照亮罕見

面對生命無可迴避的磨難，幸賴許多貴人眾志成城，匯聚微光一同為罕病點亮沉幕黑夜。各界觀點眾人智慧，共同守護罕病家庭。

閃閃發亮的夜晚
林冠合（25 歲），威廉斯氏症候群。

What makes the Taiwan Rare Disease and Orphan Drug Act Different from Other Orphan Drug Acts (And the Envy of Patients Worldwide)?

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A few years ago, I was on a panel with Yann Le Cam, the CEO of EURORDIS, and we were asked, "What is the best rare disease patient organization in the world." We both responded, with barely a hesitation, "Taiwan Foundation for Rare Disorders." It's not surprising. Some patient alliances have influenced national rare disease policies and programs; some organizations are "hands-on" with their patient community, subsidizing a range of psychological, welfare and educational services; some organizations facilitate access to medical services, such as testing, diagnosis and specialty care; and others have advocated strongly for access to rare disease treatments. But no other national rare disease organization does it all, as effectively across all of these domains as the Taiwan

Foundation for Rare Disorders.

So, when asked to comment on the 20th anniversary of Taiwan Rare Disease and Orphan Drug Act (TRDODA), I found it only natural to start with TFRD, since there would undoubtedly have been no Act without the patient group's advocacy and support. When passed in 2000, the TRDODA did not receive much international approbation, following shortly after the European Union's highly acclaimed Orphan Drug Act. However, in 2004, the Taiwan Act was acknowledged as potentially a model for orphan drug legislation for small countries. Frankly, I contend that this is a gross understatement since the TRDODA easily constitutes an appropriate template for all jurisdictions.

Taiwan was technically the fifth country to pass legislation in support of therapies for rare diseases but unlike its four precedents (USA, Japan, Australia, and European Union), Taiwan introduced an Act that was not just about orphan drugs but was first and foremost about improving the lives of persons with rare diseases.

Taiwan was also unique in moving swiftly from conception to implementation (four months legislative debate and voilà), although this should not have been surprising given Taiwan's 1995 implementation of its first National Health System in just one year.

Indeed, the success of the Rare Disease and Orphan Drug Act can only be understood against the backdrop of the national health system which, by design, is universal, comprehensive, simple, equitable, recognizing of specialized needs, and inclusive of drug coverage with only nominal out-of-pocket expenses.

Why do we say the TRDODA is perhaps the envy of rare disease patients worldwide? The Act, in just a few articles, addresses prevention, education and awareness, specialized care, support for psychological, social, educational, and financial needs, international collaboration, research, and provisions for orphan and non-orphan drugs. Looking back, it was eerily prescient about the need for policy, infrastructure, and health and social systems to effectively manage orphan therapies. It was not until 2009 that the European Union mandated its member nations to develop comprehensive National Plans for Rare Diseases (over a period of five years).

Perhaps the two most important aspects of the TRDODA are, first, it has produced significant actions that are mostly collaborative, feasible, impactful, accessible, and, sustainable, and, second, the patient organization has remained a valued and indeed an essential partner. Many of the government services are complemented or supplemented by those provided through the Foundation. An

example is the Newborn Screening Program for rare genetic conditions, which was expanded by the government following concerted advocacy by TFRD, and access is still subsidized by the Foundation for aboriginal and low-income families. In support of families identified through NBS, the TFRD has also stepped in to increase genetic counseling services and nutritional counselling.

So, what could be next for rare disease in Taiwan? I would like to offer two suggestions. First, while many thousands of Taiwanese families living with rare diseases have benefitted from the combined efforts of the government through the Rare Disease Act and the Foundation, there is much more that needs to be done, for example, reduce the time to diagnosis especially for difficult to diagnose and ultra-rare conditions, improve access to timely comprehensive care especially for those living beyond major healthcare centres, and improve availability of essential supportive services such as wheelchairs, assistive technologies for speaking, hearing, and seeing, rehabilitation and physical therapy, psychological and mental health services, and support for carers, including respite and homecare services. Most importantly, all of these initiatives need to be continuously advised by the patient community to assure integration and prioritization of needs.

Second, as Chair of Rare Diseases International, Vice-Chair of the Asia Pacific Alliance of Rare Disease Organizations, and Patient Advisor to the APEC Rare Disease Network, I would urge Taiwan Health and TFRD to take a more prominent international role, firstly to share the Rare Disease and Orphan Drug Act and to facilitate, where feasible, the development and implementation of rare disease and orphan drug legislation in other countries. Secondly, Taiwan is ideally situated to foster collaboration across sectors and countries, for example, in rare disease research, clinical excellence, medical and psycho-social training, patient registries, and orphan drug regulatory review and assessment. In a time and climate where COVID-19 is positioned to drive all healthcare policy and resources across the globe, Taiwan has emerged as a model of how a country can manage a pandemic and also maintain services to even very small patient populations.

On the 20th anniversary of Taiwan's Rare Disease and Orphan Drug Act, I congratulate all those involved on what has been achieved but more importantly I look forward to what is to come in the next 20 years. I hope that Taiwan will bring the rest of the region and world along on that journey.