

# Suffering in silence: Assessing rare disease awareness and management in Japan

In November-December 2019, The Economist Intelligence Unit surveyed 100 practising healthcare professionals from Japan, as part of an Asia-Pacific analysis of understanding and challenges faced in managing rare diseases.

Our survey respondents from Japan comprised general practitioners or primary care physicians (44%), specialist physicians (35%), nurses (6%) and pharmacists (14%). The majority of respondents reported working in private (64%) and public or teaching healthcare institutions (16%).

## Results in context: Japan's health system

- Japan's population was estimated to be 126.8m in 2019.<sup>1</sup> Life expectancy in Japan was high at 84 years in 2018. Infant mortality is low (2 deaths per 1,000 live births), however a declining birth rate and increased longevity has resulted in an ageing population that already accounts for more than 50% of healthcare costs. Non-communicable diseases provide the biggest mortality burden (82% of all deaths). Of these, cancer (30%) and cardiovascular diseases (27%) are the leading causes of death.<sup>2</sup>
- Japan is the third-highest healthcare spender, with 10.9% of GDP spent on healthcare in 2018 according to OECD figures. Government spending accounted for 84.1% of total health spending in 2018, with the remainder financed by out-of-pocket spending or private insurance.<sup>2</sup>

- Universal health insurance is provided to all Japan residents through either the National Health Insurance (NHI) or Employees' Health Insurance (EHI) schemes. Co-pay for health services is required.<sup>2</sup>
- Japan has a high number of hospital beds, with an estimated 13.1 beds per 1,000 people. The number of doctors, however, is low at 2.5 per 1,000 people in 2018. Most medical institutions are privately owned and paid via a mixed flat day-rate and fee-for-service system that may encourage inpatient care.<sup>2</sup>

## Prioritising rare disease: Modernising a well-established health policy in Japan

- Japan's rare disease policies date back to the 1970s, but in 2014 new legislation was passed to assist those living with such conditions. A year later Japan ramped up the search for causes and treatments when the newly-founded Agency for Medical Research and Development (AMED) made rare and intractable diseases one of its nine priority areas:
  - o Despite this long history, only 38% of our survey respondents correctly identified that such a policy exists; 44% did not know, while 18% incorrectly reported that there was no such national policy.
  - o When asked which elements a rare disease policy should cover, respondents frequently reported research funding and strategy

<sup>1</sup> United Nations Population Division, World Population Prospects 2019. Estimates 1950–2020.

<sup>2</sup> The Economist Intelligence Unit. Industry Report Healthcare Japan. 3rd Quarter 2019.

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(including drug development), dedicated regulatory pathways, funding for testing and treatment, and clinical diagnosis or management as most important. Respondents also recognised the importance of policy to enshrine social, education and employment rights for people with rare diseases.

- Japan has had two definitions under its rare and intractable disease policy since 2015. Any condition affecting fewer than 50,000 people in the country (roughly 3.9 per 10,000 population) falls into this category. Also included are disorders deemed to be intractable or difficult and affecting up to 180,000 people (14.2 per 10,000).<sup>3</sup>

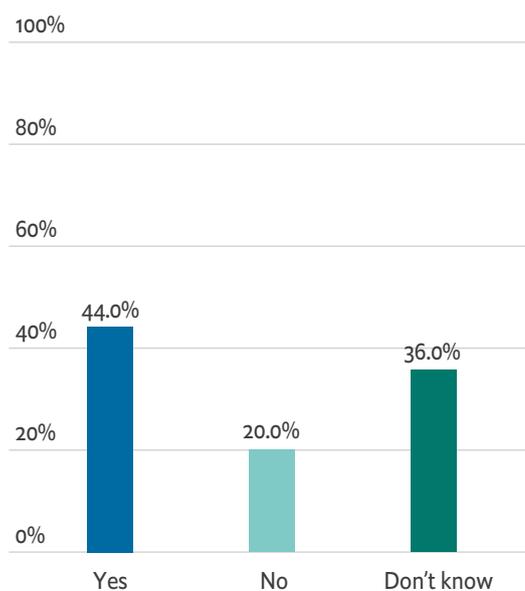
- Only 44% of our survey respondents were aware of Japan's unified rare disease definition (Figure 1). Among those who were aware, only around 50% identified the correct prevalence and inclusion of difficulty in diagnosis and management in the definition.

### Some confidence in knowledge among healthcare professionals

- Japanese healthcare professionals have only middling confidence in their knowledge of rare diseases. Our survey respondents reported an average score of 3.45 out of 5 on their own knowledge, and 3.19 out of 5 for that of their peers.
- Among our survey respondents, 14% reported seeing a new rare disease more than once a month, and 19% see a new rare disease patient present more than once every 6 months. In total, 15% reported never seeing a new rare disease patient present for care.

**Figure 1**

Knowledge of rare disease definitions among physicians in Japan; Survey responses to the question "Is there a unified definition of rare diseases in your market?"



### Defined pathways to diagnosis and overcoming challenges in delivering rare disease care

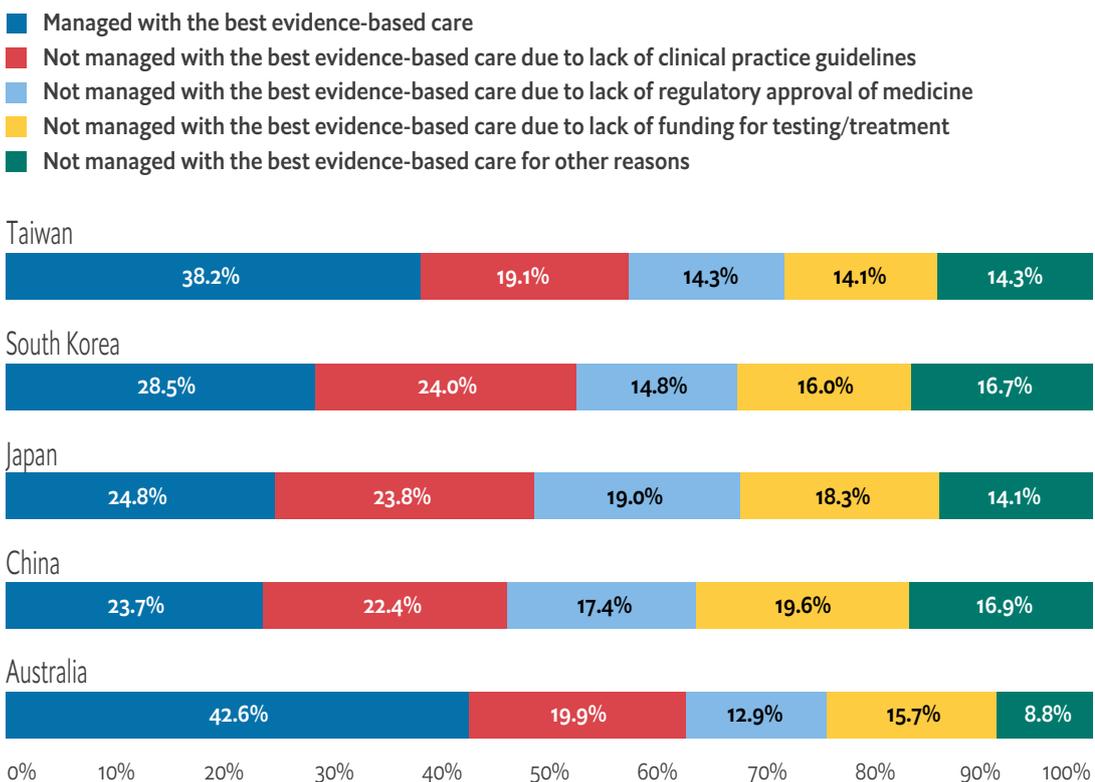
- In Japan, 26.4% of our respondents report that their rare disease patients are currently managed with the best-available care (Figure 2). Lack of appropriate treatment guidelines and lack of approved medications were the most frequent reasons cited for why care is not optimal in all cases.
- The most frequently cited challenges experienced by healthcare professionals in managing rare diseases were:
  - o Availability of information (56.4% reported this as a challenge 'nearly always' or 'always').

<sup>3</sup> Pacific Bridge Medical, "Japan Orphan Drug Update 2017," 2017; Economist Intelligence Unit calculations.

- o Reaching the correct diagnosis (65.9% reported this as a challenge ‘nearly always’ or ‘always’).
  - o Availability of specialist staff (58.8% reported this as a challenge ‘nearly always’ or ‘always’).
  - o Defined referral pathways (50.6% reported this as a challenge ‘nearly always’ or ‘always’).
  - o Communicating with patients (50.5% reported this as a challenge ‘nearly always’ or ‘always’).
  - o Patient group support (55.3% reported this as a challenge ‘nearly always’ or ‘always’).
- Despite these challenges, our Japanese respondents thought their health system was more effective than any other market in terms of arriving at the correct diagnosis, initiation of care, cost and providing overall quality care.
  - When our survey respondents were asked about the one action that should be taken to improve the lives of rare disease patients in Japan, the most common responses were:
    - o Improved education and awareness of rare diseases.
    - o Patient support, social assistance and creating a community around rare diseases.
    - o Financial assistance.

**Figure 2**

Survey responses reporting average proportion of patients managed with the optimal to sub-optimal care in five Asia-Pacific markets.



- Japan established the Initiative on Rare and Undiagnosed Diseases (IRUD) in 2015. The research programme provides a referral pathway to a panel of experts for patients who go undiagnosed for six months and whose conditions appear to be genetic. IRUD is working to expand nationally and increase international collaboration.

### A bigger role for patient organisations to play in Japan

- Among our survey respondents from Japan, 53% did not know if there was an active rare disease patient organisation. 16% incorrectly responded that there was no such organisation. Meanwhile, 55.3% of respondents reported that they experienced difficulty with patient group support nearly always or always when diagnosing and managing rare diseases.
- Despite this low awareness, healthcare professionals in Japan do appear to recognise the potential of patient representation in the rare diseases space:
  - o When considering stakeholders who should be involved in designing care pathways or packages for rare diseases, 77% of respondents from Japan felt that patient involvement was very or most important; 71% felt that family member involvement was very or most important; and 47% felt that patient organisation involvement was very or most important.
- Among our respondents who were aware of the work rare disease organisations are doing in Japan, development and provision of patient education was highlighted as the most successful activity, but one where more should be done. Our respondents wanted to see more from patient organisations across all activities, notably:
  - o Providing screening or genetic counselling services (67.7% of respondents).
  - o Participating in the design of care pathways (51.6% of participants).
  - o Input into research programmes and identifying outcomes for research (61.3% and 64.5% of respondents respectively).
  - o Input into budget distribution decisions (61.3% of respondents).