

National “Nan-Byo” Strategy 国家難病戦略

Intractable and Rare Diseases strategy of Japan

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The Permanent Mission of Japan
to the United Nations

With support from
Intractable/Rare Disease Division
Ministry of Health, Labour and Welfare
Government of Japan



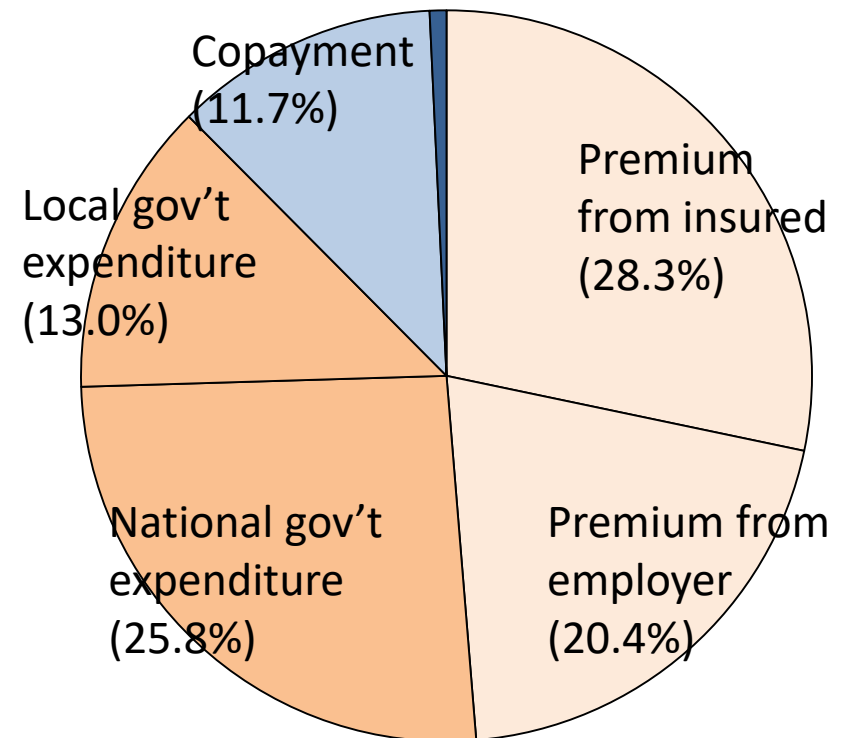
Japan's Universal Health Coverage System

- Japan has achieved one of the world's highest level of life expectancy and health care standards through a universal health insurance system.
- It is necessary to continue to ensure a safe and secure lives of citizens by firmly maintaining the universal health insurance under the current social insurance system.

Characteristics of the universal health insurance system in Japan

1. Universal coverage with compulsory public health insurance
2. Free access to mostly private medical providers
3. High-quality health care services with low costs
4. Based on the social insurance system subsidized by public expenditure

Breakdown of National Medical Expenditure by source of funding in Japan (FY2014)



Definition of “Intractable and Rare Diseases” in Japan

難病

No clear cause

No established therapy

Rare

Requires long-term care

Definitions and Regulations for Intractable/Rare Diseases in Japan/US/Europe

	Japan	US	Europe
Definition	<ul style="list-style-type: none"> Rarity*¹ (patients less than approx. 0.1%*² of general population) 	<ul style="list-style-type: none"> Rarity*¹ (fewer than 200,000 patients) 	<ul style="list-style-type: none"> Rarity*¹ (5 patients or less per 10,000 people)
	<ul style="list-style-type: none"> Unclear cause 		
	<ul style="list-style-type: none"> No effective therapy 	<ul style="list-style-type: none"> No effective therapy 	<ul style="list-style-type: none"> No effective therapy
	<ul style="list-style-type: none"> Long-term hindrance to everyday living (requires long-term treatment) 		<ul style="list-style-type: none"> Extremely serious disease that severely hinders everyday living
Related laws/regulations	Intractable/ Rare Diseases Act (2014) Amended Pharmaceutical Affairs Act (1993)* ³	Orphan Drug Act (1983)	Orphan Medicinal Product Regulation (1999)

*1: Rarity based on European definition: US less than 7, Japan less than 14, per 10,000

*2: Article 77.2 of the Pharmaceutical Affairs Act establishes an upper limit of 50,000 eligible patients as a requirement for designation as an orphan drug or orphan medical device.

*3: Amendments to Pharmaceutical Affairs Act and Adverse Drug Reaction Relief and R&D Promotion Fund Act for the purpose of promoting research and development on orphan drugs

Background to Measures for Intractable/Rare Diseases

The **SMON(subacute myelo-optico-neuropathy) disease** epidemic in around 1964 was an original impetus for the government's promotion of measures for intractable and rare diseases. Research identified phytotoxicity, and medical fee assistance was initiated in 1971 as a patient relief measure.

The Ministry of Health and Welfare established the Overview of Measures Against Intractable/Rare Diseases in 1972, and **started the Specific Disease Treatment Research Program** as one such measure.

Disease Scope

- 1.No clear cause, no established therapy, and may leave aftereffects
- 2.Chronic, and entails major financial and psychological burden

Implementation Methods

- 1.Promotion of surveys and research
- 2.Establishment of medical institutions
- 3.Reduction of co-payment of medical fees (implemented by prefectures; reduced within scope of budget with 50% subsidy rate)

Measures Initiated for the Following Diseases (Underlined diseases subject to medical fee assistance)

SMON (subacute myelo-optico-neuropathy)

Bechet's disease

Myasthenia gravis

Systemic lupus erythematosus

Sarcoidosis

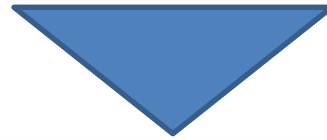
Aplastic anaemia

Multiple sclerosis

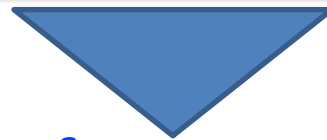
Intractable hepatitis

Establishment of Intractable/Rare Diseases Act (1)

- **Budgets insufficient** due to increase in patients
- Sense of unfairness among eligible patients (Some diseases not subject to medical fee assistance even if cause not clarified and therapy not established)



- Comprehensive and systematic work to reform intractable/rare disease measures
- Regarding medical fee assistance, utilize revenue from consumption tax increase and position as fair and stable social insurance benefits program that is sustainable in the future



2015 Establishment of Intractable/Rare Diseases Act

Establishment of Intractable/Rare Diseases Act (2)

Objectives

The Intractable/Rare Diseases Act is intended to establish a fair and stable system by codifying medical fee assistance for patients of intractable/rare diseases and allowing consumption tax revenue to be allocated for sustainable financing and also to implement measures such as establishment of basic policies, promotion of surveys and research, and implementation of programs to improve the treatment and care environment.

Summary

1. Formulate basic policies
2. Establish new, fair and stable medical fee assistance program for intractable/rare diseases
3. Promote surveys and research related to intractable/rare disease medicine
4. Implement treatment and care environment improvement program

Enactment

May 23, 2014

Enforcement

January 1, 2015*

*: Same date as the Act on Partial Amendment of Child Welfare Act (codifies medical fee assistance for patients of specific pediatric chronic diseases)

“Designated Intractable Diseases” Eligible for Medical Fee Assistance

Intractable diseases that meet all the following conditions and **for which there is strong need for appropriate, good-quality medical treatment** in light of the situation of patients and that have been designated by the Minister of Health, Labour and Welfare upon hearing the opinion of the Health Science Council.

- Number of patients in Japan not above a fixed number*
- Objective diagnostic criteria (or the equivalent) are established

*: Stipulated in MHLW ordinance as around 0.1% of the general population

Overview of Research Promotion

FY18 Budget : \10.0 billion (policy \1.6 b, practical \8.4 b)

Intractable Diseases Policy Research Program (MHLW)

- Construction of medical exam system, epidemiological studies, public awareness
- Creation and revision of diagnostic standards and examination guidelines
- Promotion of transition from pediatric to adult medical care
- Coordination with related studies and databases; etc.

Intractable Diseases Practical Research Program (AMED)

- Pathogenic analysis
- Omics analysis
- Initiative on Rare and Undiagnosed Diseases (IRUD)
- Development of new treatments and medical devices, etc.
- Physician-led clinical trials; etc.

Info Provision
Coordination



Register data based on
clinical study ID cards

- Return research findings
- Provide latest information

Participate, etc. in
clinical trials, etc.

Intractable Disease Patients

Construction of Medical Provision System

Intractable Disease Treatment Support Network

- National Centers for Advanced and Specialized Medical Care
- Intractable Disease Research Groups
- Medical Societies
- IRUD



Prefecture-designated hospitals for all intractable diseases

Designate



Coordinate

Designated hospitals for specific intractable diseases

Designate

Refer

Cooperating hospitals for intractable diseases: Regular hospitals & clinics (Primary care doctors, etc.)

Coordinate

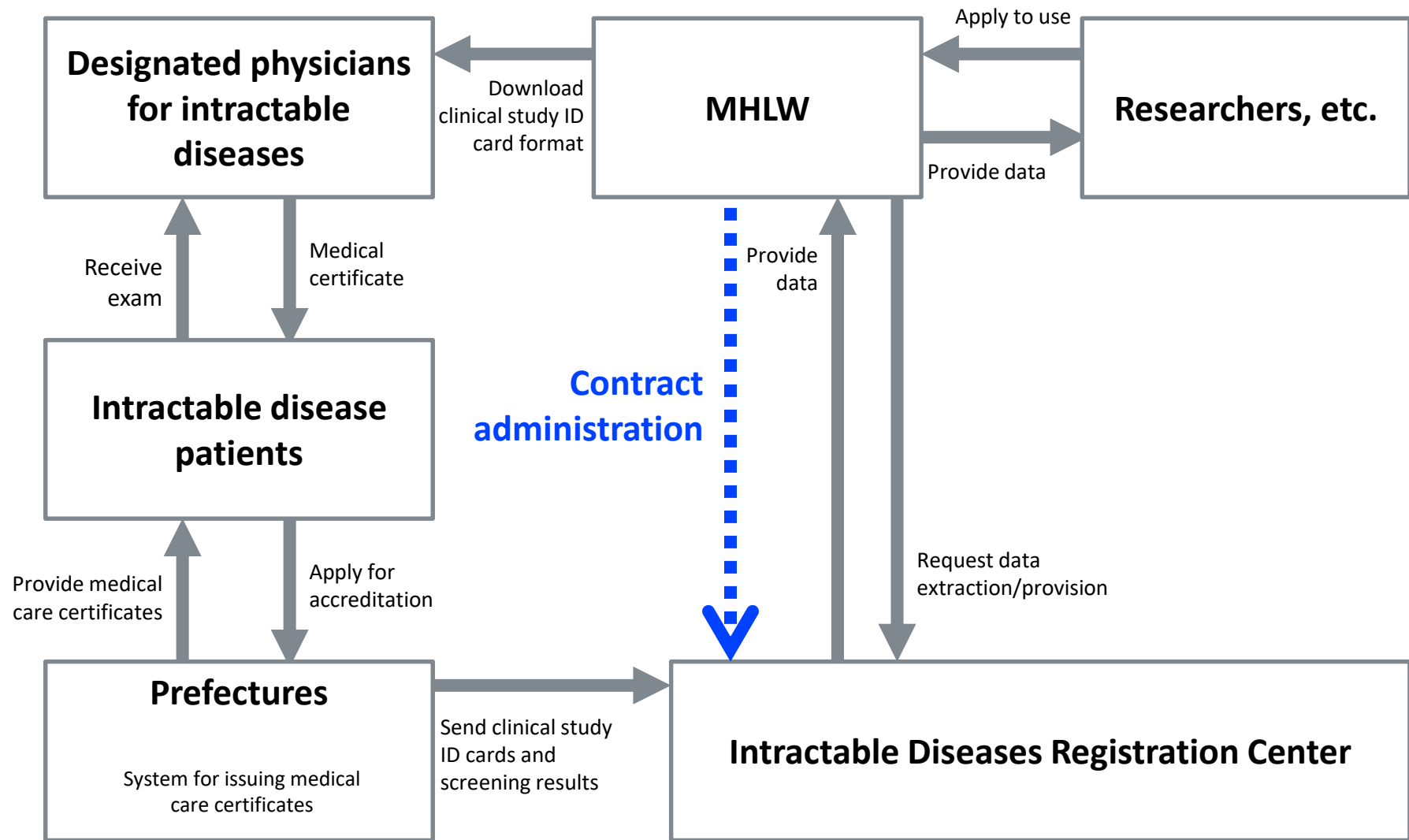
- Health Centers
- Home medical care, etc.

Receive examinations

Patients

Prepared based on “Structure of Medical System for Intractable Diseases (Report)” Reference Materials” (October 2016)

Construction of Database



Plan to contract system operation and maintenance separately to private sector

Patient Support

Patients

“I’m so worried.”

“I want to live in more comfort!”

“I want to work.”

“I want appropriate treatment! I need in-home care!”

Support

Consult

Intractable Disease Consultation and Support Centers (Intractable Disease Consultation/Support Staff/Peer Supporters)

- Various types of consultation and support (provide living-related information, support for various government procedures, support for daily living)
- Employment support (in coordination with intractable disease patient employment supporters) (For (1) people who’ve developed an intractable disease while employed, and (2) who would like to be employed)
- Promotion of community groups, etc.
- Peer support, training of peer supporters; etc.

- Health Centers
- Medical institutions, etc. (Designated intractable disease doctors, etc.)
- Welfare services
- Employment Center
- Employment and Vocational Life Support Centers for Persons with Disabilities
- Patient/family groups

Community Councils for Intractable Disease Measures

Provide support for intractable disease patients through coordination among local physicians, nurses, welfare workers, patient groups, etc., including establishment of Community Councils for Intractable Disease Measures primarily at Health Centers

Intractable Disease Patient Employment Supporters

Job consultations based on disease characteristics, information for companies on employment management for people with intractable diseases; etc. (at Employment Center)

Public Awareness through Japan Intractable Diseases Information Center (JIDIC)

Overview

The Japan Intractable Diseases Information Center publicizes disease profiles, findings of the Research Program on Measures for Intractable Diseases, and other information in order to **provide information to intractable disease patients, family members and medical professionals, etc.**

- Disease information
- Government measures for intractable diseases
- Summaries of programs and services
- Average access numbers: Approx. 2.5 million items/month



Top page of Japan Intractable Diseases Information Center

Comprehensive rare disease policy

- Should include :
 - Fair and sustainable financing
 - Survey & research
 - Treatment, care & support systems
- Should be based on the concept of “no one left behind”
- Is a prerequisite to Universal Health Coverage!

