

Suffering in silence: Assessing rare disease awareness and management in Taiwan

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

Our survey respondents from Taiwan comprised general practitioners or primary care physicians (46%), specialist physicians (34%), nurses (10%) and pharmacists (10%). Respondents were split between private (54.0%) and public (21%) or teaching healthcare institutions (25%).

Results in context: Taiwan's health system

- Taiwan's population was estimated to be 23.7m in 2019.¹ The average life expectancy in 2018 was 80.4 years. Infant mortality is 4.3 per 1,000 live births. Taiwan has stagnant population growth, and its population is ageing at a rapid pace. Cancer, heart disease and pneumonia were the leading causes of death in 2017.²
- Total health expenditure was US\$35.6bn in 2017. The mandatory National Health Insurance (NHI) system—covering all registered Taiwan residents with a comprehensive package of inpatient, outpatient and preventive care—accounts for around half of all health spending. Co-payment is required for all services, and out-of-pocket spending accounts for 33.6% of all health expenditure.²
- There are shortages of key healthcare professionals in Taiwan—many have

left for higher-paid positions overseas—with over 46,000 doctors and 159,000 registered nurses in 2017. There are over 450 hospitals and 10,400 clinics in Taiwan; the majority are privately operated.²

Prioritising rare disease: Health policy goes from strength to strength in Taiwan

- Taiwan has made highly-regarded, long-standing efforts to address rare diseases. The first Rare Disease Control and Orphan Drug Act was passed in 2000. In late 2017, the Rare Diseases and Rare Genetic Disorders Care and Services Plan was introduced.
 - o These policy responses are notable for their focus on not only provision for drug development and medical care, but a broader consideration for patients' rights, legal protection and social care.
- Taiwan does not have a defined number of cases for a disease to be classified as rare. Instead, its Rare Diseases and Medicine Review Committee is authorised to set and regularly review the target prevalence for its policies. Since 2000, any disease with less than one person per 10,000 population is deemed rare.
- Healthcare professionals responding to our survey from Taiwan had the highest awareness of the existence of a unified rare disease definition in any of our study markets, with 83% correctly reporting that one exists (Figure 1).

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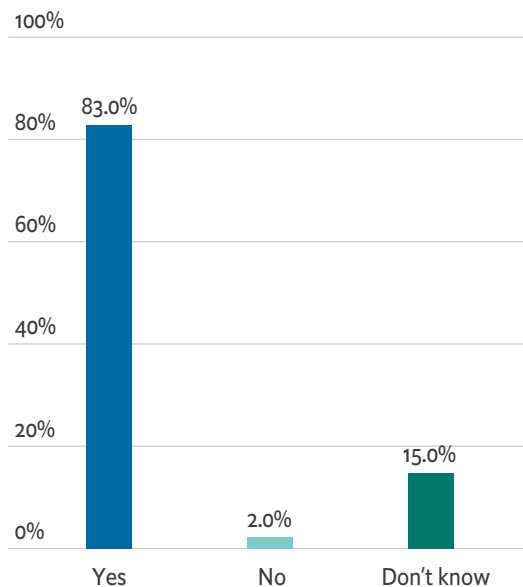
¹ United Nations Population Division, World Population Prospects 2019. Estimates 1950–2020.

² The Economist Intelligence Unit. Industry Report Healthcare Taiwan. 3rd Quarter 2019.

Additionally, 75.9% correctly identified the prevalence cut-off in the definition. Among the small number of respondents who reported there was no unified rare disease definition, the most valued attributes of any definition were reported to be prevalence and difficulty in diagnosis.

Figure 1

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



Less confidence in knowledge and exposure to rare diseases among healthcare professionals in Taiwan

- Healthcare professionals from Taiwan reported seeing a new rare disease patient presenting for care less-frequently than any other market in our study. Only 15% reported seeing a new patient more frequently than once every six months, and 24% reported never seeing such patients.
- Healthcare professionals in our survey rated rare disease knowledge in Taiwan among the lowest of our study markets (an average of 2.69 for themselves and 2.71 for their peers on a scale of 1 to 5).

- o This is in contrast to the high proportion of respondents who reported awareness of various aspects of rare disease policy and care. Whether this lower confidence is due to lack of exposure to such patients or reflects a better grasp of the scope of missing knowledge among a more informed community in Taiwan is unclear.

Prioritising social care for rare disease patients is an important challenge

- Taiwanese respondents report that on average 40.8% of rare disease cases are managed with the best evidence-based care, the second highest in our research (Figure 2).
- Multiple areas were cited by our survey respondents as a challenge in diagnosing and managing rare diseases, most notably:
 - o Reaching the correct diagnosis (56.6% report this is nearly always or is always a challenge).
 - o Access to medicines (59.3% report this is nearly always or is always a challenge).
 - o Funding for diagnosis (48.7% report this is nearly always or is always a challenge).
 - o Funding for treatment (51.3% report this is nearly always or is always a challenge).
 - o Availability of specialist staff (51.3% report this is nearly always or is always a challenge).
 - o Access to ongoing professional training (47.3% report this is nearly always or is always a challenge).

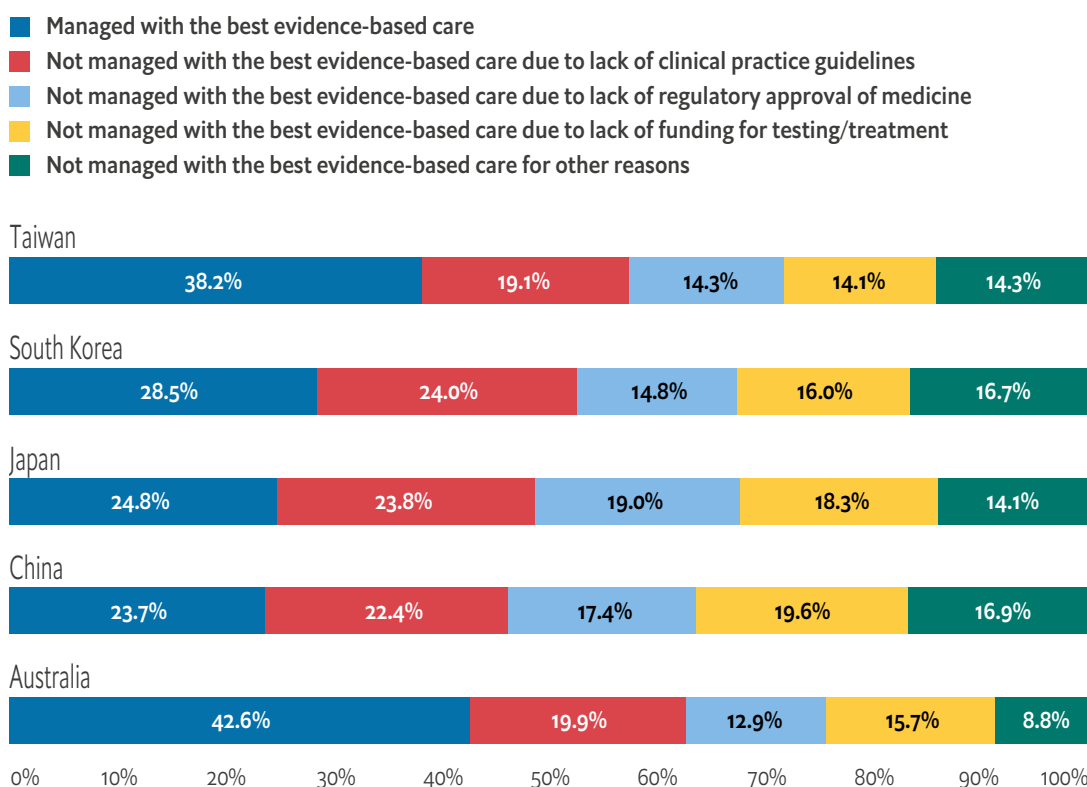
- Among the elements of Taiwan’s health system that survey respondents felt were most effective, arriving at a correct diagnosis, initiation of care and quality of care scored highest. At the opposite end, speed of diagnosis, cost of care and providing social care were reported as least effective.
- When our survey respondents were asked about the one action that should be taken to improve the lives of rare disease patients in Taiwan, the most common responses were:
 - o Increased coverage through health insurance schemes.
 - o Provision of social welfare assistance and rehabilitative services.

Patient organisations in Taiwan are more visible and activities are valued by healthcare professionals

- Awareness of patient organisations among healthcare professionals in Taiwan was the highest in our research; 59% reported correctly that such groups exist, and only 7% reported incorrectly that they did not.
- Among healthcare professionals responding to our survey from Taiwan, 87% and 74% felt that patients and patient organisations, respectively, were important or the most important stakeholders in designing care pathways.

Figure 2

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



- When considering the activities of rare disease patient organisations, our respondents from Taiwan wanted to see more participation across the board. Areas respondents were particularly keen for development and growth in the areas of:
 - o Disease awareness activities (69.5%).
 - o Design of care pathways and provision of patient education (62.7% and 50.8% respectively).
 - o Identifying outcomes for research or designing research (66.1%).
 - o Influencing policy and budget allocation (55.9% and 57.6% respectively).
- One notable patient group, the Taiwan Foundation for Rare Disorders (TFRD), has been active in advocating for rare disease patients with government stakeholders for around two decades and was a contributor to the 2000 Rare Disease Control and Orphan Drug Act. TFRD also plays additional roles such as collaborating with health authorities in screening activities.³

³ Dharssi *et al.* *Orphanet Journal of Rare Diseases* (2017) 12:63 DOI 10.1186/s13023-017-0618-0