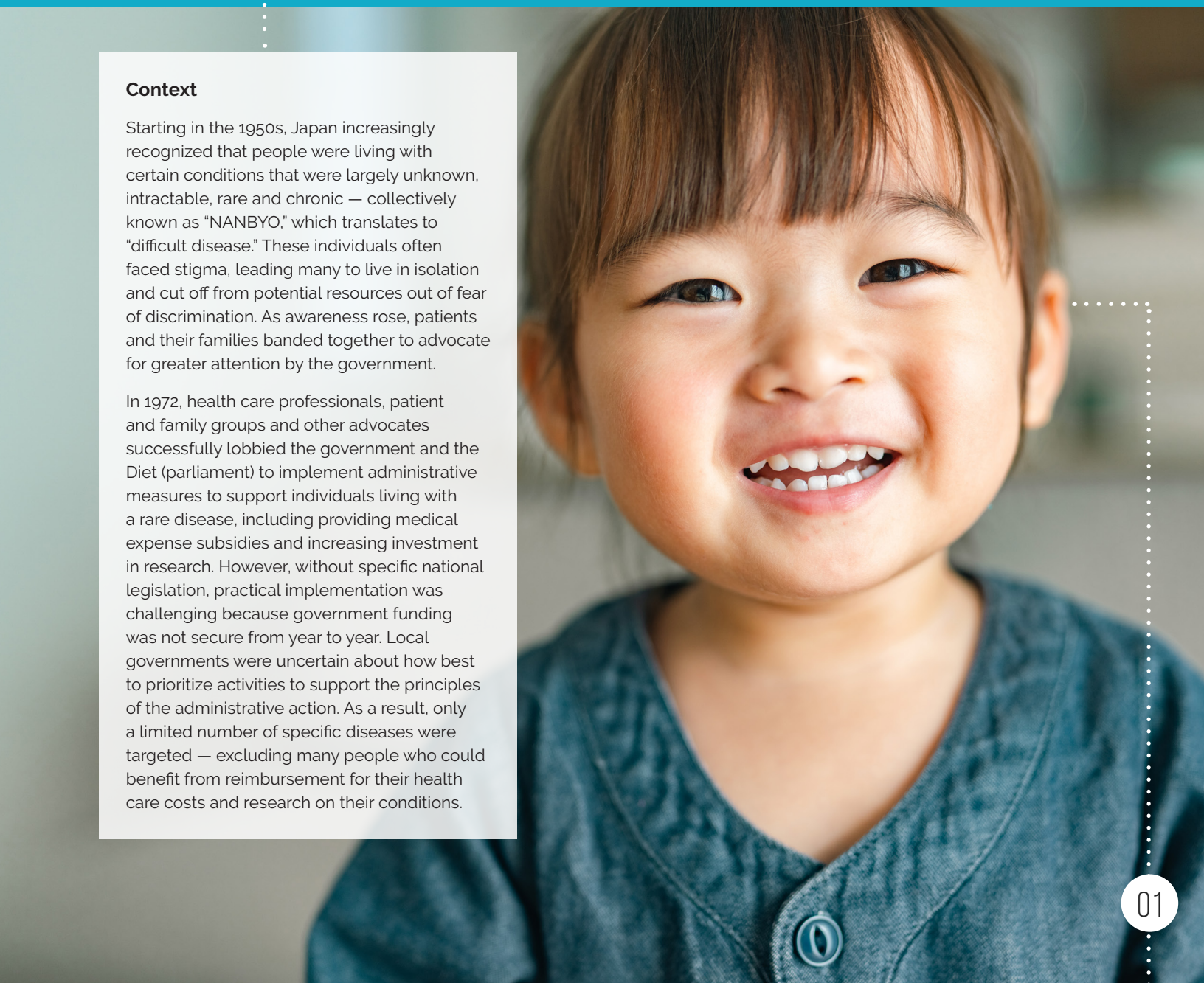
CASE STUDY: NANBYO Act (Japan)

PILLAR: Policy

National legislation that supports people living with rare conditions in Japan — including reimbursement for diagnostic services and care



Context

Starting in the 1950s, Japan increasingly recognized that people were living with certain conditions that were largely unknown, intractable, rare and chronic — collectively known as "NANBYO," which translates to "difficult disease." These individuals often faced stigma, leading many to live in isolation and cut off from potential resources out of fear of discrimination. As awareness rose, patients and their families banded together to advocate for greater attention by the government.

In 1972, health care professionals, patient and family groups and other advocates successfully lobbied the government and the Diet (parliament) to implement administrative measures to support individuals living with a rare disease, including providing medical expense subsidies and increasing investment in research. However, without specific national legislation, practical implementation was challenging because government funding was not secure from year to year. Local governments were uncertain about how best to prioritize activities to support the principles of the administrative action. As a result, only a limited number of specific diseases were targeted — excluding many people who could benefit from reimbursement for their health care costs and research on their conditions.

NANBYO Act

Overview of program

In 2014, as a result of collaboration among patients and families, advocates, physicians, researchers and policymakers, Japan passed the "Act on Medical Care for Patients with NANBYO " (commonly known as the NANBYO Act) — formalizing government reimbursement of medical services for individuals with rare conditions.

The NANBYO Act seeks to 1) Develop effective treatment methods and improve the quality of medical care for people living with a rare disease; 2) Establish a fair and stable system for providing subsidies for medical expenses, particularly for individuals living with different rare conditions; and 3) Promote public understanding and enhance measures to help ensure the social participation (including employment and welfare) of people living with NANBYO. Based on the "Joint reform of the taxation and social security systems" that the government was promoting at the time, the NANBYO Act was positioned as a sustainable social security benefit that is paid for by the country's increased revenue from consumption (also known as sales) tax.

Patients have had a foundational role in improving the lives of people in Japan with a rare disease and were a major force behind the establishment and passage of the NANBYO Act. They continue to be actively involved in reviewing progress and possible reforms to the legislation as members of the NANBYO Measures Committee, which also includes doctors, experts, government officials and media. A separate Committee for Designated NANBYO considers whether additional diseases should be included.

The annual NANBYO Forum, started in 2010, is a platform for dialogue among patients, their families, policymakers and administrative officials. In the same year, an intermediary organization took on the role of secretariat and launched Rare Disease Day Japan, a grassroots effort to raise public awareness about rare and intractable diseases across the country.

In support of the NANBYO Act's focus on expanding research to better understand unknown conditions, the Japan Agency for Medical Research and Development established the Practical Research Project for Rare/ Intractable Diseases. This group aims to share learnings from Japan with researchers, clinicians and patients globally — ultimately advancing research and driving access to care across the country and around the world.

Results

Since the implementation of the NANBYO Act, the number of diseases that qualify for reimbursement of associated medical expenses has grown from only four in 1972 to 341 recognized diseases in 2024 — a significant increase in the number and proportion of people living with a rare condition who now benefit financially and from more research on their disease.

With greater investment in research, Japan has stepped up its use of advanced technology in medical diagnostics — including next-generation genetic sequencing. Researchers are improving their ability to identify genetic mutations associated with different rare disorders. In the first three years of the Initiative on Rare and Undiagnosed Diseases, researchers analyzed more than 8,000 DNA samples from patients with unexplained illnesses and their family members. The initiative solved the medical mystery of more than a third of all cases it received and has identified the underlying disease-causing mutations for over 1,000 patients with a rare disease.

As a result of the NANBYO Act, there have been notable discoveries in the field of NANBYO. In 2015, researchers at the Center for Medical Genetics at Keio University School of Medicine discovered a genetic mutation causing delayed development and low platelet counts — a condition named after the researchers called Takenouchi-Kosaki syndrome. The next year, researchers at the Yokohama City University Graduate School of Medicine discovered three genetic mutations that cause a rare condition associated with epileptic seizures called West syndrome.[1]

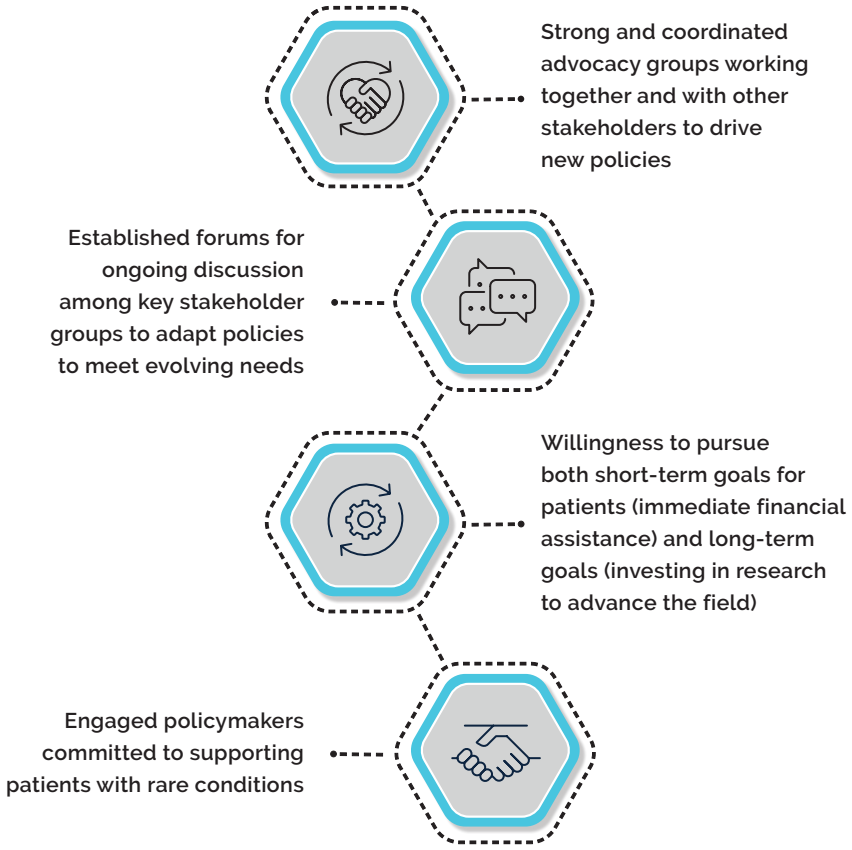
Learnings

- **Engaging diverse stakeholders is key:** Consultation with various groups — health providers, researchers, patients and their families — in the crafting and ongoing review of the NANBYO legislation was critical to secure widespread support for the Act and assure its successful implementation.
- **Diagnosis by itself is not enough:** To ensure better outcomes for patients, the health system must have both the tools to accurately diagnose a rare disease and the resources for patients to get treatment.
- **Ongoing assessment of policy is essential:** A formal mechanism for stakeholders to regularly review and revise the NANBYO Act has helped to ensure that the legislation adapts to meet the needs of additional patient groups.
- **Policy change is the starting point, not the end goal:** Continuous communication through various channels has been important in raising awareness of the NANBYO legislation and how to access subsidized care.

Goals & next steps

Upcoming efforts are diverse and include integrating databases for pediatric and adult NANBYO patients so that information follows a child with a rare disease who becomes an adult living with a rare disease; advocating for NANBYO patients who do not have a disability certificate to be eligible for employment without discrimination under law[2]; reviewing additional target patients based on the diseases eligible for medical expense support retroactive to the 'date of diagnosis of serious diseases' and the severity criteria; and sharing learnings from Japan's health care system and the NANBYO Act with the international community.

Success factors & considerations



[2] Act for Eliminating Discrimination against Persons with Disabilities

References

1. **d42473-019-00037-3.pdf (nature.com)** NANBYO Research: Connecting Japan and Beyond for Improved Patient Care
2. Act for Eliminating Discrimination against Persons with Disabilities