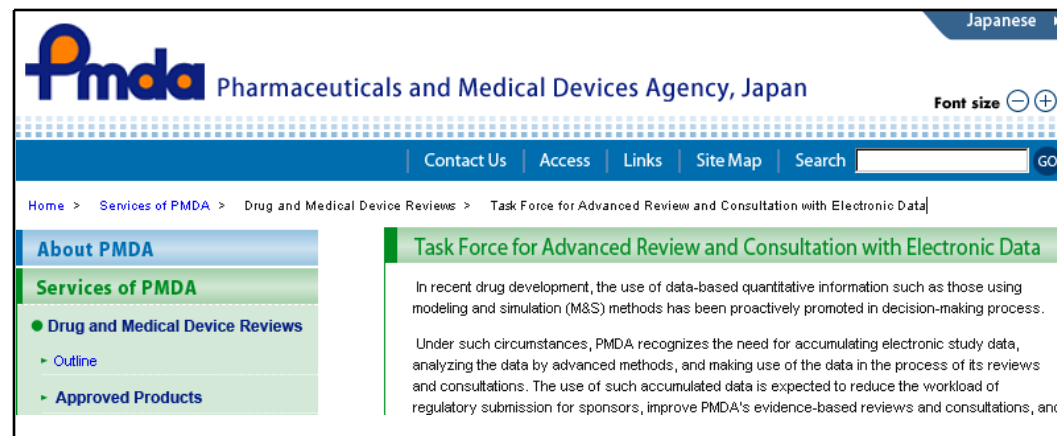
Specific  
Strategy

## Strengthening the PMDA

- System enhancement for the Pharmaceutical Affairs Consultation on R&D Strategy
- Organizing and enhancing the consultation service in close coordination with the Drug Discovery Support Network
- PMDA-initiated promotion of research and analysis based on clinical data
- Increase of the quantity and quality of the large-scale medical information database for early achievement of the 10-million data set
- Identification of an appropriate financial base for the PMDA's tasks and necessary measures

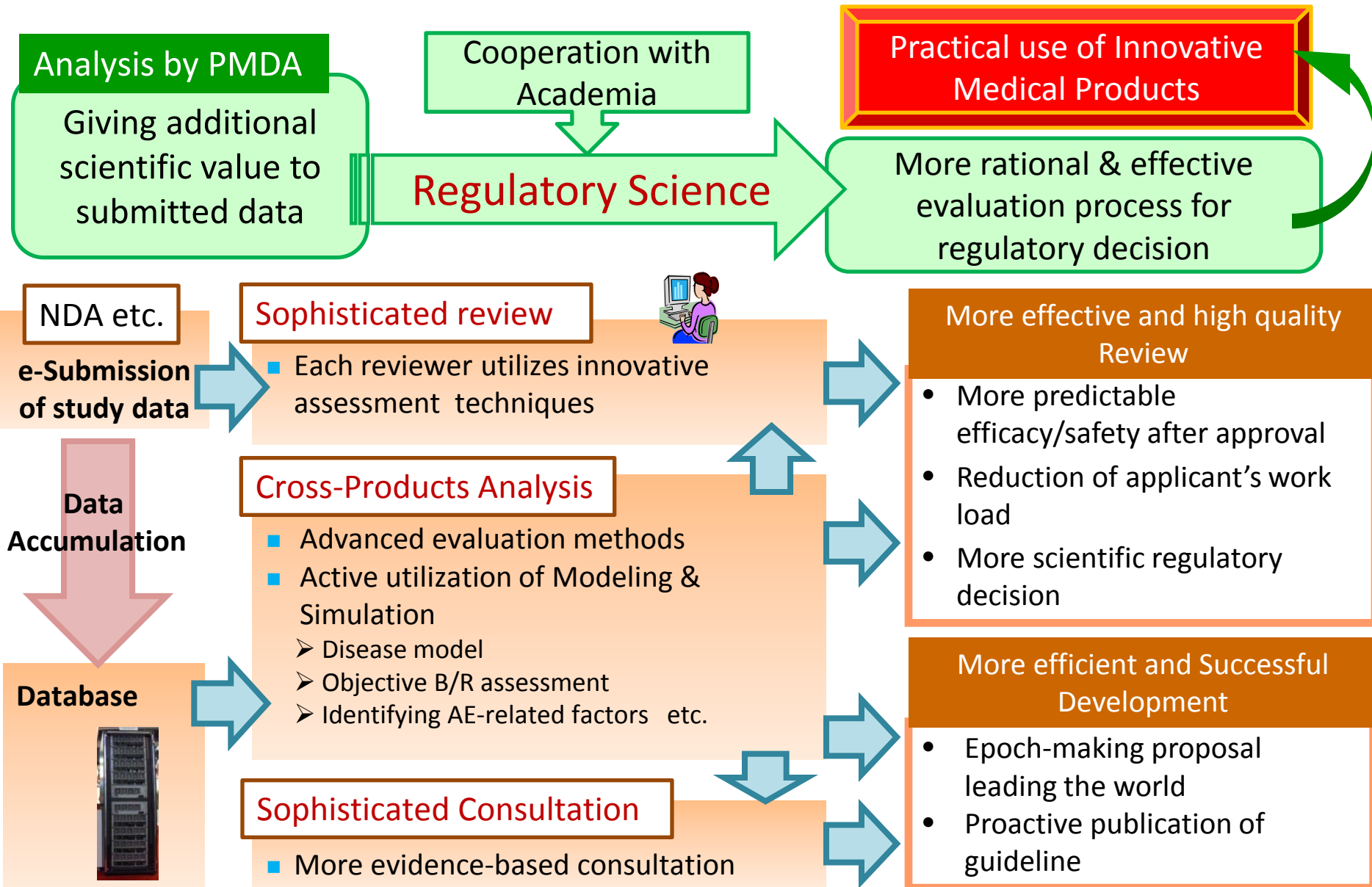
# Task force for advanced review/consultation

- PMDA started a discussion in the view of mandating electronic submissions in the future, and internally established "Task force for advanced review and consultation with electronic data" on Sept 1st, 2013
  - <http://www.pmda.go.jp/english/service/taskforce.html>





# Advanced workflow of review/consultation



# Accumulation and Utilization of Data

## NDA submission

### e-Submission of data

- ◆ Submission of electronic data from clinical and nonclinical studies

Storage of electronic data in the dedicated server and registration in the database



Visualization and analysis of data, supported by browsing software

## Regulatory Review

### Use of electronic data

- ◆ Accessible, visualized electronic data for each reviewer
- ◆ Easy to identify individual clinical case data, drilling down of data
- ◆ Operation of various analyses - simple, subgroup analysis for the present



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Scientific discussion and decision making on the basis of internal analysis result

## Utilization of Accumulated Data

### Integration of cross-products information

- ◆ Utilization of exhaustive information by therapeutic category for review/consultation
- ◆ Internal review on particular theme – e.g.) active utilization of Modeling & Simulation
  - Review on pediatric dosage
  - Preparation of disease model
  - Development of evaluation indicator
- ◆ Utilization in preparation of guideline



What the review authority can do with the information of all products.

Contribution to efficient development through review/consultation and GL publication based on further analyses by dry-lab

# Proposed timeline for electronic study data system development

TENTATIVE  
Currently under discussion

- FY2013
  - Surveys, procurement of hardware/software, test run\*
- FY2014 to FY2015
  - Continue the test run\*; to be in full operation after the Lab is open
- FY2016 (prospect)
  - Submission of clinical electronic data for NDA
  - (With transitional period)
- After FY2017
  - Submission of non-clinical electronic data for NDA
  - (To be discussed)

\*Electronic data viewing and internal analysis

# Future goals

- Guideline development
- Increase of development success rate
- Making proposals leading the world

↓  
Increased development efficiency  
(Shortened development time, cost reduction)



**PMDA analysis**  
→ Additional value to study data

**Regulatory science = Science of prediction and analysis**

**Development of practical and efficient evaluation/decision process**

- Cooperation with academia?

- Increased predictability of efficacy and safety
- Decreased burden on applicants
- More scientific discussion and decision making

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Increased efficiency and improved quality of review

Development of Japan's original innovative drugs and medical devices

# Importance of global data standards

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- PMDA
  - For fast access and easy handling of submitted clinical trial data in many new drug applications
  - For future use of accumulated clinical trial data for cross-products analysis
- Industry/Sponsor
  - For efficient and qualified process to make submission materials
  - For efficient use of medical records of medical institutes for clinical trials
  - For promotion of participating global development and using Japanese clinical trial data for submission to foreign regulatory authorities

PMDA plans to request patient level clinical trial data in electronic format which complies **CDISC standards**

# FY2013 pilot project (outline)

- Purpose
  - To confirm that the clinical data submitted as a part of approval application for new drugs is appropriately stored and managed with in-house system, and that persons in charge can analyze the stored data by utilizing introduced software.
- Data to be used
  - Clinical data including those of Japanese subjects, which was amassed **according to the CDISC standards**, and are under regulatory review or going to be filed to PMDA (more than 1 clinical study per 1 product, around 3 products)

Through the pilot project, PMDA will confirm the feasibility of reviewing and analyzing data in CDISC formats, using actual data from clinical trials.

# Relationship between PMDA and CDISC

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- PMDA regularly attend the meetings of CDISC in Japan as observer since July 2013.
  - Japan CDISC Coordinating Committee (J3C)
  - CDISC Japan Users Group (CJUG) SDTM, ADaM
- Contribution of CJUG members to the introductory lecture of CDISC in PMDA
- Presentation by PMDA in International Interchange and Japan Interchange

# Summary

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- The advanced review/consultation with electronic data will accelerate review process and reduce the burden on applicants.
- Cross-product investigation of accumulated study data by PMDA will result in more qualified review/consultation and efficient drug development in the future.
- Standardization of trial data is of great importance for reviewing new drugs and future cross-product investigation.
- PMDA plans to accept CDISC compliant data in new drug application for efficient and high-quality new drug review. As a results, this may lead to standardization of study data and efficient drug development in Japan.
- PMDA would like to keep a good relationship with CDISC to promote the standardization of study data as well as conduct of study in Japan.



# Thank you for your attention

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- PMDA Homepage
  - <http://www.pmda.go.jp/english/index.html>
- Drug and Medical Device Reviews
  - [http://www.pmda.go.jp/english/service/outline\\_s.html](http://www.pmda.go.jp/english/service/outline_s.html)
- “Task force for advanced review and consultation with electronic data” Homepage
  - <http://www.pmda.go.jp/english/service/taskforce.html>