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Rainbow Across Borders

Communication strategies for rare diseases

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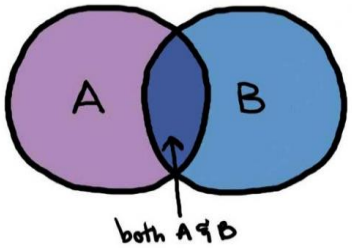
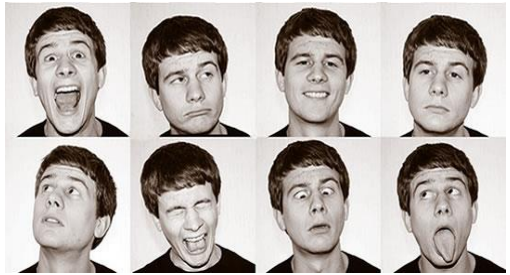
How do we communicate?

In writing

Verbally



Using pictures



Expression/
emotions

Taking
action

Listening

How do you communicate when...

You want to reach lots of people

Traditional pharma 'broadcast' model

Pharmaceutical company

Predominantly unidirectional information flow for 'blockbuster' drug

Large HCP 'audience'

How do you communicate when...

You want to reach a small number of people

You have only limited information

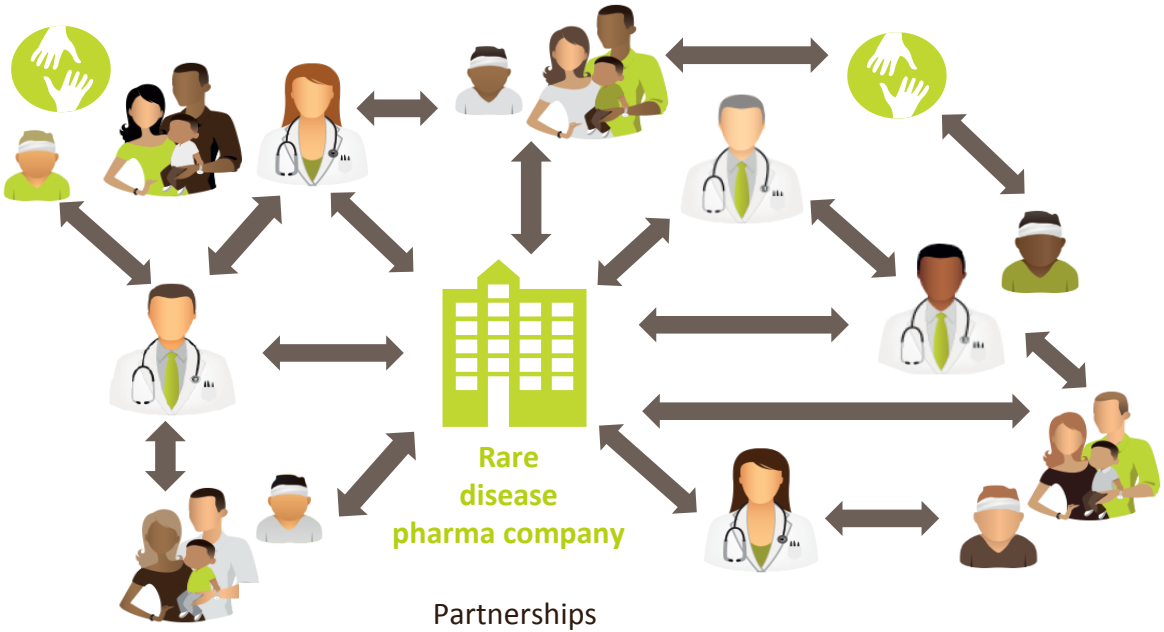
Challenges for rare disease communications

- Complex pathologies, with limited disease information
- Difficulty identifying patients
- Small pools of experts and lack of clinical guidelines
- Fragmented care pathways
- Limited treatment options
- Barriers to patient access

Rare disease network model

Fewer but multiple partners:

- HCPs
- Patients
- Families
- PAGs



Partnerships
Networked communications
Bidirectional information flows
Patient-centred care

The RARE communications model

Research excellence	Collate all available information Work in partnership to improve understanding of all aspects of the disease
Advocacy	Raise awareness of the issues facing those affected Understand the perspectives of those presenting barriers, and build scientifically and ethically based arguments for change
Reach	Look beyond local HCPs and fellow patients to those who may have the knowledge or experience to help Educate and empower others to help you in your cause
Engagement	Build a network for ongoing bi-directional communication with HCPs, health authorities, pharma companies and other patient advocates Work together as equal partners, understanding and respecting each other's needs and motivations

Discussion points

Do you agree with the challenges for rare disease communications outlined here? Are there others that you think we should discuss?

What is your perception of the RARE communications model? Have you implemented some of these principles in your own activities?

Putting the RARE communications model into practice

Some examples of taking small steps towards solving big problems

Questions to ask when applying the RARE communications model

What do I want to communicate and to whom?

What is the best way to do this?

What specific challenge do I have?

Whom can I ask for help?

Types of communications and audiences

Peer-reviewed journal publications	• Clinical/HEOR studies; survey data; case studies • **Audience**: HCPs, regulatory authorities/payors, pharmaceutical and medical devices industry; also patient groups and patients
Scientific/medical and other congress presentations	• Poster or oral presentations of clinical/HEOR studies; survey reports; other research case studies • **Audience**: Depending on congress focus – HCPs, health authorities, patients and patient groups, general public
Reports (print, online)	• Survey data; white papers discussing a key issue, challenge or opportunity; congress or meeting highlights; case study series or compendia of patient stories/experience • **Audience**: Everyone
Newsletters, brochures, videos	• Conference/meeting highlights, survey data, updates on activities or campaigns, literature or study updates, case studies, patient stories • **Audience**: Everyone
Social media	• News; conference updates; events; personal experience • **Audience**: Everyone

Collate and build upon existing disease understanding

1. Challenge

There is little awareness of the real-life impact of Disease X in Asia. Although there are many publications describing the natural history of the disease, most are written by clinicians based in the US or Europe

Need to generate a better understanding of how Disease X affects patients in my region

2. Barriers

Large geographic area, heterogeneous healthcare systems, cultural and language differences across the various countries

3. Approach

Demonstrate how Disease X affects individuals and families in your region by gathering data on the day-to-day challenges and experiences of other patients. Conduct online surveys and phone interviews to understand age at onset of symptoms, age at diagnosis, diagnostic journey, treatment received, impact of daily activities/work/school, etc

4. Getting help

Opportunity to foster collaboration between patients, patient organizations, healthcare professionals and pharmaceutical companies across the region to ensure the survey reaches the broadest possible audience. This will allow the survey/interviews to be conducted in local languages and will help to apply the research findings to a local context

Identify relevant clinicians

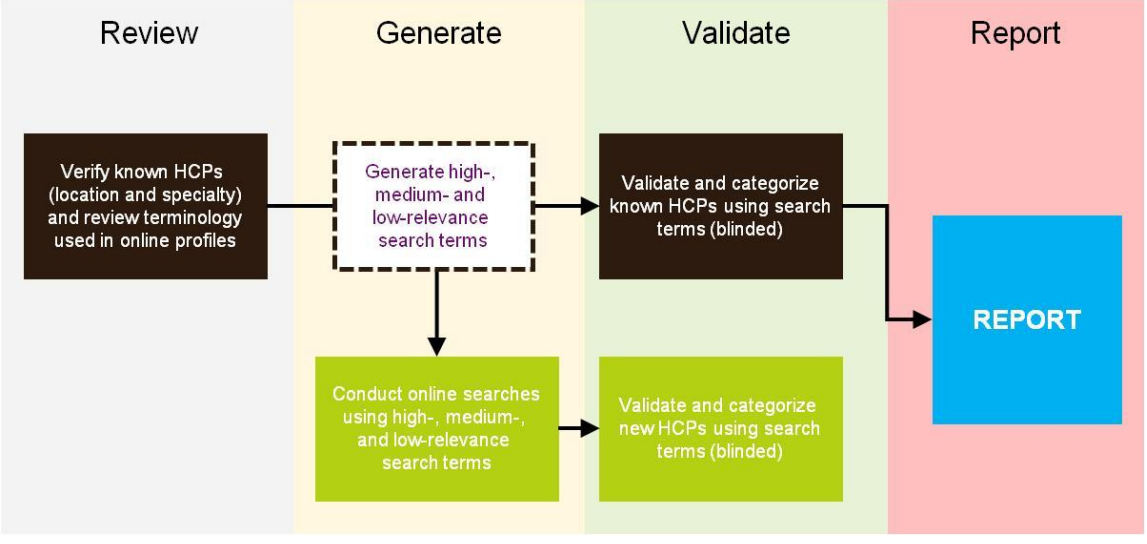
1. Challenge

It isn't clear where patients with Disease X may be being diagnosed or treated. We needed to find and engage those HCPs who are involved in the day-to-day care of patients with this condition so that we can work together on a local level

2. Barriers

Limited number of HCPs known, so difficult to ask for referrals

3. Approach



Using publicly available information about 368 HCPs worldwide that are known to treat Disease X, we developed an ideal profile of the kind of person that treats Disease X – what terms they use to describe their research, what societies they members of, what congresses they attend, etc

We used this information to conduct online searches to identify new HCPs that matched our criteria. Once identified, we made contact with the new HCPs to discuss local standards of care and how it can be improved

4. Outcome

From a starting point of 368 HCPs globally, we identified 1158 local HCPs who diagnose/treat Disease X

Understanding clinical practice

1. Challenge

Care pathways for MPS VI are fragmented and standard of care variable across Asia

Need to better understand how real-life clinical practice in the Asia–Pacific region compared with current guidelines for the diagnosis and management of a rare genetic disease

2. Approach

HCPs of interest were identified through desk research and referral from known experts

A quantitative and qualitative online survey was designed to understand current disease management practices in Asia

The results were collated, analysed and presented to a group of local expert clinicians as part of an advisory board meeting

3. Outcome

The results of the survey were developed into a review publication in consultation with participating experts

Contents lists available at SciVerse ScienceDirect

Molecular Genetics and Metabolism

journal homepage: www.elsevier.com/locate/ymgme

Current diagnosis and management of mucopolysaccharidosis VI in the Asia-Pacific region

Wuh-Liang Hwu [a], Torayuki Okuyama [b], Wai Man But [c], Sylvia Estrada [d,e,f], Xuefan Gu [g], Joannie Hui [h], Motomichi Kosuga [b], Shuan-Pei Lin [i], Lock-Hock Ngu [j], Huiping Shi [k], Akemi Tanaka [l], Meow-Keong Thong [m], Duangrurdee Wattanasirichaigoon [n], Pornswan Wasant [o], Jim McGill [p,*]

ARTICLE INFO

ABSTRACT

Introduction: Mucopolysaccharidosis (MPS) type VI (Maroteaux–Lamy syndrome) is a clinically heterogeneous lysosomal storage disorder. It presents significant diagnostic and treatment challenges due to the rarity of the disease and complexity of the phenotype. As information about MPS VI in Asia-Pacific countries is limited, a survey was conducted to assess current practices for diagnosis and management of MPS VI in this region. The participants were selected based on their experience in diagnosing and managing MPS patients.

Methods: The survey comprised 29 structured quantitative or qualitative questions. Follow-up consultations were undertaken to discuss the data further.

Results: Thirteen physicians from eight countries or regions (Australia, China, Hong Kong, Japan, Malaysia, Philippines, Taiwan and Thailand) were surveyed. At the time of the survey twenty-two patients with MPS VI were directly treated by the respondents and most (~80%) had rapidly progressing disease. A wide range of medical specialists are involved in managing patients with MPS VI, the most common being orthopedic surgeons, pediatricians and geneticists. The availability/accessibility of diagnostic tools, therapies and national insurance coverage vary greatly across the countries/regions and, in some cases, between different regions within the same country. Currently, there are national MPS management groups in Australia and Japan. Australia, Taiwan and Hong Kong have local guidelines for managing MPS and local MPS registries are available in Australia, Taiwan, and Japan.

Conclusions: This survey highlights differences in the diagnosis and management of MPS VI between Asia-Pacific countries/regions. Important barriers to advancing the identification, understanding and treatment of MPS VI include the paucity of epidemiological information, limited access to laboratory diagnostics and therapies, low disease awareness, and a lack of monitoring and treatment guidelines. There is a clear need to facilitate communications between physicians and establish regional or national disease registries, a multidisciplinary referral network, and a centralized diagnostic and management framework.

Please cite this article as: W.-L. Hwu, et al., Current diagnosis and management of mucopolysaccharidosis VI in the Asia-Pacific region, Mol. Genet. Metab. (2012), doi:10.1016/j.ymgme.2012.07.019

Discussion points

What is your greatest communication challenge in rare diseases?

What information sources and contacts do you have access to?

Who might be able to help?

What research have you and your colleagues carried out to improve awareness and understanding of your disease of interest? How did you communicate it?

What information would you like to receive from pharmaceutical companies and in what format?

What information from patients or patient organisations do pharmaceutical companies need to support development of diagnostic tools and treatments?

Summary

The challenges posed by rare diseases can only be overcome by a consistent series of small steps

There are clear opportunities to develop innovative communication strategies to address may of problems posed by rare diseases

Data are the key to progress – every opportunity should be taken to generate data to provide a better understanding of the real-world impact of rare diseases and support arguments for improved care

To be truly effective, communication must take the form of a conversation between all key stakeholders, with an emphasis on collaboration and mutual benefit

All members of the rare disease community have the opportunity and ability to develop and contribute to rare disease communications

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Thank you