

Overview of the Plan for Promotion of Medical Research and Development

Cabinet Secretariat
Office of Healthcare Policy

Introduction

① New Horizon of Medical R&D

- Innovative medical technologies...exponentially developed all over the world
- Ethics, social implications and transparent procedure...highly important, because the research targets of medical R&D are human beings
- Prevention before the onset of symptoms...increasingly recognized as important

< Cycle of Medical R&D >

(1) Basic Research

-Elucidation of the mechanisms of diseases

(4) The Evaluation of the Effect and the Setting of the New Research Themes

-Evaluation in a large number of patients.
e.g. Epidemiology, Clinical epidemiology, etc.



(2) Translational Research toward Clinical Research and Trial

-Evaluation on effectiveness and safety
-Study of regulatory science
-Clinical research and trials

(3) Clinical Use

-Additional study in a small group
-Practical application on clinical site

② Status of The Plan for Promotion of Medical R&D

- Based on the Healthcare Policy (Cabinet decision on July 22, 2014)
- Covers the five years from FY2014, foreseeing the next 10 years

I. Basic Policy on Medical R&D

① Current Issues to Be Solved in Medical Sciences in Japan

- Basic Research: The effort to develop the results of basic research toward clinical use is insufficient.
- Clinical Research: The support function (e.g. data management, ethics, etc.) is insufficient.
- Industry: Corporate scale is small. Start-up companies are few.
- Government: Bureaucratic sectionalism

② Ten Pillars of the Basic Policy

1. Establishing systems for putting basic research outcomes into practical application
 - (1) Necessity for fundamental reform to improve the environment of clinical research and clinical trials
 - (2) Promotion of “Cycle of Medical R&D” and realization of open innovation
2. Establishing new systems for improving development of new drugs and medical devices
3. Toward the implementation of evidence-based medical care
4. ICT(Information and Communication Technology) for healthcare
5. R&D toward the world’s most advanced medicine
 - (1) Regenerative medicine
 - (2) Genomic medicine
 - (3) Other cutting-edge R&D
6. Conducting research from an international perspective
 - (1) Selection of research themes from an international perspective
 - (2) International collaboration and international contributions
 - (3) International harmonization of regulations, etc.
7. Capacity building
8. Research integrity and compliance (ethics, guidelines, acts, etc.)
9. Research infrastructure
10. Intellectual property management

II. The Intensive and Planned Medical R&D Policy

○ New National Framework for Medical R&D

1. Expected Functions for Japan Agency for Medical Research and Development (A-MED)

- ① Management of medical R&D
- ② Management of data produced from clinical research and trials
- ③ Support for practical application
- ④ Support for improving R&D infrastructure
- ⑤ Promotion of international strategy

2. Research Projects in order to Smoothly Put the R&D Research Outcomes into Practical Application

- ① Drug Discovery
- ② Medical Device
- ③ Translational and Clinical Research Core Centers
- ④ Regenerative Medicine
- ⑤ Genomic Medicine
- ⑥ Cancer
- ⑦ Psychiatric and Neurological Diseases/Disorders
- ⑧ Emerging/Re-emerging Infectious Diseases
- ⑨ Rare/Intractable Diseases etc.

3. Establishment and Utilization of Common Infrastructure

4. The Status of Clinical Trials Core Hospital in the Medical Care Act

- Set requirements for Clinical Trials Core Hospital in the Medical Care Act
Promote high-quality clinical research and trials, a requisite for development of innovative drugs and medical devices based on Japanese technology

III. Requisite for the Intensive and Planned Medical R&D Policy

○ Follow-up

- Within approximately five years at least, Headquarters of Healthcare Policy will evaluate the progress, considering the changes in the circumstances around the medical R&D, and make necessary changes.

< Key Performance Indicators ① >

Drug Discovery

By FY2015

- Consult/Evaluate seeds 400 cases
- Support promising seeds 40 cases
- License out 1 case

By around 2020

- Consult/Evaluate seeds 1500 cases
- Support promising seeds 200 cases
- License out 5 cases
- Identify drug targets 10 cases

Medical Devices

By FY2015

- Formulate 10 new guidelines for the promotion of medical device development
- Expand the domestic medical device market (2.4 trillion yen in 2011→2.7trillion yen)

By around 2020

- Double medical device exports (approximately 500billion yen in 2011 → 1trillion yen)
- Implement more than five innovative medical devices
- Expand the domestic medical device market: 320 million yen

Translational and Clinical Research Core Centers

By FY2015

- Investigator-initiated clinical trials 21 cases per year
- First in Human (FIH) clinical trials (including Industry-initiated clinical trials) 26 cases per year

By around 2020

- Investigator-initiated clinical trials 40 cases per year
- FIH clinical trials (including Industry-initiated clinical trials) 40 cases per year

Regenerative Medicine

By FY2015

- Initiate clinical research and trials using human stem cells (e.g. age-related macular degeneration, corneal disorders, knee meniscal injury, bone and cartilage reconstructions, blood disorders, etc.) approximately 10 cases
- Develop technology for drug discovery using iPS cells

By around 2020

- Clinical application of new drugs using iPS cell technologies
- Increase the number of official approvals
- Expand target diseases in clinical research/trials to approximately 15 subjects*
- Implement medical devices regarding regenerative medicine
- Proposal of methods for cardiac safety assessment using iPS-derived cardiomyocytes toward international harmonization

*Includes 10 subjects in Goals by 2015

Genomic Medicine

By FY2015

- Collaborate with Bio Bank Japan, National Center Biobank network, Tohoku Medical Megabank, etc.
- Establish a whole genome/polymorphism database on diseases
- Complete whole genome reference panel in the Japanese and susceptibility genes for disease outcome
- Develop precision medicine of adverse reactions to antiepileptic drugs

By 2020-30

- Drastically reduce prevalence of adult-onset diseases (diabetes, stroke, myocardial infarction, etc.)
- Establish precision medicine on carcinogenesis and adverse reactions to anti-cancer agents, etc.
- Initiate clinical research on genomic medicine for dementia, etc.
- Develop innovative diagnostics and therapeutics in neuromuscular diseases/disorders, etc.

< Key Performance Indicators ② >

Cancer

By FY2015

- Identify 10 seeds of new anti-cancer agents
- Identify 5 biomarkers for early diagnostics and immuno-therapeutics
- Reduce 20% of cancer mortality rate (20% by 2015, compared to age-adjusted mortality rate in people aged less than 75 years old in 2005)

By around 2020

- Within five years, start 10 clinical trials in innovative anti-cancer agents
- Initiate more than six clinical trials on new anti-cancer agents, including unapproved and off-label drugs, in pediatric, intractable, and rare cancers, etc.
- Obtain at least one additional PMDA approval and clinical indication in drugs for pediatric and rare cancers, etc.
- Resolve so-called 'device-lags' and 'drug-lags'
- Establish standard treatments in pediatric, geriatric and rare cancers (draw at least three guidelines)

Emerging/Re-emerging Infectious Diseases

By FY2015

- Establish whole genome database on pathogens, elucidation of pathophysiology, and mapping of pathogens in Asian countries (for the purpose of improving public health capability to deal with influenza, dengue fever, diarrheal diseases, drug-resistant bacteria, etc.), through a mechanism of sharing pathogens and clinical information at the global level

By around 2020

- Identify drug target sites and develop rapid diagnostic methods based on the whole genome database in the obtained pathogens (influenza, dengue fever, diarrheal diseases, drug-resistant bacteria, etc.)
- Conduct clinical research/trials and apply for PMDA approval of norovirus vaccine and intranasal influenza vaccine

By around 2030

- Develop new vaccines (e.g. universal vaccines for influenza)
- Develop new antibiotics, antiviral agents, etc.
- Eradicate polio and measles etc. (tuberculosis by 2050) through cooperation with WHO and other countries

Psychiatric and Neurological Diseases/Disorders

By FY2015

- Establish diagnostic methods for dementia at a very early stage using molecular imaging
- Discover at least one new biomarker candidate for psychiatric disorders and adverse drug reactions, and complete clinical evaluation for identification process

By around 2020

- Initiate clinical trials on innovative drug candidates in psychiatric disorders including dementia
- Establish objective diagnostic methods in psychiatric disorders
- Establish proper drug interventions in psychiatric disorders
- Draw maps of structures and functions of the entire brain network

Rare/Intractable Diseases

By FY2015

- Initiate more than seven new clinical trials towards PMDA approval (Severe pulmonary hypertension, prion diseases including Creutzfeldt-Jacob disease, etc.)

By around 2020

- Expand indication of new drugs and existing drugs in more than 11 diseases (ALS, distal myopathy, etc.)
- Promote international collaboration in clinical research/trials with European and U.S. databases

(Appendix) Expected Future Image

By around 2020

- Start 10 clinical trials in innovative anti-cancer agents
- Initiate clinical research on genomic medicine for depression and dementia, etc.
- Develop rapid diagnostic methods for infectious diseases
- Identify 10 drug targets
- Promote implementation of more than five innovative medical devices



Strategies

KPI's to be achieved through interministerial collaborative projects

By FY2015

- Identify five biomarkers for detecting cancers at an early stage
- Establish a whole genome/polymorphism database on diseases
- Initiate clinical research and trials using human stem cells (e.g. age-related macular degeneration, corneal disorders, knee meniscal injury, bone and cartilage reconstructions, etc.)



Expected Future Image

1. Provide the world's leading medical care
→ Extension of health expectancy
2. Enhance industrial competitiveness in the fields of pharmaceuticals and medical devices
→ Contribution to economic growth
3. Take initiatives for international collaboration and contribution in the field of medicine