

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

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

Our survey respondents from China comprised general practitioners or primary care physicians (44%), specialist physicians (36%), nurses (6%) and pharmacists (14%). The majority of respondents reported working in public hospitals (74%) with 13% working in a teaching hospital, and 8% employed in a private hospital or outpatient clinic.

Results in context: China's health system

- China's population was estimated to be 1.43bn in 2019.¹ The average life expectancy was estimated to be 76 years in 2018, and infant mortality was an estimated 11.8 deaths per 1,000 births—significantly higher than the OECD average of 3.8. China's rapidly ageing population, coupled with its low birth rate, represents an impending healthcare challenge for the government.² Non-communicable diseases account for 85% of deaths in China, with ischaemic heart disease, chronic obstructive pulmonary disease, lung cancer and Alzheimer's the leading drivers of mortality.
- Around 95% of the population has some basic level of health insurance, although 36% of total health expenditure is still

attributable to out-of-pocket spending.² Three public health insurance schemes exist, including the mandatory employment-based Urban Employee Basic Medical Insurance. Voluntary private health insurance remains rare, accounting for just 4% of healthcare spending in 2016 according to the WHO.³

- China has around 3.6m doctors and 4m nurses, with an estimated 2.3 doctors per 1,000 population. There are an estimated 32,000 hospital beds in China, or 3.9 beds per 1,000 population. While 60% of China's hospitals are private, the majority of inpatient care (over 80%) is provided by public hospitals. Top-tier public hospitals provide around 50% of all care, despite representing only 5% of the total number of hospitals.²

Prioritising rare disease: A lack of data to inform policy in China

- China's Centre for Disease Control estimates—given the size of the country's population—that China has the world's largest number of people with rare diseases (16.8m in 2014).³
- China has no formal definition for rare disease, although local medical experts generally accept a cut-off of one in 10,000 population for neonatal diseases, and one in 500,000 for others. Any numerical boundary enshrined in policy would be largely academic due to the lack of epidemiological data.⁴

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

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

³ Peipei Song *et al.*, "Innovative measures to combat rare diseases in China," *Intractable & Rare Diseases Research*, 2017.

⁴ Jiangjiang He *et al.*, "China has officially released its first national list of rare diseases," *Intractable and Rare Diseases Research*, 2018.

Our survey found that 31% of respondents from China did not know if there was a unified rare diseases definition, while 59% reported that one did exist.

- Beyond a low prevalence, the most frequently cited considerations for a unified rare disease definition by healthcare professionals in China were: heredity or genetic basis of the disease; severity or chronic nature; difficulty in diagnosis and financial burden of the disease.
- China's National Health Commission created an Expert Committee of Rare Disease Treatment and Support in 2016 and published the first national list of rare diseases two years later. However, national figures on incidence and prevalence exist for only 14 of the 121 rare diseases on the list which China recently issued.
- In China, policymakers look more narrowly at the possible population benefits from existing medical interventions. Its list of rare diseases therefore focuses on relatively common ones for which some treatment is available.⁵
- An increasing number of orphan drugs are receiving approval and, since October 2019, the large Chinese medical insurance schemes appear to be looking at ways to improve coverage.
- Health authorities are working on a National Rare Diseases Registry System which is projected to cover 50 conditions by 2020.⁶

Healthcare professionals seem more confident in their knowledge despite seeing patients infrequently

- Respondents in our survey rated rare disease knowledge in China with an average of 3.69 out of 5 for their own knowledge, and 3.62 out of 5 for their peers' knowledge.
- More than half (55%) of respondents in China reported seeing a new rare disease patient at least every 6 months, with 16% reporting a new patient presenting on a monthly basis.

Multiple challenges to delivering high-quality care in China

- Respondents from China, on average, give their health system the highest grades for speed and quality of any in the survey. While these healthcare professionals clearly have confidence in the system, this may not reflect the reality for rare disease patients. Respondents reported that only 24.4% of patients receive the best-available care, the lowest proportion in our study (Figure 1).
- In terms of difficulty in diagnosing and managing rare diseases, all categories were considered a challenge more often than not. The median category was 4 (on a scale where 1 is never and 5 is always) for the following elements: availability of evidence; reaching the correct diagnosis; access to medicines; speed of medicine approval; funding for diagnosis or treatment; defined referral pathways; communicating with patients and general populations' knowledge.

⁵ Jiangjiang He *et al.*, "China has officially released its first national list of rare diseases," *Intractable and Rare Diseases Research*, 2018.

⁶ Peipei Song *et al.*, "Innovative measures to combat rare diseases in China," *Intractable & Rare Diseases Research*, 2017.

- Notably, availability of specialist staff was reported as ‘always a challenge’ by 37.0% of respondents.
- When asked what one action should be taken to improve the management of rare diseases in China, responses were split between improving the financial support available, improved government support, and better defining diagnosis and treatment pathways.

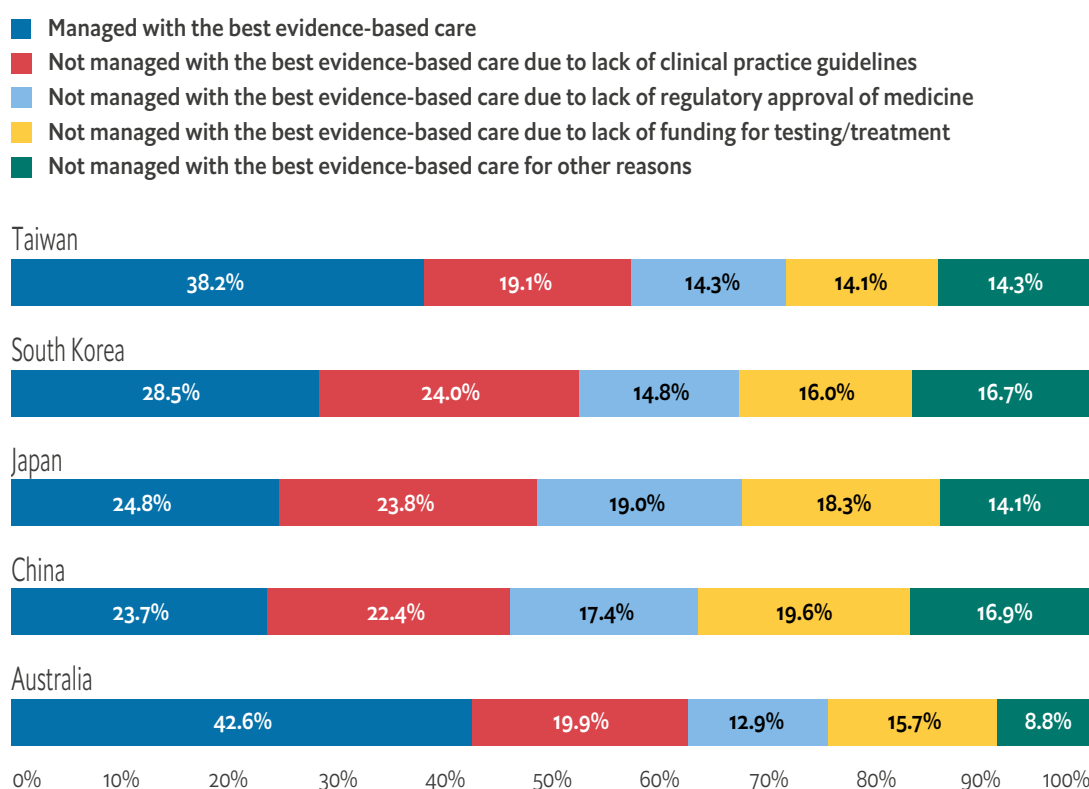
Low awareness of the potential contributions by patient groups

- 41.0% of healthcare professionals responding to our survey did not know if rare disease patient organisations are active in China, and 20.0% incorrectly reported they are not active.

- There are several well-established patient groups working in China, notably the Chinese Organisation for Rare Disorders (CORD) and Illness Challenge Foundation—representatives of whom contributed to our research.
- Broadly, healthcare professionals in China felt that patient organisations should contribute more frequently in all areas (between 61.5% and 69.2% of responses).
 - o Notably, a small but significant proportion of respondents felt that patient groups should not be as involved in matters of designing care pathways (17.9%) and identifying outcomes needing research (15.4%). This finding was not seen in the other markets of study.

Figure 1

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



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