

The Ice Bucket Challenge: The public sector should get ready to promptly promote the sustained development of a system of medical care for and research into rare diseases

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Summary

In order to promote public awareness and raise charitable donations for patients with amyotrophic lateral sclerosis (ALS), a charity activity known as the "Ice Bucket Challenge" went "viral" from social media in the US to the rest of the world in the summer of 2014. The Challenge had an obvious impact with a large number of participants and increasing charity donations. However, the effort has also garnered criticism for wasting water, possible safety concerns, and its status as a publicity stunt or grandstanding. A system of medical care for and research into rare diseases has been established in some countries in order to protect the rights and interests of patients with rare diseases, but such systems have yet to be established in other countries. An activity like the "Ice Bucket Challenge" is clearly not enough to improve the plight of patients with ALS or other rare diseases. However, the public awareness attracted by this challenge may provide the impetus for those countries that lack a system of medical care for and research into rare diseases to establish such a system. The public sector should bear the responsibility for taking on the important task of promoting the sustained development of a system of medical care for and research into rare diseases.

Keywords: Amyotrophic lateral sclerosis, rare diseases, orphan drugs, health insurance system

What is the hottest event on the Internet this summer? The answer would be the "Ice Bucket Challenge." Over the past two months, social networking platforms have been saturated with videos of people dumping buckets of ice water on their heads in order to promote public awareness and raising charitable donations for patients with amyotrophic lateral sclerosis (ALS). After it first started in the US, the challenge has gone "viral" through social media to become the most successful and influential fund-raising event, and the challenge has even spread as far as countries like China, South Korea, Japan, Germany, and France.

The simple rule of the challenge is to "either donate \$100 to a given cause or douse yourself with ice water, film it, and pass the challenge on to 3 people *via* social

media." Participants like celebrities, political figures, and business leaders have experienced the "freezing" water, and the challenge is still spreading. Actually, this challenge had nothing to do with ALS originally, it came from a dare that was circulating among a group of professional athletes with the rule of whoever refused to take an ice bath had to give \$100 to a charity of the challenger's choosing. Currently, however, the challenge has provided a windfall of charitable donations for ALS patients.

Why would a public charity activity catch the attention of people worldwide? The activity features low barriers to entry, a strong sense of participation, and self-aggrandizement as more and more people post videos of themselves on social networking platforms taking the "Ice Bucket Challenge". The activity has also been helped by celebrity appeal. Furthermore, being drenched with ice is an alternative to contributing actual money, so the lack of a required donation encourages the vast spread of the challenge, expressing shared values and communicating personal information about the participants. The challenge has had a profound impact.

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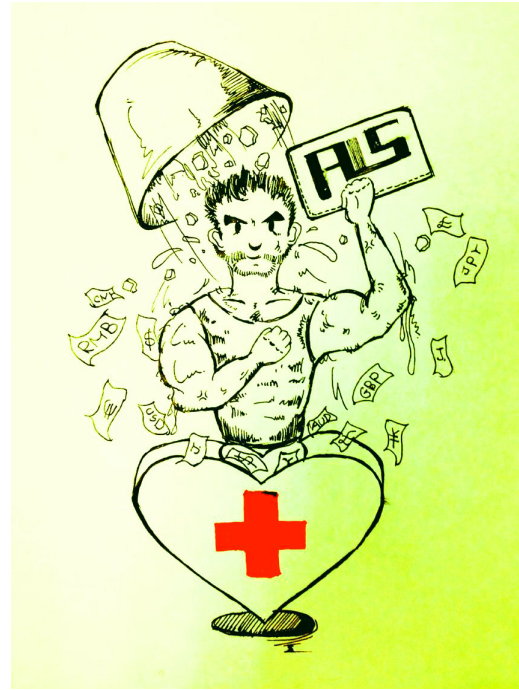
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As of August 25, the ALS Association in the US had received \$79.7 million in donations compared to \$2.5 million during the same time period last year (July 29 to August 25) (1).

However, the "Ice Bucket Challenge" has received mixed reviews as it has spread rapidly around the world. Though there are many people who support the challenge, others criticize it for wasting water, possible safety concerns, and its status as a publicity stunt or grandstanding. The challenge in China is a good example. The challenge resulted in charitable donations of RMB 6.94 million yuan for ALS patients, 4.01 billion pageviews, and 3.72 million discussions prior to August 25 (2,3). However, few of the videos appear to contain any substantive information about ALS, such as why the money is needed or how it will be used. So to what extent could this activity help patients with ALS? More importantly, when the "Ice Bucket Challenge" fever eventually dies down, who will fill the "vacancy" and maintain attention on and support patients with ALS or other rare diseases? These are thought-provoking questions.

The ALS Association has stated that raising public awareness and improving research into ALS are much more important than donations in the long term (4). Researchers of rare disease have indicated that countries that lack a system of medical care for and research into rare diseases need to promptly institute the following changes: *i*) a specific definition and classification of rare diseases; *ii*) specific legislation to encourage discovery and development of orphan drugs; *iii*) government-funded special biomedical research programs to enhance basic and applied research on rare diseases; and *iv*) patients' advocacy organizations and disease registry networks to provide vast information on rare diseases (5-7).

Although an amusing charity activity may be enjoyable, rare diseases including ALS pose a serious challenge in China. To date, there is still no official definition of rare diseases in China and little official information about rare diseases is available. According to the World Health Organization (WHO), rare diseases are rare and often debilitating or even life-threatening diseases or conditions with a prevalence of 0.65-1% (7). Based on this standard, there are about 5,000-6,000 rare diseases in China, and the number of Chinese patients with ALS in particular may be as high as 20,000. Over the past decades, many nonprofit patients' advocacy organizations have thoroughly publicized rare diseases, but most Chinese have paid little attention to rare diseases. China has not been home to a noticeable public event like the Ice Bucket Challenge for ALS patients. The lack of a specific definition of rare diseases, the lack of legislation concerning rare diseases, and the lack of a system of medical care for and research into rare diseases have hampered protection of the rights and interests of patients



(Illustration by Jing Fan)

suffering from rare diseases.

Currently, orphan drugs – the medicinal products intended for the diagnosis, prevention, or treatment of rare diseases – are a major facet of how rare diseases are dealt with. Worldwide, the orphan drug legislation and supporting policy measures have been formulated and implemented in some countries and regions, such as the US, Europe, Japan, and Australia. The incentives of financial subsidies, market exclusivity, tax credits, fee waivers, fast track approval, and protocol assistance have resulted in substantial improvements in the treatment of patients with a range of rare diseases (6,7). China, however, lacks systematic economic and regulatory incentives to encourage the development of drugs for rare diseases, so few orphan drugs have been discovered by pharmaceutical companies. Orphan drugs are highly expensive and most are not covered by the health insurance system. Moreover, strict rules on importing orphan drugs and the relative delay of research and development of orphan drugs have resulted in patients with rare diseases becoming a neglected vulnerable group in China.

Over the past few years, calls to draft legislation on rare diseases and protect the rights and interests of patients with rare diseases have increased in China. Who should be responsible for taking on this important task? Based on the experience of those advanced countries with a system of medical care for and research into rare diseases, the public sector should get ready to promptly promote the sustained development of a system of medical care for and research into rare diseases. The public sector should formulate a specific definition of rare diseases, draft specific legislation on orphan drugs,

and improve the health insurance system. The public sector should also provide sufficient support in terms of basic medical assistance, education, employment, and facility improvements to help and care for patients with rare diseases.

In conclusion, an activity like the "Ice Bucket Challenge" is clearly not enough to improve the plight of patients with ALS or other rare diseases. However, the public awareness attracted by this challenge may provide the impetus for those countries that lack a system of medical care for and research into rare diseases to establish such a system. The public sector should bear the responsibility for taking on the important task of promoting the sustained development of a system of medical care for and research into rare diseases.

References

1. ALS Association. Ice Bucket Challenge Donations Reach \$79.7 Million. <http://www.alsa.org/news/media/press-releases/ice-bucket-challenge-082514.html> (accessed August 25, 2014)
2. Sina Weibo. Ice Bucket Challenge. <http://gongyi.weibo.com/r/215182> (accessed August 25, 2014)
3. Sina Weibo. Ice Bucket Challenge. <http://www.weibo.com/> (accessed August 25, 2014)
4. ALS Association. The ALS Association Research Program. <http://www.alsa.org/research/> (accessed August 24, 2014)
5. Tang W. Hopes for intractable and rare diseases research. *Intractable Rare Dis Res.* 2013; 2:1-2.
6. Song PP, Gao JJ, Inagaki Y, Kokudo N, Tang W. Rare diseases, orphan drugs, and their regulation in Asia: Current status and future perspectives. *Intractable Rare Dis Res.* 2012; 1:3-9.
7. Song PP, Gao JJ, Inagaki Y, Kokudo N, Tang W. Intractable and rare diseases research in Asia. *Biosci Trends.* 2012; 6:48-51.

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