

- 1 Anon. Progress in global measles control and mortality reduction, 2000–2007. *MMWR Morb Mortal Wkly Rep* 2008; **57**: 1303–06.
- 2 WHO. Outcome of the sixty-first World Health Assembly resolution: global immunization strategy. May 26, 2008. [http://apps.who.int/gb/ebwha/pdf\\_files/EB123/B123\\_2-en.pdf](http://apps.who.int/gb/ebwha/pdf_files/EB123/B123_2-en.pdf) (accessed Nov 8, 2009).
- 3 LiCalsi C, Maniaci MJ, Christensen T, Phillips E, Ward GH, Witham C. A powder formulation of measles vaccine for aerosol delivery. *Vaccine* 2001; **19**: 2629–36.
- 4 Sabin AB, Flores AA, de Fernandez CJ, et al. Successful immunization of children with and without maternal antibody by aerosolized measles vaccine I: different results with undiluted human diploid cell and chick embryo fibroblast vaccines. *JAMA* 1983; **249**: 2651–62.
- 5 Bellanti JA, Zeligs BJ, Mendez-Inocencio J, et al. Immunologic studies of specific mucosal and systemic immune responses in Mexican school children after booster aerosol or subcutaneous immunization with measles vaccine. *Vaccine* 2004; **22**: 1214–20.
- 6 Khanum S, Garelick H, Uddin N, Mann G, Tomkins A. Comparison of Edmonston-Zagreb and Schwarz strains of measles vaccine given by aerosol or subcutaneous injection. *Lancet* 1987; **1**: 150–53.
- 7 Sabin AB. My last will and testament on rapid elimination and ultimate global eradication of poliomyelitis and measles. *Pediatrics* 1992; **90**: 162–69.
- 8 Hiremath GS, Omer SB. A meta-analysis of studies comparing the respiratory route with the subcutaneous route of measles vaccine administration. *Hum Vaccin* 2005; **1**: 30–36.
- 9 Low N, Kraemer S, Schneider M, Restrepo AM. Immunogenicity and safety of aerosolized measles vaccine: systematic review and meta-analysis. *Vaccine* 2008; **26**: 383–98.
- 10 WHO. The Initiative for Vaccine Research: strategic plan 2006–2009. 2006. <http://www.who.int/vaccines-documents/DocsPDF06/854.pdf> (accessed Nov 8, 2009).
- 11 WHO. Measles Aerosol Project. 2009. [http://www.who.int/immunization\\_delivery/new\\_vaccines/technologies\\_aerosol/en/index.html](http://www.who.int/immunization_delivery/new_vaccines/technologies_aerosol/en/index.html) (accessed Nov 9, 2009).
- 12 Tosh PK, Boyce TG, Poland GA. Flu myths: dispelling the myths associated with live attenuated influenza vaccine. *Mayo Clin Proc* 2008; **83**: 77–84.
- 13 Fernandez-de CJ, Kumate-Rodriguez J, Sepulveda J, Ramirez-Isunza JM, Valdespino-Gomez JL. Measles vaccination by the aerosol method in Mexico. *Salud Publica Mex* 1997; **39**: 53–60 (in Spanish).

## Rare diseases and legislation in China



First national conference for rare diseases in Beijing, 2009

Public awareness of rare diseases is increasing in China. People with rare diseases and their families, patients' advocacy groups, health-care professionals, lawyers, and representatives of the People's Congress are working together to establish a Rare Diseases Prevention and Treatment Law. On the basis of WHO's definition of a rare disease, at least 10 million people are living with rare diseases in China.<sup>1</sup> This estimate seems conservative for a population of more than 1.3 billion in China.

Rare diseases in China include osteogenesis imperfecta, neuromuscular diseases, Fabry's disease, Gaucher's disease, phenylketonurias, haemophilia A and B, lymphangioliomyomatosis, albinism, and acromegaly.<sup>2</sup> Unfortunately, Chinese patients with such diseases generally do not have access to appropriate health care, especially orphan medicines. Very few agents used for rare diseases in developed countries have entered the Chinese formulary, and few such drugs are affordable without a supportive policy or health-care insurance. Consequently, many patients are unable to fulfil activities of daily living, and might lose their jobs, become disabled or isolated, and even die prematurely.

Aymé and co-workers<sup>3</sup> discussed the role of patients' advocacy groups—such groups have largely contributed to supporting patients with rare diseases, establishing policies, and addressing patients' unmet needs. The Chinese organisations for rare disease are also engaged in development and dissemination of information, increasing public awareness, raising funds, and

improving patients' access to health care. These groups include the Home of Babies of the Moon—the China Albinism Association, the Haemophilia Home of China (HHC), the Neuro-Muscular Disease Association of China, the China Organisation of Lymphangioliomyomatosis (LAM-China), and the China-Dolls Care and Support Association. Some organisations have collaborated nationally and internationally, and the disease-specific groups are planning to form an alliance in China.

The China-Dolls Care and Support Association was founded in 2007, and is a non-profit, non-governmental organisation for people with osteogenesis imperfecta. The word china has a dual meaning. One is porcelain, signifying that these patients are as fragile as porcelain; the other is the country China, emphasising those with this disease are Chinese citizens, and cannot be ignored and should not be discriminated against.<sup>4</sup> The slogan of the organisation is "Bones are brittle, but our love is still tough". The organisation is most active in bringing public attention to rare diseases in China. On Rare Disease Day, Feb 28, 2009, the association convened a symposium to discuss legislation on rare diseases in China.<sup>5</sup> With strong support from the China-Dolls Care and Support Association and the China Social Welfare Education Foundation (CSWEF), the first national conference of osteogenesis imperfecta patients was held in November, 2009, in Beijing. About 200 patients with this disease and other rare diseases attended the meeting. The meeting covered a range of issues from

medical care, education, and employment, to policy and law. During the conference, there were various activities such as patient's training, a volunteer medical service, focus group meetings, and tours. With the advent of the third Rare Disease Day on Feb 28, 2010, the China Charity Federation's assistance foundation for rare diseases, together with CSWEF's China-Dolls Fund for Rare Disorders, is advocating a series of campaigns, called Rare Disease Month, to inform people about, and draw special attention to, rare diseases.<sup>6</sup>

The activities of rare disease organisations in China have boosted public and governmental concerns. However, China has no legislation for rare diseases, lagging far behind the USA, the European Union, Australia, Singapore, Japan, South Korea, and Taiwan. There were only two provisions for rare diseases and orphan drugs in the 1999 edition of Chinese drug-registration regulation, and the most recent 2007 edition just provides unchanged policies. One of the provisions deals with special needs for clinical trials for new orphan-drug development; that is, the sponsoring company can reduce the sample size of clinical trials and even apply for trial exemption for orphan drugs. The other provision states that new orphan drugs with substantial clinical advantage might get special new-drug review and approval.<sup>7</sup> Unfortunately, Chinese pharmaceutical companies have no such drugs in development.

A Bill of rare diseases is under review by the National People's Congress (NPC) of China. The Bill and its accompanying proposal were developed with collaboration from patients, advocacy groups, and representatives of the NPC. The proposal describes the burden of rare diseases in China and the difficulties patients face, and suggests solutions. Proposals include: definition of Chinese rare diseases; establishment of reimbursement mechanisms and succour networks for screening, prevention, diagnosis, and treatment of rare diseases; importation of orphan drugs proactively by governmental agencies instead of passively waiting for drug applications from foreign companies; and support and encouragement of new drug research and development from the native pharmaceutical industry.<sup>8</sup>

China is reforming the national health-care system and health-insurance plans with an initial 3-year (2009–11) budget of about US\$124 billion.<sup>9</sup> We believe legislation for rare diseases and orphan drugs is also a

high priority.<sup>10</sup> We hope the proposed Bill is considered seriously and passed by the NPC, so that millions of Chinese people with rare diseases can benefit from health insurance and treatment alternatives with orphan drugs. We look forward to seeing more orphan drugs with promising safety, efficacy, and cost-effectiveness profiles developed by both multinational and local companies under the rare disease law's protection and stimulation. This situation will be win-win for both the drug industry and patients with rare diseases. As has been said before for rare diseases, "It is now time for action".<sup>11</sup>

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- 1 Ling H, Liu D. People with rare diseases need immediate concerns: China Charity Federation's succour action for people with rare diseases initiated. *China Med Herald* 2009; **6**: 4 (in Chinese).
- 2 Zhang YJ, Wang JB, Guo JJ. The US Orphan Drug Act and its impacts on drug research and development. *Chin J Pharm Econ* (in press).
- 3 Aymé S, Kole A, Groft S. Empowerment of patients: lessons from the rare diseases community. *Lancet* 2008; **371**: 2048–51.
- 4 China-Dolls Care and Support Association. About the China-Dolls Care and Support Association. [http://www.rarediseaseday.org/2009/country/result?country\\_id=cn](http://www.rarediseaseday.org/2009/country/result?country_id=cn) (accessed Jan 22, 2010).
- 5 China-Dolls Care and Support Association. Seminars on rare diseases legislation. Feb 28, 2009. [http://www.rarediseaseday.org/2009/events/show/id/134/country\\_id/cn](http://www.rarediseaseday.org/2009/events/show/id/134/country_id/cn) (accessed Jan 24, 2010).
- 6 China Charity Federation. Call for campaigns for 2010 rare disease month. <http://www.chinacharity.cn/gyzx/tlk100113.aspx> (accessed Jan 24, 2010) (in Chinese).
- 7 China State Food and Drug Administration. Provisions for drug registration. 2007. <http://eng.sfd.gov.cn/cmsweb/webportal/W45649039/A64028429.html> (accessed Jan 23, 2010).
- 8 Sun ZQ. Proposal for expediting the assistance to people with rare diseases. March 10, 2008. <http://sunzhaoqi08.blog.sohu.com> (accessed Jan 24, 2010) (in Chinese).
- 9 Chen Z. Launch of the health-care reform plan in China. *Lancet* 2009; **373**: 1322–24.
- 10 The Lancet. Making rare diseases a public-health and research priority. *Lancet* 2008; **371**: 1972.
- 11 Remuzzi G, Garattini S. Rare diseases: what's next? *Lancet* 2008; **371**: 1978–79.