Introduction of Ministry of Health, Labour and Welfare (MHLW) / Pharmaceuticals and Medical Devices Agency (PMDA) and recent updates in Japan

2nd meeting of Commission Expert Group on
Safe and Timely Access to Medicines for Patients (STAMP)
6 May 2015 in Brussels

Presented by Yoshihiko Sano,
MHLW/PMDA Liaison official stationed at EMA

About MHLW/PMDA

Ministry of Health, Labour and Welfare

Planning basic policy, enforcement of administrative measures based on law

- Marketing authorization of pharmaceutical and medical devices
- Issue emergency safety information and direct product withdrawal
- Safety measures for emergent and significant cases

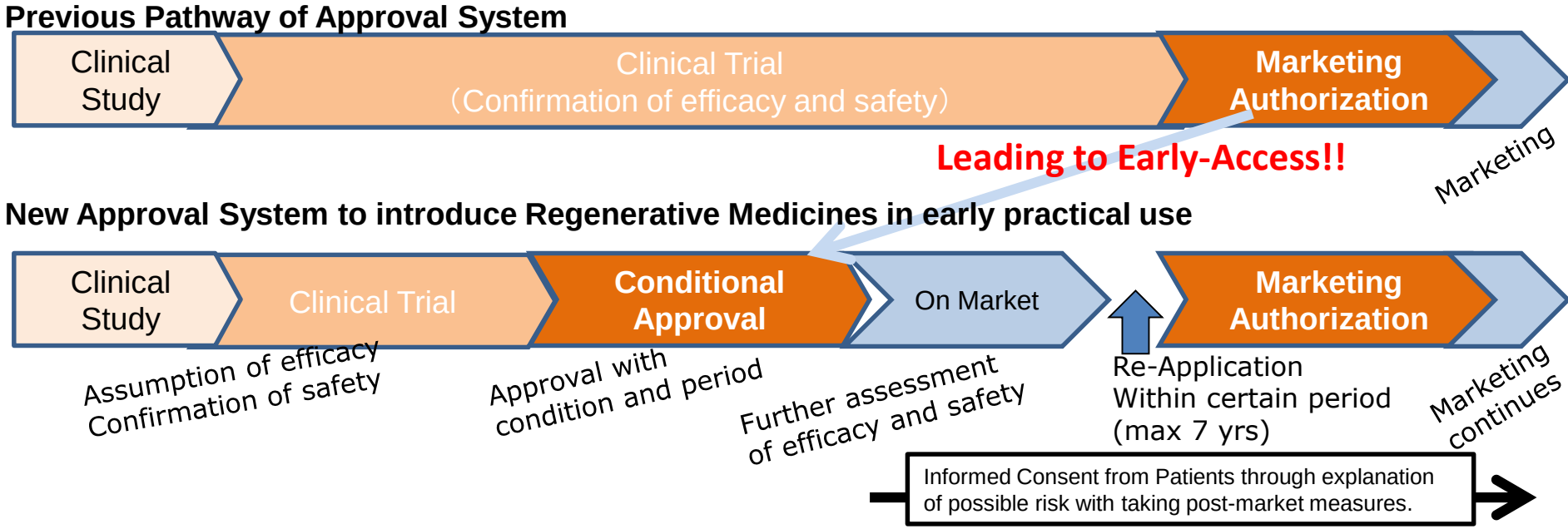
Pharmaceuticals and Medical Devices Agency

Review, examination and data analysis Three-pillar system: Review/Safety/Relief

- Scientific review; GMP/GLP/GCP inspection; Consultation on the development of pharmaceuticals and medical devices for marketing authorization
- Collection, analysis and dissemination of information relating to quality, efficacy and safety of pharmaceuticals and medical devices

Act on Pharmaceuticals and Medical Devices

- Enacted on 25 November 2014
- Revisions of drugs/medical devices articles and addition for regenerative medical products
- Expedited approval system to introduce conditional/term-limited authorization for regenerative medicines



Strategy of SAKIGAKE as a Package

～Lead the world in the practical application of innovative medical products～

Promote the strategy package facilitating all the process from R&D, clinical research/trials, pre- and post- marketing safety, insurance coverage, through globalization of innovative products which are to be put into practical use earlier in Japan. Specifically, this package is targeting innovative pharmaceuticals/medical devices/regenerative medicine which can cure serious illnesses (such as rare diseases/cancer etc.) unless established therapy is available.

Prioritized Policy I — ***SAKIGAKE***

Prioritized Policy II — **Scheme to rapid authorization of unapproved drug**

Pre-Clinical Research → **Clinical Research /Trial** → **Approval** → **NHI* Price Listing** (*:National Health Insurance) → **facilitate the environment for industry activities** → **International Deployment**

Accelerate R&D through supporting each stage

- Coalition between “Network for Drug Discovery” and “Pharmaceutical Affairs Consultation on Research and Development (R&D) Strategy”
- Support of Drug-Repositioning (DR) and development of off-label use
- Development of safety assessment technique for using iPS derived cells followed by international standardization
- R&D through public-private joint project

- High-quality clinical trials by Clinical Trial Core Hospital・ NC and coalition with research group for rare diseases
- Support for orphan drug R&D
  Support for ultra-orphan through the R&D to Early designation
- Support for Drug Development through Medical Information and Communication Technology (MICT)
  - ・ DB of Medical Information
  - ・ Rapid and effective Clinical Trials
  - ・ Incorporation into review for approval

- Analysis on Modeling and Simulation (M&S) conducted by PMDA
- Utilizing Pre-application Consultation
- Strengthening measures on post-marketing safety
  - ・ Development of system of patient registry
  - ・ Research on biomarker

- Improve the predictability of NHI drug price
  - ・ Discussion on Premium to promote the development of new drugs and to eliminate off-label use

- Strengthening industry competitiveness
  - ・ tax incentive
  - ・ HR Development
- Support for SME and venture
  - ・ Discussion on funding system for review user fee to be implemented
- Utilization of the data from clinical research of rare disease / cancer for post-marketing surveillance

- Mutual understanding of the process from R&D to approval with the trading partner, to promote export

Strengthen the structure of PMDA (consultation, review, safety measures in terms of quality and quantity)

Promotion of Regulatory Science (Developing guidelines/assessment for the state-of-the-art technology)

Designation as *Sakigake*

Designation as *SAKIGAKE* plans to achieve realization of promote development of innovative medicines/medical devices/regenerative medicines in Japan.

Designation Criteria

1. Early development and application in Japan prior to other countries (including simultaneous applications)
2. Prominent effectiveness (i.e. radical improvement compared to existing therapy) can be expected

Designation Advantage

: To shorten the time to approval　: To facilitate R&D

①Prioritized Consultation
Waiting time:
2 → 1 month

② Substantial Pre-application Consultation
De facto review before application

③Prioritized Review
12 → 6 months

④Review Partner
PMDA manager
as a concierge

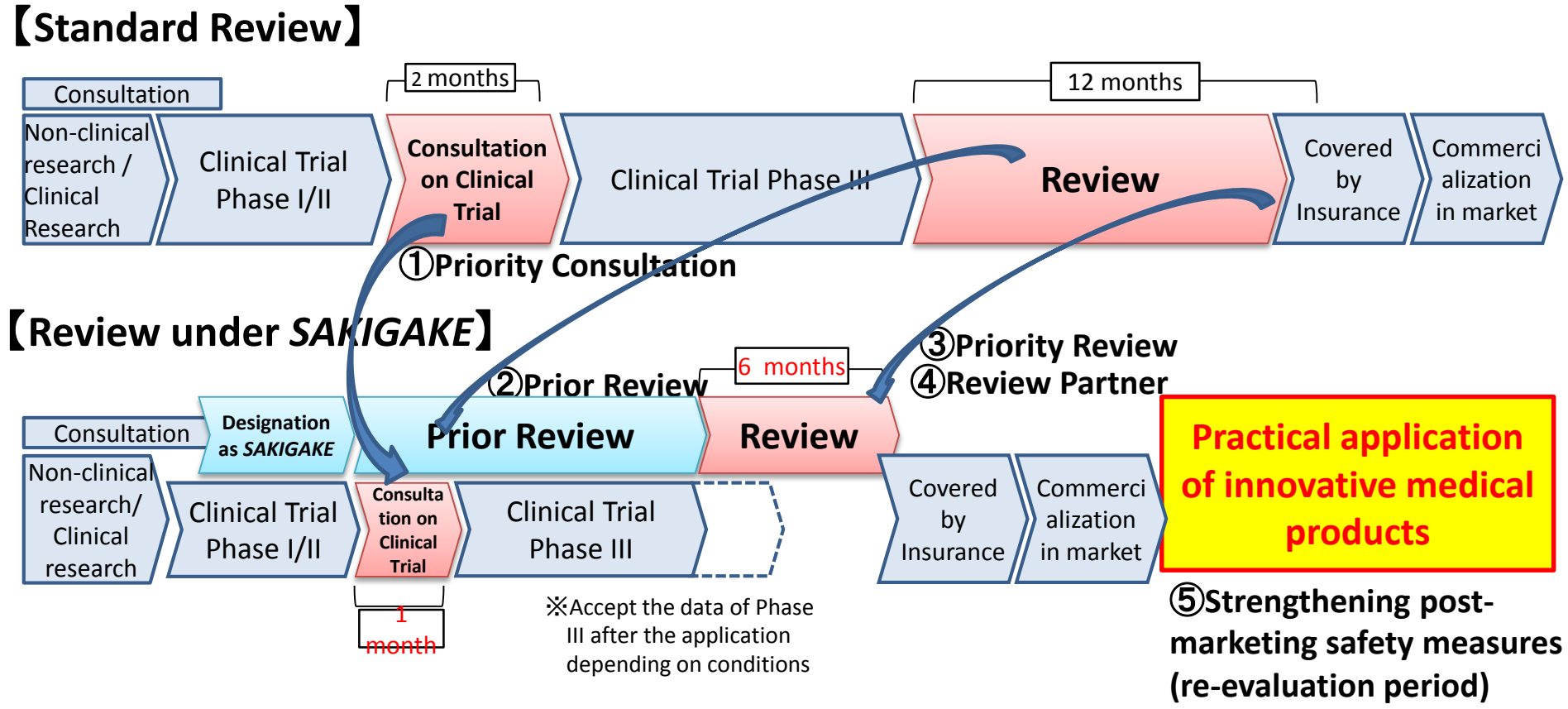
⑤Substantial Post-Marketing Safety Measures
Extension of re-examination period

Designation Procedure

1. **Initiation by applicant:** After PMDA's evaluation, notified within 60 days.
2. **Initiation by MHLW:** When MAH agrees with proposal of MHLW's designation, through PMDA's evaluation, notified within 30 days.

(Ref) Full version of strategy of *Sakigake* as a package: http://www.mhlw.go.jp/english/policy/health-medical/pharmaceuticals/140729-01.html

General Timeframe of *SAKIGAKE*



Trial implementation of *Sakigake* review designation system

Tentative translation (extracted), PFSB/ELD Notification No 0401-6, dated on 1st April 2015

- **Requirements for designation: all four requirements needs satisfied**

(1) Medicine's Innovativeness

In principle, it needs innovativeness with new functional mechanism. (Even for the same functional mechanism as already approved medicines, if it has a new indication for its target disease or innovative DDS system etc. and is expected to improve efficacy drastically, it can be included.)

(2) Seriousness of target diseases

Target diseases are to be applied either following conditions: life-threatening serious diseases or symptoms (difficulty in normal social life) continue due to no medication for complete cure.

(3) Extremely high efficacy for target diseases

There is no approved medicine with same indication or drastic improvement in efficacy is expected comparing to existing medicines or therapies (including drastic improvement in safety).

(4) Inclination to early development and application in Japan prior to other countries

Applicants should emphasize development of their candidate medicines from early stage in Japan and plan to submit their applications prior to other countries (including simultaneous applications). In addition, it is desirable to be confirmed their development in Japan is steadily promoted based on either or both of following conditions:

- First in Human (FIH) test is conducted in Japan
- Proof Of Concept (POC) test is conducted in Japan

- **Procedure**

Public offering to MHLW (8 -29 May); Hearing (15 June to 17 July); Submission of application (7 to 21 August)

(Note) It is a tentative translation and authorized translation provided later is to be prioritized.

Danke schön!

Muchas gracias

Merci beaucoup

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- MHLW English website: http://www.mhlw.go.jp/english/index.html
- PMDA English website: http://www.pmda.go.jp/english/index.html