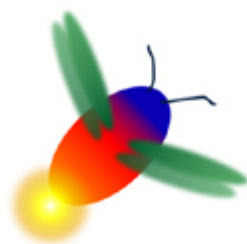


Spotlight on...

How specific legislation for rare diseases and orphan drugs is changing life for some patients in Taiwan...



財團法人罕見疾病基金會
TAIWAN FOUNDATION FOR RARE DISORDERS

In Taiwan, the Rare Disease and Orphan Drug Act was implemented in 2000 - the fifth such legislation to be adopted worldwide. This legislation offers support and assistance to patients with rare diseases who need medical treatment, encourages rare disease research, and raises public awareness. With the establishment of the Committee for the Review of Rare Diseases and Orphan Drugs, the government has classified 167 diseases under the rare disease category thereby protecting patients whose ailments are now included in National Health Insurance coverage for major diseases and injuries and whose co-payment can be waived. Furthermore, medicinal products for 81 rare diseases and special nutrient supplements for 40 rare diseases have been reviewed, established, and announced, and additional medical cases to be subsidised have been reviewed.

For the diagnosis, treatment, and drug costs for rare diseases that are not covered under the National Health Insurance Act, Article 33 of the Rare Disease and Orphan Drug Act is followed, and the Bureau of Health Promotion appropriates a budget in the form of subsidies. With the establishment of a logistics centre for rare disease special nutrient supplements and drugs, 32 items were supplied in 2008 to 17 hospitals and used for 303 patients with rare diseases. The subsidized budget reached over NT\$27.7 million (€600,000). In addition, ten emergency drugs for patients with rare diseases were supplied to hospitals for diagnostics and treatment. In 2008 thirteen patients used these drugs and the budget subsidised was NT\$360,000 (€8000).

Providing international medical laboratory referral services for rare disease cases, information in Taiwan concerning the referrals of samples to international laboratories was integrated and established to provide international medical collaboration channels for rare diseases. The government and patient alliance the Taiwan Foundation for Rare Disorders subsidised 40% of the referral test costs. Between 2000 and 2008, a total of 312 cases were subsidised. In addition, rapid review principles for 12 rare diseases were formulated to shorten the review process for international laboratory referrals.

Construction of complete medical service networks for genetic and other rare diseases is underway via the establishment of a rare disease reporting database and single-window inquiry service. By the end of 2008, a total of 3,314 cases of rare diseases had been reported from hospitals, and genetic consultation centers were established in northern, central, southern, and eastern parts of Taiwan (10 medical centers) to provide necessary assistance.

In order to obtain the benefits offered under the country's Rare Disease and Orphan Drug Act, patients in Taiwan must submit via their physician or medical facility a rare disorders report sheet (including suspected cases), along with an abstract of the disease, and related medical tests, to the Bureau of Health Promotion, Department of Health, Executive Yuan. Patients officially acknowledged as having a rare condition may then apply for the reimbursement of medical expenses incurred in their local medical centre or one of the country's regional teaching hospitals. Expenses covered include diagnostics, treatment, medicinal products, and special nutritional supplements. The reimbursement cap is 70% of actual expenses but families who qualify for low-income status can receive reimbursements up to 100% for drugs and nutritional supplements for the patient.

***"We can't take care of our children forever,
but a well-established system can."***

-The Taiwan Foundation for Rare Disorders

The Taiwan Foundation for Rare Disorders – championing the cause

Established in 1999 by two parents of children with rare diseases, the Taiwan Foundation for Rare Disorders (TFRD) championed the adoption of the Rare Disease and Orphan Drug Act. The patient advocacy group has the mission of improving the life of rare disease patients by assisting rare disease patients to receive proper medical treatment and rehabilitation, securing orphan drugs and special nutrients and fulfilling patient needs in terms of education, employment and other activities.

The TFRD undertakes or participates in the provision of patient subsidies; genetic counselling; transferring specimens abroad for diagnosis; providing nutritional counselling; performing workshops; arranging scholarships; organising patient activities and tours; fostering patient groups and formal associations; and holding public and campus advocacy activities. Financed largely by donations from the public and from enterprises, the foundation has established several subsidy funds in order to ease the financial burden placed on the families of rare disease patients. These help compensate in cases where medical insurance is lacking, or can be used to aid in emergency situations, and to assist patients who need adapted in-home and in-center services. Furthermore, for parents who have given birth to a baby with a rare disease and who are planning to have another child, TFRD has available a care assistance subsidy for prenatal examinations. In 2008, TFRD distributed funding for 99 cases of medical aid, 81 cases of livelihood assistance, 43 cases of prenatal services, and 81 cases of in-home and long-term patient care. The total amount of subsidies distributed came to \$NT9,366,891 dollars (€204,000).

Genetic and nutritional counselling

Since 2001, together with the Taiwan Human Genetic Society, the National Taiwan University Hospital, and the Mackay Memorial Hospital, the TFRD has integrated the Human

Resources subsidies fund, as provided by the Council of Labor Affairs, Executive Yuan, to conduct a one-year Genetic Counseling Training Program. Graduating students from areas relevant to genetic studies are hired as genetic counsellors. They make clinical rounds at the National Taiwan University Hospital and Mackay Memorial Hospital and receive on-site training by genetic specialists. Additionally, they assist patients and doctors with relevant services, connecting patients and hospital workers. The foundation also set up the "National Rare Diseases Service Center" and plans to promote such cooperative models to the genetic health counselling centers in Taiwan and set up an initial genetic counselling network. To date, eleven genetic counselling centres have been established in various medical centers and teaching hospitals in Taiwan. The Bureau of Health Promotion has evaluated the facilities to assure the quality of the genetic counselling centres.

To help patients with genetic metabolic disorders and their families, the TFRD regularly conducts "Nutritional Classroom" workshops to demonstrate the preparation of different foods, and educate the patients through basic nutritional facts. For patients who have restricted choices of food, the TFRD published the "Nutrition Handbook" which provides patients, free of charge, with nutritional information and recipes suitable to their disease and dietary restrictions. There are currently handbooks for phenylketonuria, and maple syrup urine disease, glycogen storage disease type I, amino acid metabolic disorders, homocystinuria, disorders of the urea cycle, and disorders of leucine catabolism.

In 2003, the TFRD introduced nutritional low protein substitutes such as Loprofin protein mix, egg, and egg whites. These allow patients to make low protein desserts on their own. Another item on the list is MTC oil which can also be consumed by patients. In 2008, the group distributed "Low-Protein Food Coupons" specifically for patients with certain genetic metabolic disorders, such as phenylketonuria, maple syrup urine disease, amino acid metabolic disorders and homocystinuria. These coupons can be used to obtain special low-protein foods such as noodles or rice. Patients only have to pay shipping costs.

Activities and support for the patient and families

In order to support the inner strength of patients and help them adapt psychologically to daily life, the TFRD has established a Performing Workshop that meets once a week and consists of singing and painting. The workshop has a dual purpose. There are benefit performances and exhibitions for social interaction, and the activities can also help the patients build self-esteem as well as reducing physical pain and psychological pressure. In 2008, the TFRD Taipei office held a 26-week painting class for more than 47 patients and their families and a 26-week singing class for more than 59 participants. In March 2008, TFRD's Taichung and Kaohsiung branch offices also began offering 21-week singing classes for patients and their families. Each has over 45 participants.

Patients who can perform may join the Heavenly Melody Chorus and give performances to the public on invitation. The most exciting thing for the patients was the concert tour held in August and September, during which patients had the chance to stand on the stage and perform to an audience of over 2,000 spectators.

In order to strengthen the rare disease family and help the whole family to face living with a rare disease, the TFRD has arranged a series of family support courses since 2003. Topics include children's education, communication between children and parents, emergency medical care, and life education. Together with volunteers, medical personnel and families, in 2003 the TFRD conducted the first ever Firefly Family Summer Camp, which was very well

received. TFRD has since been providing various activities including one-day and two-day trips. In 2008 the foundation held five additional activities for patients, who got the opportunity to go to the baseball stadium to meet professional baseball players, to rice fields to experience traditional sowing, and to a tea garden to taste various teas. With the help of more than 150 volunteer workers, 599 patients and their families have participated in these recreational activities.

Public and campus advocacy activities

The TFRD strives to raise public awareness of rare disorders through education, advocacy, and media coverage in order to win respect and concern from the general public. Different venues have been exploited including rare disorder symposiums, literature, drama, television announcements, and broadcast programmes. In 2008, TFRD cooperated with various organisations, schools, and enterprises to promote public advocacy. The Hsin Love World Broadcast Programme (featured in [OrphaNews Europe 21 November 2007](#)) is exclusively sponsored by the Kuan-Shu Education Foundation. Broadcast by both ZhengShen and BCC stations, the show features rare disease patients who meet with celebrities and share their stories, experience and thoughts on many diverse topics - love, life, death, family, art, food and travel, for example. The programme won the 2008 Golden Bell Award for best broadcast hostess and was nominated for the best broadcast programme.

More than 104 rare disease activities and speeches were held in 2008 with the support of enterprises and organisations such as universities, rotary clubs, the National Taiwan University of Science and Technology EMBA Alumni Club, Nu Skin, ING Insurance, Chinese Professional Baseball League, and others. Also in 2008, more than 20 media interviews were conducted to strengthen public awareness on rare disease issues. There were 195 news reports on or relevant to rare diseases. For children with rare diseases and their parents, entering school requires preparation in order to choose between regular and special classes and to select the most appropriate school. The TFRD started a "Schooling Counseling Service" which provides a pre-school conference that integrates relevant information and educational resources. Parents afterwards have more information when selecting the most suitable school for their children. For rare disease patients who attend school, the TFRD holds campus advocacy speeches geared toward classmates and teachers. It is hoped that these can reduce unfriendly and/or unfair treatment stemming from a lack of information regarding rare diseases and also help the patients to better enjoy their school life.

The TFRD looks forward to continuing working in tandem with the country's Rare Disease and Orphan Drug Act to bring relief to the country's rare disease patients and their families.