DIA 2013
49th Annual Meeting

PMDA's Efforts in Medicinal Area

Takao Yamori, Ph.D.
Director of Center for Product Evaluation and
Deputy Director General of the Office of Review Innovation
Pharmaceuticals and Medical Devices Agency

Disclaimer

- The views and opinions expressed in the following PowerPoint slides are those of the individual presenter and should not be attributed to Drug Information Association, Inc. ("DIA"), its directors, officers, employees, volunteers, members, chapters, councils, Communities (formerly known as SIACs) or affiliates, or any organization with which the presenter is employed or affiliated.
-

Today's Presentation

1. Introduction: Products from Japan
2. Current Status of Pharmaceutical Affairs Consultation on R&D Strategy
3. Establishment of the Science Board

Today's Presentation

1. Introduction: Products from Japan
2. Current Status of Pharmaceutical Affairs Consultation on R&D Strategy
3. Establishment of the Science Board

Innovative Medicinal seeds from Academia in Japan

ACTEMRA® Injection (Tocilizumab (r-INN))

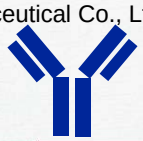
Approved in JAPAN; April 2005
(First marketing authorization)

- Target Identification / Target Validation

Professor Tadamitsu Kishimoto (Osaka University, Japan) discovered IL-6 as the pathogenic factor of Castleman's disease (Blood 1989; 74:1360-1367)

- Extensive research & Development

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



Innovative Medicinal seeds from Academia in Japan

XALKORI® capsules (Crizotinib(INN))

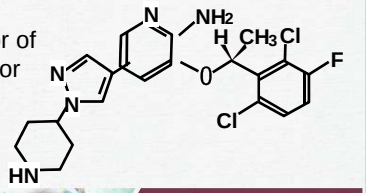
Approved in JAPAN; May 2012
(International Birth Date: Aug. 2011)

- Target Identification / Target Validation

Professor Hiroyuki Mano (Jichi Medical University, Japan) discovered EML4-ALK fusion oncogene in non-small-cell-lung cancer (Nature 2007; 448:561-6 etc.,)

- Extensive research & Development

XALKORI ® (Crizotinib) is the ATP competitive inhibitor of tyrosine kinase of the Hepatocyte growth factor receptor developed by Pfizer Inc.



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

TOKYO SKY TREE

Completion : February 2012
Height : 634m

Today's Presentation

1. Introduction: Products from Japan
2. Current Status of Pharmaceutical Affairs Consultation on R&D Strategy
3. Establishment of the Science Board

Pharmaceutical Affairs Consultation on R&D Strategy

Valley of Death

-Shortage of funds, Knowledge on Regulation and developmental strategy

Pmda 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

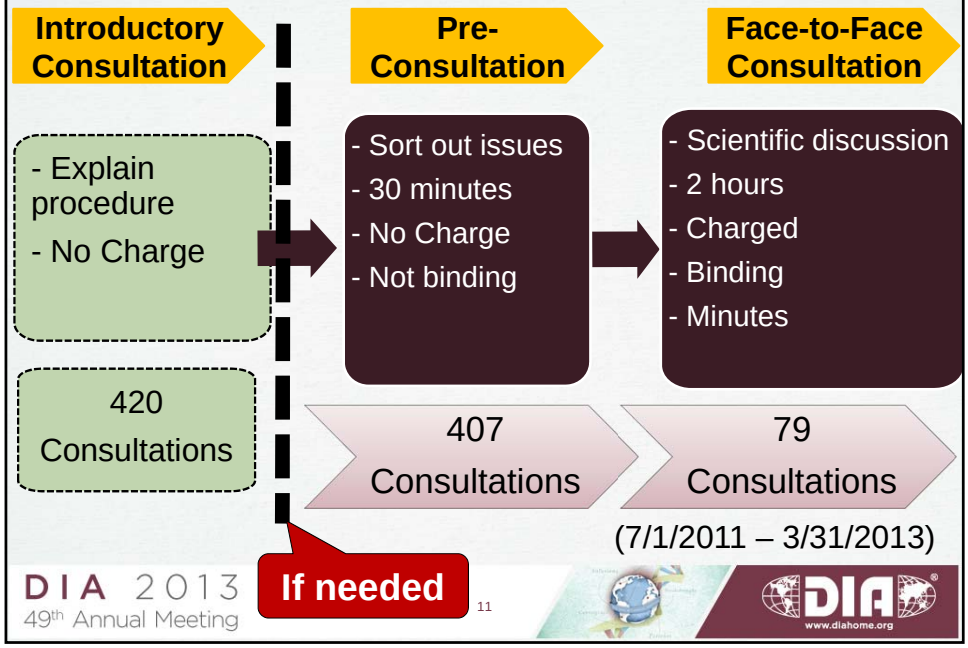
Innovative Products

* Further studies are handled by the Regular Consultation

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

Consultation on endpoints or sample size of early clinical trial

Flow of R&D Strategy Consultation



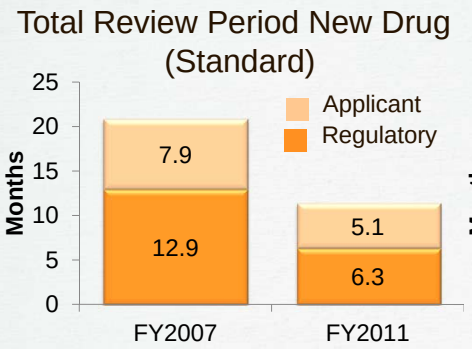
Case of Face to Face consultation

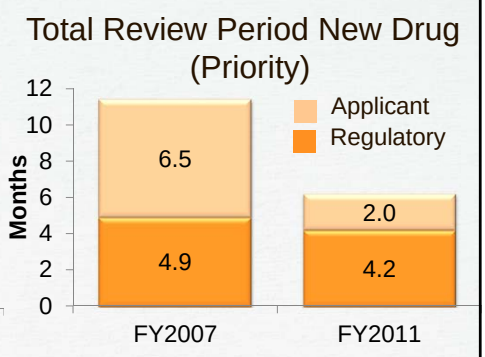
Consulter	Product under development	Intended performance, Intended use, Indications
National Institute of Neuroscience, NCNP Department of Molecular Therapy Shin'ich Takeda	Morpholino oligos (Antisense)	Remedy for Duchenne muscular dystrophy (DMD)
Molecular Medicine and Therapy, Medicine (ART), Tohoku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

Today's Presentation

1. Introduction: Products from Japan
2. Current Status of Pharmaceutical Affairs Consultation on R&D Strategy
3. Establishment of the Science Board

Environments surrounding PMDA





- Achieved our targets
- What are the strategies and approaches PMDA could use to integrate the ever advancing technologies?

Issues of PMDA

① Conducting review and consultation understanding of the research activities in state-of-the-art technologies,

② Conducting review and consultation in the state of the art technologies from early stage of development,

③ Training reviewers to catch up on the accelerating innovative technologies and contributing in the establishment of practical use of state of the art technologies.

Science Board

For PMDA To Be More Science-Based

Basic Research

Seeds of new drug / medical devices

Quality Tests → Non-clinical tests → Clinical Trial → Review → Approve → Post Marketing

Practical use

Innovative medical products

Pharmaceutical consultation on R&D Strategy | Clinical Trial Consultation | Review | Post Marketing Safety Measure

Offices of Review (Drugs & Medical Devices), Office of Safety

Office of Review Innovation

Establishment of the Science Board

The Science Board was established in May 2012 to discuss how PMDA can better cope with products with advanced science & technology, in each developmental stage such as basic research, development support, product review, and post market safety measures.

Board members

Academia

Science Board and Office of Review Innovation

Office of Review Innovation

Director General

Secretariat Director | Associate Director General

Mission

Reform PMDA review system and related services based on science with consideration of actual medical practices

PMDA Office

Review/ Audit/ Inspection

RS | Safety | SGD

RS: Office of Regulatory Science
SGD: Office of Standards and Guidelines Development

Science Board

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

Committee

- Recommendation on PMDA tasks
- Improvements in the scientific aspects of review

Subcommittee

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

Pharmaceuticals | Medical Devices | Bio-based Products | Cellular & Tissue-based products

Projects Across Multi-Offices in PMDA

Main Roles of Science Board

- Pick out Issue
- Coach for Issue

Main Roles of Science Board
- Pick out Issue -

Committee

- Picking out Science & Technology which could be potentially applied to innovative products(Drug/Medical Device) in the near future among the Cutting-edge of Exploratory Research,
- On the advanced scientific & technology, Requesting the subcommittee to discuss further for providing review and consultation services appropriately in PMDA.

Request

Subcommittee

On the subject of advanced scientific technology, profound researchers and the PMDA reviewers deliberate assessment tools in order to provide review and consultation services appropriately.

Exchange Opinions

Review Offices

Study meetings with profound researchers

Main Roles of Science Board
- Coach for Issue -

Committee

- Discussion on the proposed subjects
- Requesting adequate subcommittee to discuss further for the issues.

Review Offices

- Problem consciousnesses in PMDA
- Anxious to exchange views on subjects with the Science Committee.

Propose

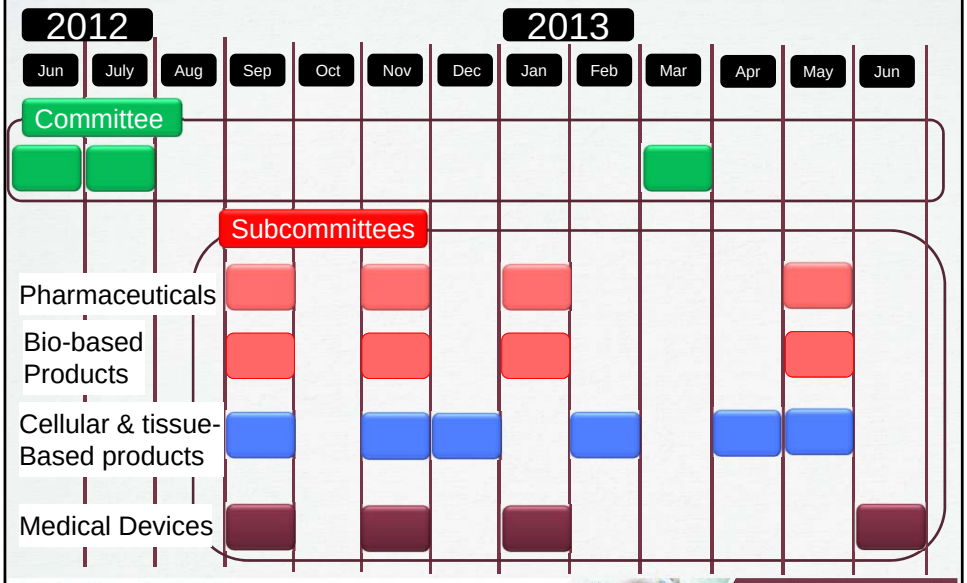
Request

Subcommittee

Profound researchers and the PMDA reviewers deliberate the referred issues from the Science Committee.

Exchange Opinions

Past Activities



Outcome of Science Board

- A report on a summary of discussion by the subcommittee for each subject
- An annual report

Schedule of Science Board (Committee)

Aug 20, 2013	FY2013	•Report from Subcommittee
The end of 2013		•Report from Subcommittee
Around Mar 2014		•Report from Subcommittee •Annual report

Possible issue on each Subcommittee

➢ Pharmaceuticals & Bio-products

- Non-clinical pharmacology studies of anticancer drugs
- Personalized medicine
  - Biomarkers

➢ Cellular & Tissue- based products

- The subject on securing quality and safety of cellular and tissue-based products.
  - Tumorigenicity
  - Requirements of Cell Processing Center (CPC)

➢ Medical Devices

- Challenges to develop registry
- Scope of the follow-on medical devices
- View on developing combination products

Summary

- *Pharmaceutical Affairs Consultation on R&D Strategy* is offering consultation for innovative products developed by academia/venture businesses.

- Science board was established for review/consultation in PMDA to become more science based.

Pharmaceuticals and Medical Devices Agency

Thank you for your attention !

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