Japan Agency for Medical Research and Development (AMED)

Missions and Challenges

Makoto Suematsu, MD, PhD
President
Japan Agency for Medical Research and Development

JSGT2015 and APGTC2015 in Osaka July 24, 2015
Our goal is to fast-track medical R&D that directly benefits people not only by extending lifespans but also by improving quality of life: supporting “life” as biological existence, diary living, and lifespans.

A success of AMED (Japan Agency for Medical Research and Development) highly depends on alliance and cooperativity of three ministries (METI, MEXT, MHLW).

To this end, we need to challenge for an art of probability.

Not only budgets but also officers and scientists with new mindsets.

3 “MUST NOT” rules in AMED organization.
Organization of AMED

- Office of Audit
- Department of Planning and Management
- Department of General Affairs
- Department of Financial Affairs
- Department of Research Integrity and Legal Affairs
- Department of Intellectual Property
- Department of Research Promotion
- Department of Industrial-Academic Collaboration
- Department of International Affairs
- Department of Research Infrastructure
- Department of Clinical Research and Trials
- Department of Innovative Drug Discovery and Development

- Support Section
- Management Section
- Project Section

- Permanent Staff: 102
- Fixed-term Staff: Approx. 200

- Auditor
- Executive Director
- President
- Council of Research and Management
- Advisory Board
Longitude/cross-sectional collaboration between “Department of Research Promotion Matrix structure for accelerating Medical R & D

Department of Research Promotion

7 Research Projects

Drug Research
Regenerative Medicine Research
Cancer Research
Neurological, Psychiatric and Brain Research
Rare/Intractable Disease Research
Infectious Disease Research
Emerging Research

Industrial-Academic Collaboration
Support for practical application such as industrial-academic collaboration

International Affairs
Promotion of strategic international researches

Research Infrastructure
Support for accommodating R&D platforms such as BioBank, etc.

Clinical Research and Trials
Support for high-quality clinical studies/clinical trials

Innovative Drug Discovery and Development
Support through the Drug Discovery Support Network for realizing academia drug discovery
Undiagnosed Disease Program in NIH
diagnose (and treat) those undiagnosed (from 2008, Dr William Gahl, NIH)

Undiagnosed Disease Patients

Home doctors

Collection of data and examination

Regional Core Hospitals

Analyses by physicians with different specialties

Diagnose known diseases

Assessment by specialist of clinical genetics

Genome analysis center

NGS (ISO, CLIA etc) metabolomics

Data banking

Strong team of physician scientists

Local Specialists of Clinical Genetics

The Japan’s National Liaison Council for Clinical Sections of Medical Genetics etc.

Genetic counselling

Medical Consultation

Judge treatable or not?

Return of the Research Findings to Patients Individually

G and M data

Phenotyping

Return of the Research Findings to Patients Individually
Mutation of riboflavin receptor causes “ALS-like” severe phenotypes in a newborn baby.

AMED will start
✔ Joining in IRDiRC in this autumn
✔ Organizing IRUD (Initiative for Rare and undiagnosed diseases) under collaboration with NIH-UDP Program
Dr. William Gahl, MD, PhD
Founder of undiagnosed Patients Program, NIH
“Not sequencers but real physicians” “Phenotype is everything”
In genome medicine, “likely pathogenic” variant now may turn out “likely benign” 5 years later; Necessity for real-time data sharing of clinical genetics through DB

SPECIAL REPORT

ClinGen — The Clinical Genome Resource

Heidi L. Rehm, Ph.D., Jonathan S. Berg, M.D., Ph.D., Lisa D. Brooks, Ph.D., Carlos D. Bustamante, Ph.D., James P. Evans, M.D., Ph.D., Melissa J. Landrum, Ph.D., David H. Ledbetter, Ph.D., Donna R. Maglott, Ph.D., Christa Lese Martin, Ph.D., Robert L. Nussbaum, M.D., Sharon E. Plon, M.D., Ph.D., Erin M. Ramos, Ph.D., Stephen T. Sherry, Ph.D., and Michael S. Watson, Ph.D., for ClinGen

On autopsy, a patient is found to have hypertrophic cardiomyopathy. The patient’s family pursues genetic testing that shows a “likely pathogenic” variant for the condition on the basis of a study in an original research publication. Given the dominant inheritance of the condition and the risk of sudden cardiac death, other family members are tested for the genetic variant to determine their risk. Several family members test negative and are told that they are not at risk for hypertrophic cardiomyopathy and sudden cardiac death, and those who test positive are told that they need to be regularly monitored for cardiomyopathy on echocardiography. Five years later, during a routine clinic visit of one of the genotype-positive family members, the cardiologist queries a database for current knowledge on the genetic variant and discovers that the variant is now interpreted as “likely benign” by another laboratory that uses more recently derived population-frequency data. A newly available testing panel for additional genes that are implicated in hypertrophic cardiomyopathy is initiated on an affected family member, and a different variant is found that is determined to be pathogenic. Family members are retested, and one member who previously tested negative is now found to be positive for this new variant. An immediate clinical workup detects evidence of cardiomyopathy, and an intracardiac defibrillator is implanted to reduce the risk of sudden cardiac death.
AMED has launched Initiative for Rare and Undiagnosed Diseases (IRUD)
IRUD臨床専門分科会
・疾患エキスパートによる専門分科会
・IRUD診断委員会による依頼を受け、専門的な症例検討を追加する
・必要に応じてIRUDに参加する拠点病院で直接診療

IRUDに参加する拠点病院
拠点病院が全国に設置されるよう体制整備を支援していく

IRUD診断委員会
・幅広い診療科の医師で構成
・臨床遺伝専門医を中心として臨床カンファレンスを開催
・地域の臨床医と積極的に連携し、地域をあげて取り組む

IRUD constitutes a network consisting of multiple hospitals to save patients with RUD with equal opportunities all over Japan
AMED has participated in IRDiRC to share IRUD data for saving patients over the world.

- Japan has joined IRDiRC in July 30, 2015
- Data sharing for patients
- Machine-readable consent
- Microattribution
Advocacy activities by patients and their supporters;
Patients constitutes a stakeholder that participates in designing clinical researches

Permission by Julie Fleshman, President and CEO, PanCan

Pancreatic Cancer Action Network

KNOW YOUR TUMOR℠
Powerful Knowledge. Personal Treatment℠

Increase and influence government and private funding for research
Promote running "smart" clinical trials
Heighten public awareness and visibility

Increase clinical trial enrollment rate
Identify the best practices and disseminate them across the country

2015
2020
Discovery Innovation Screening Consortium: DISC

Purpose: Enrich “All Japan” screening library by integrating unique compounds of pharmaceutical companies for use in HTS by the Drug Discovery Support Network

“All Japan” Screening Library

- AIST (N²PC) Natural products
- Univ. of Tokyo Low MW compounds
- NIBIOHN mAb-Phage/aptamer
- AMED/iD3 Low MW compounds pharma-companies

Impact of DISC

Whole: create the first non-competitive collaborative space in Japan and improve overall pharma R&D productivity

Academics: increase the chance to find quality lead and tool compounds for their own newly discovered targets

Industry: maximize potential value of their own compounds through increased chance to be screened out against new targets discovered by universities, etc.

Already established: AIST, NIBIOHN, Univ.Tokyo

Public-Private Partnership to take advantage of unique compounds & HTS technology of pharma-industry for more effective translational research
- iD3 to select targets & cover HTS cost
- Pharma to provide access to premium compounds and HTS technology/equipment

Compound storage, assay plate preparation, HTS implementation and cherry picking to be outsourced to industry
To construct a system that can predict toxicological risk, etc., with a certain significance level by integrating various databases developed to date in our country, while identification of true hit compounds and generation of lead compounds have been done by eye ball of medicinal chemists with experience and expertise.

Constructing the system within 3 years, followed by the 2-year validation period.
AMED Mission

1 Global Cooperation for Controlling Diseases:
   ① Infectious diseases
   ② Regenerative medicine
   ③ Neuropsychiatric diseases
   ④ Rare diseases (and undiagnosed diseases) and R/D for orphan drugs

2 Empowerment of Medical R&D in Japan
   ① Opening resources of drug libraries
   ② Accelerating nation-wide registry for optimizing cancer Tx
   ③ Establishing infrastructure of clinical genetics (CLIA lab, etc)
   ④ Fostering young physician scientists for medical R/D
   ⑤ Utilization of private-public partnership to stimulate medical R/D

3 Renew selection processes and improve flexibility of budget systems

4 Clinical database
   (under supervision by Cabinet Secretariat, Office of Healthcare Policy)