PMDA Update

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Pharmaceuticals and Medical Devices Agency (PMDA), Japan

2nd Taiwan-Japan Conference

October 31st

Agenda

- Recent trend in medical policy in Japan
- Promotion of Regulatory Science at PMDA
- Activities associated with the revision of Pharmaceutical Affairs Act
- 3rd mid-term plan of PMDA
- Conclusion

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Barrier(Valley of Death) for Practical Application

Basic Research — Discovery of target and Seeds
Applied Research — Exploratory Research ⇨ Optimization Research
Non-clinical Study — Quality/Pharmacology Toxicology/PK
Clinical Trial — Phase I-III

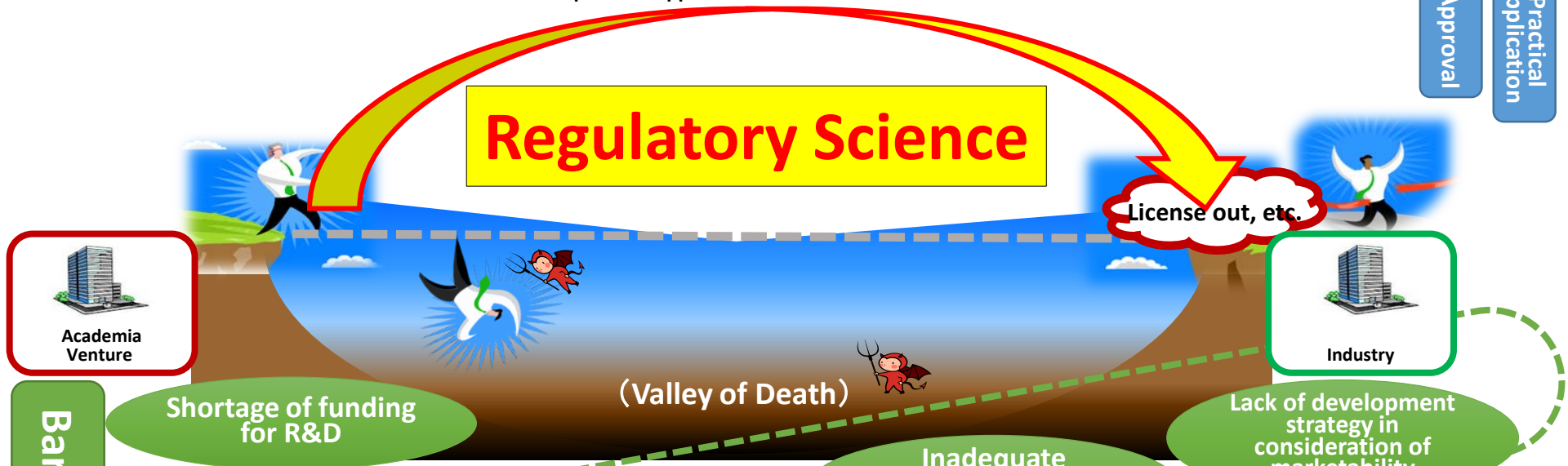
Discovering logic/knowledge on disease

Research towards practical application

Approval

Practical Application

Regulatory Science



Barrier

- Shortage of funding for R&D
- Lack of human resource
- Lack of intellectual property strategy
- Inadequate understanding of regulation
- Lack of development strategy in consideration of marketability

Need studies and manufacturing processes in compliance with Pharmaceutical Affairs Act and related standards.

Approach(e.g.)

- Translational Research Network Program
- Network to support drug discovery
- Promotion Program for Practical Use of Innovative Pharmaceuticals, Medical Devices, and Regenerative Medicines
- Pharmaceutical Affairs Consultation on R&D Strategy

Japan Revitalization Strategy - JAPAN is BACK -

（Cabinet Decision on June 14, 2013）

Extended national “Healthy life expectancy”

<Future vision of the society>
The society where people can receive necessary healthcare services at the most advanced level in the world

Foster the industry specialized in extended healthy life, by developing innovative pharmaceuticals, medical devices and regenerative medicines first in the world, and by introducing these products to the market through speedy review process.

Measures

- Strengthen PMDA organization both in size and in quality
While maintaining a keen attention to post marketing product quality and safety, further reduction of review time (achieve “0” review lag) and improved quality will be pursued.

- **Establish control tower function (Japanese version of NIH)**
  Build systems where integrated research management, bridge between research and clinical practice, and world-class, high-quality clinical research/trial will be securely carried out.

  *Reform of regulation and system to accelerate generative medicine research and environmental improvement for practical use of regenerative medicine products are required.

Health and Medical Strategy

(Agreed by Chief Cabinet Secretary, Minister of Health, Labour and Welfare and other related Ministers on June 14, 2013)

Basic philosophy (Three philosophies)

- Realize extended healthy life society
- Contribute to economical growth
- Contribute to the world

Measures

- **Establish organization to promote research and development**
  ~ Prepare "All Japan" support system ~
- Establish Japanese version of NIH
- Streamline and enhance the consultation program, in close cooperation with drug discovery support network
- **Strengthen PMDA**
- Expand and enhance the pharmaceutical affairs consultation on R&D strategy program **~ Provide better perspective for development, promoting speedy implementation ~**
- Promote PMDA's own analysis and study of clinical data, etc.

※Development of the security evaluation system using iPS cells is required for better new drug development

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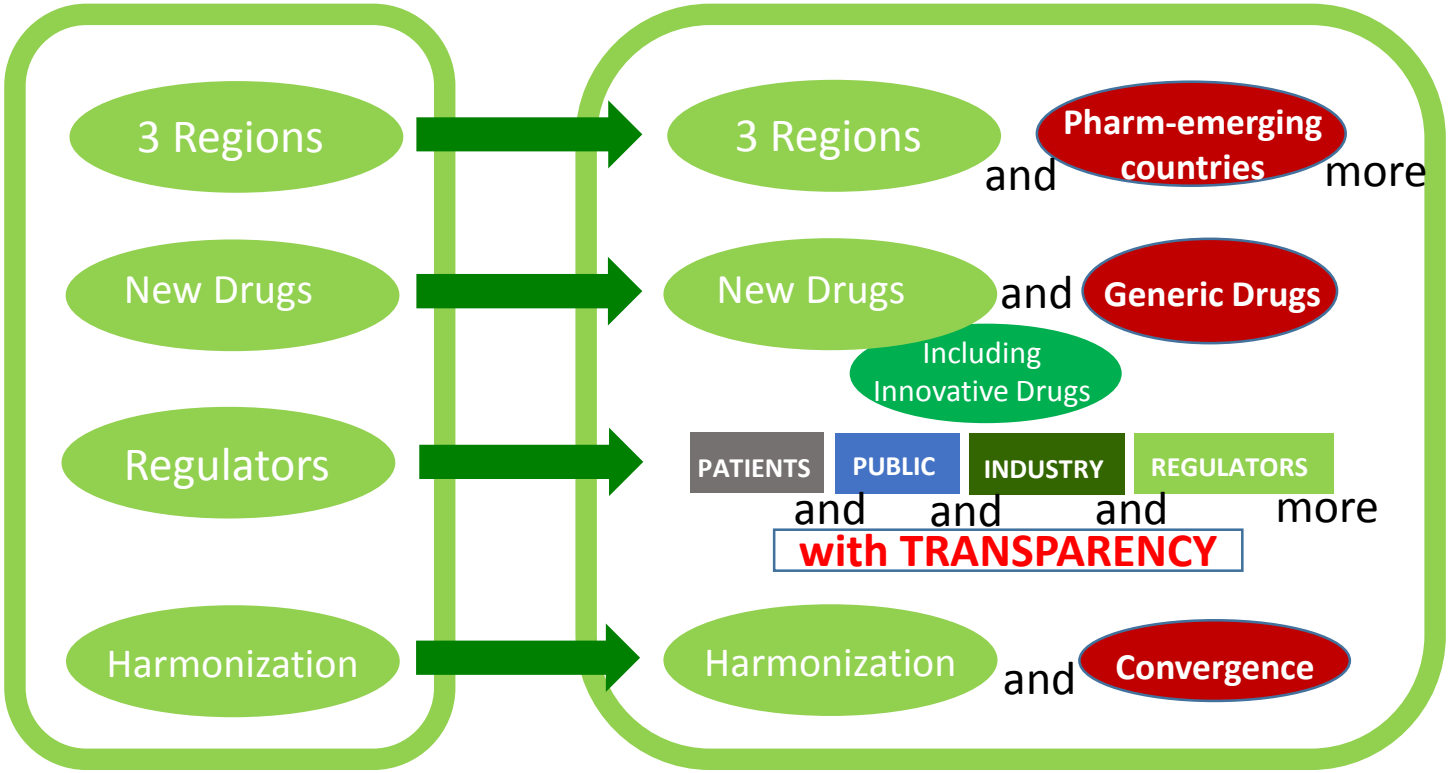
Rapid Paradigm Shift Surrounds The International Regulatory Community



Regulatory Science: Scientific yet ethical Judgment

・Scientific yet ethical judgment is necessary for pharmaceutical affairs

・Mad Science should not be the basis of scientific evidence

・Regulatory Science was proposed by Japan's pharmaceutical organizations（1987）

Regulatory Science for Pharmaceutical Affairs

The 3 Pillar-system

1. one-dimensional research necessary for ensuring quality, efficacy, and safety（Micro Science）
2. Multi-dimensional research of comprehensive research for evaluating the results of the one-dimensional research（Macro Science）

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3. translational research for enacting measures to promote innovations of medical products and its dissemination based on the above results （Engineering）

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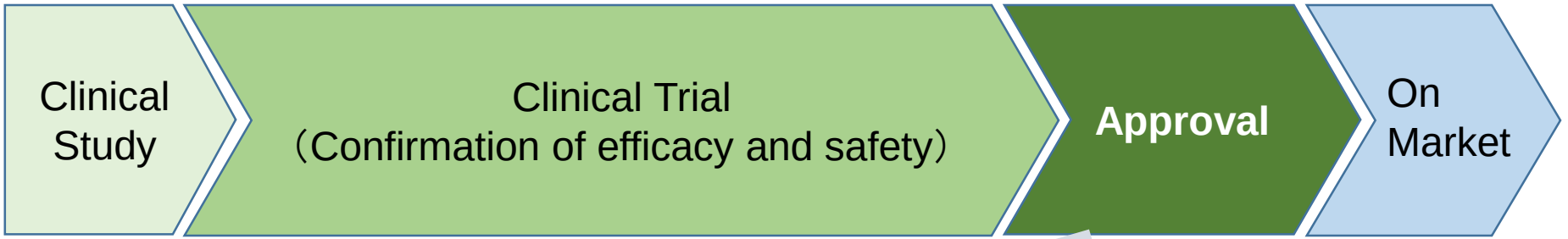
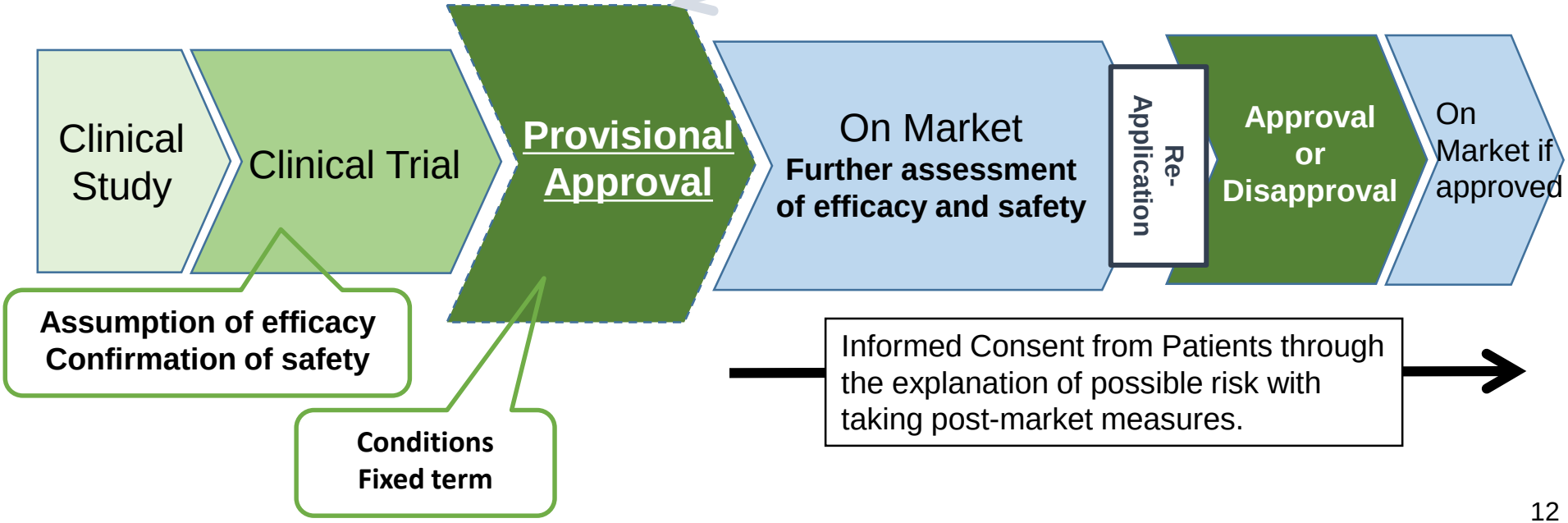
New approval system for regenerative medicine product will be introduced

[Current approval system]



[Adaptive Licensing System]

Early-Access!!



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3rd 5-year mid-term plan of PMDA (FY2014-2018)

Major challenges

Shortening the time from early development to approval
"Zero" review time lag Support for elimination of development time lag

High quality review/consultation services

Enhancing safety measures

Globalization

Specific measures

Accelerated review process
(Improvement of approval predictability)

Improvement of prior assessment
(substantial acceleration of approval review process)

Enhanced overseas inspection system

Drastic improvement of consultation service
Active involvement from the early development phase
- Improvement of pharmaceutical affairs consultation service on R&D strategy
- Improvement of clinical trial consultation service

Enhancement of regulatory science research and human resource development
- **Development of advanced review/consultation framework using innovative assessment techniques**
- **Cross-products analysis of accumulated large data sets by PMDA using innovative techniques**
- Utilization of Science Board (cooperation with the academia)

Utilization of medical information database

Readiness for introduction of risk management plan

Appropriately accommodate the most advanced technologies including personalized medicine and regenerative medicine

Prerequisites:
US/EU-equivalent system and human resources with excellent skills

Goal

- Development of Japan's original innovative drugs and medical devices
- Marketing of cellular and tissue-based products

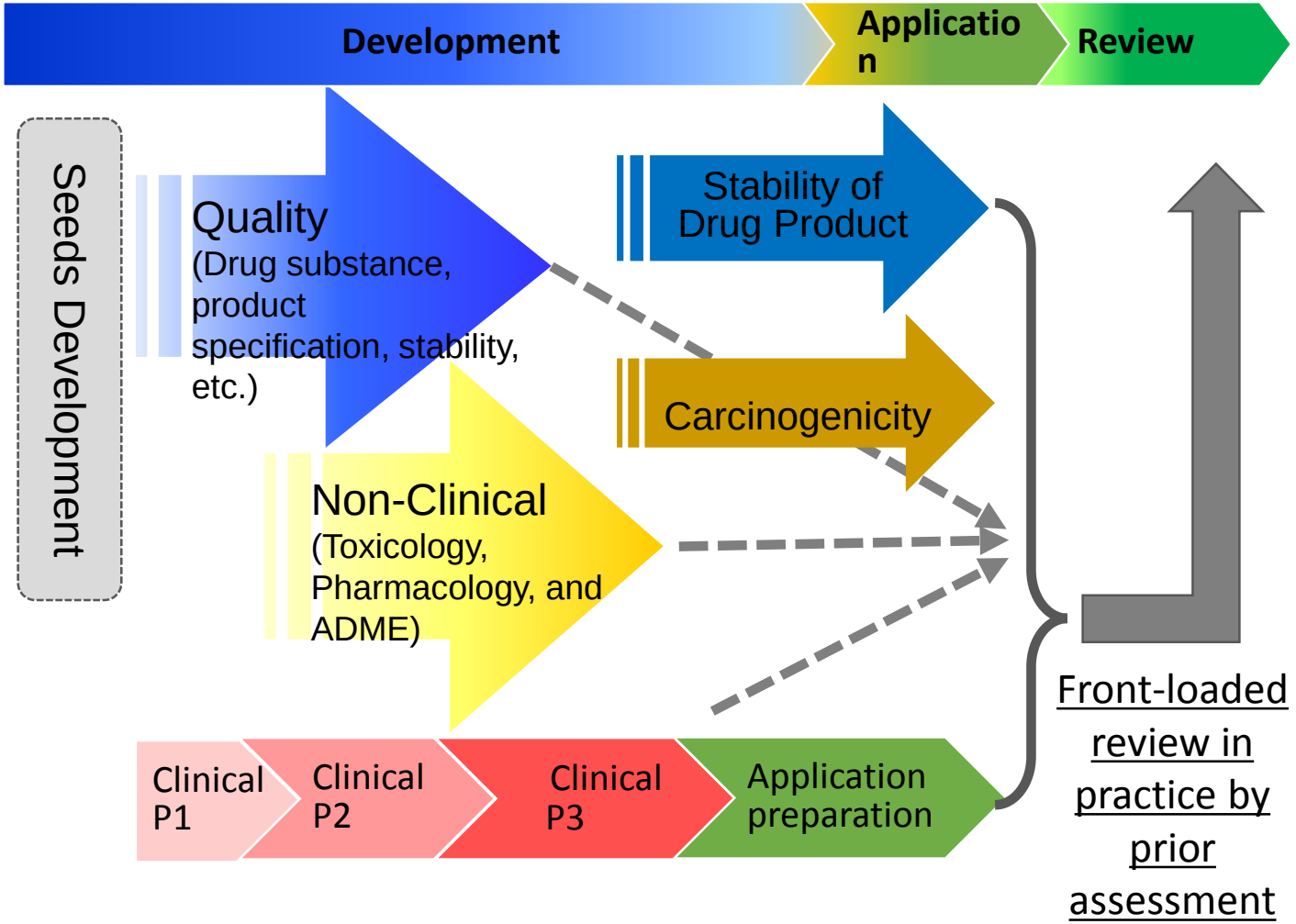
Activation of the industry

Extending health and life span of Japanese people

Contribution to global medicine

Responding to social needs such as Japan Reconstruction Strategy and Health/Medical Care Strategy

Reinforcement of Prior Assessment Consultations



Gradual evaluation based on each test result

More smooth reaction by applicant

Outcomes of the Science Board

Current Perspective on Evaluation of Tumorigenicity of Cellular and Tissue-based Products Derived from induced Pluripotent Stem Cells (iPSCs) and iPSCs as Their Starting Materials (Aug. 20, 2013)

- Tumorigenicity of Cellular and Tissue-based Products
- Assessment on the Undifferentiated Cells/Tumorigenic Cell Contaminants and Tumorigenicity in Cellular and Tissue-based Products Derived from iPS cells, etc.
- Assessment and Management of Tumorigenicity of Human (Allogeneic) iPS Cells Used for Manufacturing iPS Cell-derived Products

Summary of Discussion on Non-clinical Pharmacology Studies on Anticancer Drugs (Nov. 15, 2013)

- Current status of non-clinical pharmacology studies on anticancer drugs and the concept of evaluation in regulatory review
- Role and expectations for non-clinical pharmacology studies in future development of anticancer drugs (role of non-clinical pharmacology studies and their contribution to the development of anticancer drugs, taking into account of the recent advancement of personalized medicine)

Summary of the discussions on assessment of the current status of personalized medicine relating to drug development and review

- *(English material is in the make and will be up when ready)*

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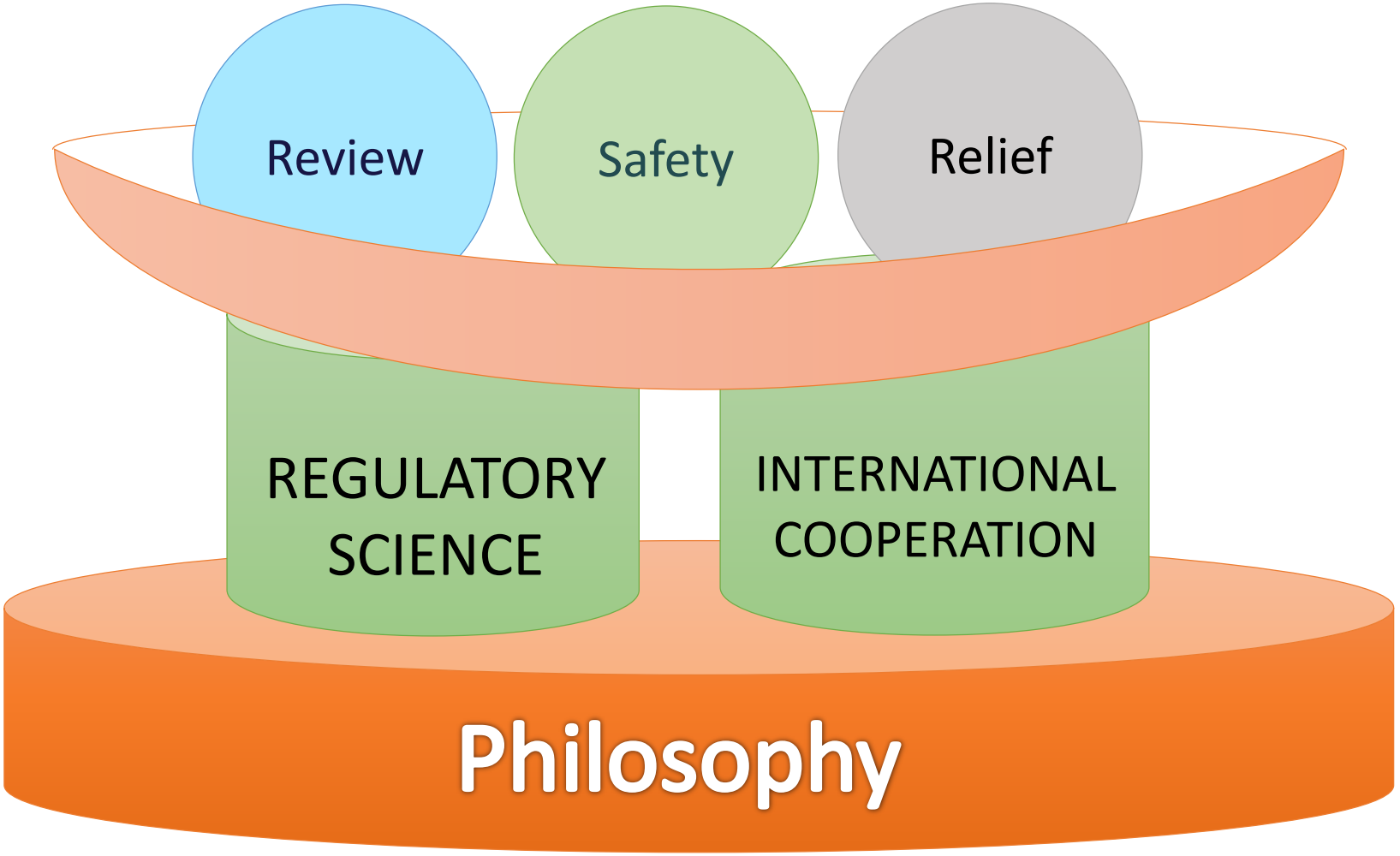
To Improve Public Health



Thank you for your attention!

謝謝！