World Orphan Drug Congress Asia 2014

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SINGAPORE 10–11 JUNE 2014

The World Orphan Drug Congress 2014, Asia's largest orphan drug industry event, took place in Singapore on 10–11 June 2014.

Committed to fostering partnerships and collaborations, the congress brought together various key stakeholders in Asia's developing orphan drugs community to discuss the development of, and facilitation of access to, effective treatments for rare diseases.

Over 60 participants representing patient advocacy groups, non-governmental organizations, pharmaceutical companies and specialized advisory firms with an interest in rare diseases attended the 2-day meeting. Patient advocacy groups were particularly well represented —delegates from advocacy groups based in China, South Korea, Japan, Singapore, Hong Kong, Malaysia and India made up 27% of the attendees. Large pharmaceutical companies such as Shire, BioMarin, Genzyme, Novartis, AstraZeneca, GlaxoSmithKline and Eisai, who share a common interest in developing new medicines for rare diseases, accounted for 36% of the participants. The industry specialisms present included marketing, clinical research, medical affairs, regulatory, market access, medical education, communications and the CEO of a leading rare disease pharmaceutical company.

AMICULUM, a global healthcare communications and consulting business, was a sponsor of the World Orphan Drug Congress Asia 2014. In line with the spirit and objectives of the meeting, AMICULUM took the opportunity to launch its new rare disease agency, Comradis, in Asia. Comradis is dedicated to raising awareness and improving understanding of rare diseases and uncommon cancers. Comradis may be a new agency in Asia, but AMICULUM already has significant experience working with industry clients and multiple other stakeholders involved in the development of new treatments for rare diseases and cancers.

The Comradis booth in the exhibition area of the congress served as a rare platform to offer invaluable opportunities for networking with peers, industry, investors and patient groups.

The AMICULUM team certainly enjoyed being part of the World Orphan Drug congress and has committed to return as a sponsor at the June 2015 congress.

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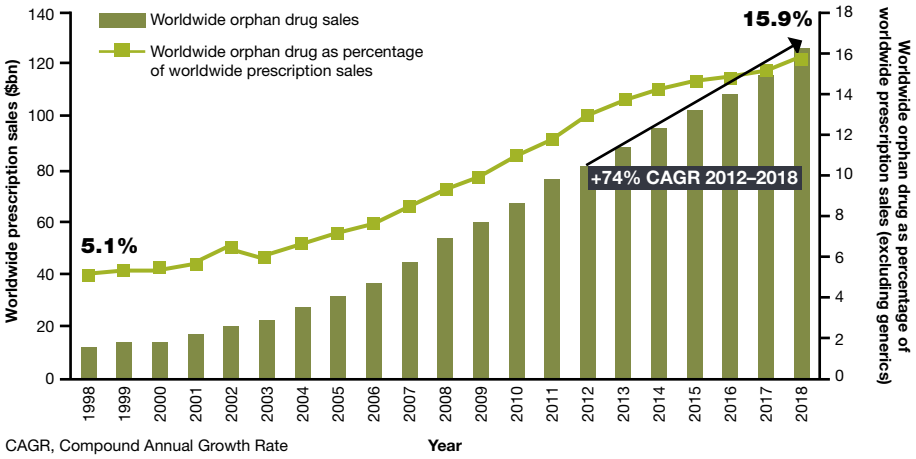
Rare diseases and orphan drugs: the current state of play in Asia

Over 7,000 rare diseases have been identified to date and an estimated 250 million people worldwide are suffering from these conditions. With Asia representing over half the world's population, rare diseases are far from being uncommon in the region and they pose a significant healthcare burden.

As treatments for rare diseases represent a high unmet medical need, they have attracted a great deal of interest from the global pharmaceutical industry, including large pharmaceutical companies and smaller speciality businesses. Cynthia Loo, Executive Director Asia Pacific at BioMarin, presented orphan drug sales projections published by Evaluate Pharma (23 April 2013) which estimated that the worldwide orphan drug market is set to reach 127 billion USD by 2018 (see graph below).



Worldwide orphan drug sales and share of prescription drug market (1998–2018)
Source: EvaluatePharma® (23 Apr 2013)

Orphan drug policies adopted in several countries provide further incentive for research in this area. Joff Masukawa, Vice President and Global Head of Government Relations and Public Affairs at Shire, USA, highlighted that over 160 orphan drugs have been made available between 1995 and 2005 in the USA alone. It is estimated that over 400 orphan drugs have been launched worldwide over the last 30 years. While this represents significant progress, there are still many rare diseases for which no effective treatment is available.

Pharmaceutical companies face numerous hurdles to bring an orphan drug to market

- High research and development (R&D) cost
- Long R&D process
- Unique and unaddressed challenges in clinical trial data
- Challenging patient recruitment
- Low public awareness
- Regulatory inconsistencies
- Lack of reimbursement programmes
- Poor patient access
- Difficulties in establishing collaborations

Following in the footsteps of the US FDA's Orphan Drug Act, some Asian nations including Japan, Taiwan, Hong Kong, Korea and Singapore have recently implemented orphan drug legislations that offer subsidies and other incentives to encourage drug development

However, the uptake of, and access to, orphan drugs varies significantly across these countries

Singapore

The government is working on a new scheme to help patients reduce the financial burden of treatments for rare diseases and uncommon cancers. Currently, the cost of treatment is so high that in some cases physicians are reluctant to prescribe specific medicines due to the financial impact on the patient's family. Many patients in Singapore rely on partial funding provided by pharmaceutical companies. Additionally, there is a need to increase awareness among all stakeholders and to provide adequate training for caregivers so that they are equipped and prepared to recognize and respond to unconventional emergency situations.

> "It's not all about funding, it's also about awareness and training caregivers"
>
> – Patricia Ng, President of the Singapore Rare Disease Society

South Korea

Despite having raised public awareness and extended government support for the treatment of rare diseases, access to orphan drugs remains a significant challenge in South Korea. Smaller pharmaceutical companies that are developing orphan drugs often do not have a large enough presence to implement significant changes and this adds to the complications of access to treatment.

Japan

Despite the presence of government support, two main challenges remain to be addressed: pricing and reimbursement, and approval and uptake of new medicines. In 2010, the government established a 2-year trial of 'premium pricing' designed to reduce the financial burden of drug development. The trial was deemed successful and was consequently extended for 2 years. The challenge for the rare disease community is to make this initiative permanent. Additionally, the launch of new medicines is often delayed in Asia, when compared with the USA or Europe, mainly as a result of delayed and lengthy local regulatory procedures. To encourage faster access to treatment, the government recently launched a 'premium' for new orphan drugs that are first launched in Japan. However, the biggest barrier remains the '14-day rule' which stipulates that in the first year of launch in Japan, new medicines cannot be prescribed to a patient for more than 14 days. This poses a major challenge, particularly for the treatment of chronic rare diseases and has the cascading effect of further reducing patients' access to new treatments.

In summary, the Asian orphan drugs market is rapidly evolving, with many differences between individual countries in the region. It is often the case that orphan drugs become available in Asia, 9–18 months after they are launched in Europe or the USA; this is primarily due to delayed regulatory and reimbursement processes. It was a common aspiration among congress delegates for the launch of orphan drugs in Asia to occur at the same time as in other parts of the world.

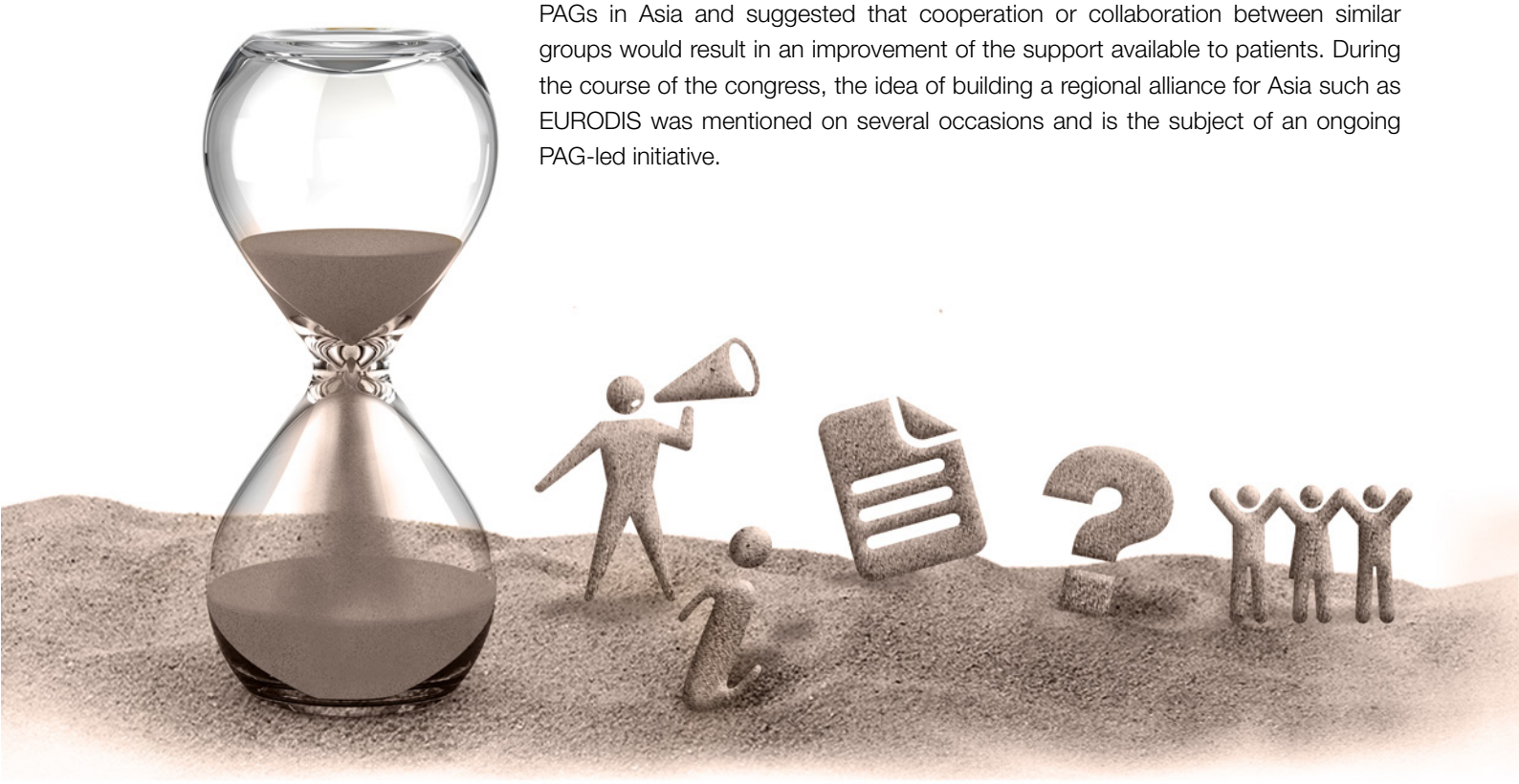
Patient Advocacy Groups in Asia: facilitating patient empowerment

As in other regions of the world, patients with rare diseases and uncommon cancers, along with their caregivers, face numerous challenges

- Access to information
- Misdiagnosis and delayed diagnosis
- Financial burden of disease
- Lack of reimbursement
- Poor access to treatment
- Uncertainty about the future
- Emotional and psychosocial impact

The lack of information and support from physicians are challenges that are common to Patient Advocacy Groups (PAGs) across Asian countries. Anthony Castaldo, President of Hereditary Angioedema (HAE) International, USA, explained that 75% of the average physicians (including family doctors) and 60% of emergency room doctors have knowledge of HAE that is either poor or very poor, resulting in patients and caregivers having to play an active advisory role in cases of emergency. This issue was also highlighted by Patricia Ng who has a daughter living with Pompe disease. Offering a potential solution, the Supporting Organization for Patients with Rare Diseases Japan (SORD) mentioned the use of a self-help system such as a pocketbook to be kept at all times by the patient, including medical records, in preparation for emergencies.

These challenges have motivated patients and caregivers to come together and set up PAGs in many Asian countries. However, most of these patient communities are scattered and they face common challenges. Several speakers alluded to the potential benefits of gaining a greater understanding of the support available from PAGs in Asia and suggested that cooperation or collaboration between similar groups would result in an improvement of the support available to patients. During the course of the congress, the idea of building a regional alliance for Asia such as EURODIS was mentioned on several occasions and is the subject of an ongoing PAG-led initiative.



The power of Patient Advocacy Groups: communicating patients' needs in a multi-stakeholder industry

"Patient Advocacy Groups created the path towards improving the quality of lives of people who suffer from rare diseases"

– Anthony Castaldo, President of Hereditary Angioedema International USA

PAGs are key influencers in Asia, being ideally positioned to communicate the needs of patients suffering from rare diseases to key stakeholders, including pharmaceutical companies and local governments. During discussions around the role of PAGs in the management of rare diseases, passion, a solid base with clear goals, and good communication skills were highlighted as key components of their success. Advocates speaking at the meeting recognized the need to consider the use of a diverse range of media platforms to convey their messages accurately and with sufficient impact to the intended targeted audience.

It was recognized that there are some key areas in which PAGs could benefit from additional support. These include:

- Media training
- How to engage government agencies
- Access to public relations agency services
- Assistance with the development of media toolkits
- Training on effective use of social media including websites, YouTube, etc

The potential role of pharmaceutical companies in providing support with these or other types of training or networking initiatives was discussed at the meeting; however, it was agreed that the independence of PAGs remained essential for their credibility and sustainability.

Early access programmes: bringing new medicines to patients faster

The widespread availability of better sources of information on rare diseases in Asia, particularly on the Internet, means that patients are now significantly better informed than previously about their conditions and any available treatments. However, as access to treatment remains a major challenge, the topic of early access (ie pre-marketing authorization) to new medicines was a recurrent topic at the congress.

What is an early access programme? As explained by Mark Corbett, Senior Vice President, Clinigen Global Access Programs, Clinigen, UK, it is a mechanism that enables patients with an unmet medical need to be provided with access to a medicine, prior to it being commercially available in that country.

Some considerations for early access include:

Uncertainty of demand

- Due to the lack of data, it is often difficult to predict how many patients are affected by the disease and therefore require the drug
- A lack of information can make it difficult to predict how many patients might benefit and for how long treatment might be required, thus, complicating assessment of the potential impact on healthcare budgets

Ethical/regulatory requirements

- Is the drug completely unlicensed or has it been approved in a major market?
- Is it licensed for other indications in the relevant country?
- Should use of the drug be granted on a cohort compassionate basis, or for named patients?
- Should it be provided at no cost to the patient?
- How does the changing regulatory environment impact future access to the drug?

Collection of data

- How many patients are affected by the disease?
- Is there sufficient information on the disease to help regulators understand the urgency of access to the drug and benefits to patients?

Multiple country requests

- As different countries have varying regulations for access to orphan drugs, how is it possible to explain making one available in one country but not another?

Mark Corbett explained that early access to treatment can lead to several benefits:

- Ethically respond to patient/physician request
- Accelerate access to innovative products where treatment options are limited
- Develop and improve clinical links with medical community
- Capture patient information and ‘real-world’ experience
- Control medicine supply, manage risk and set price precedent if necessary
- Maintain supply chain integrity, avoiding counterfeiting and international pharmacy trade
- Allow access to countries where commercialization is not possible

While provision of early access to new treatments might be possible for some patients, the legislative differences between Asian countries means that uniformity of access may be difficult to achieve.

Asia: a region with uncommon opportunities

The 2-day meeting clearly demonstrated that countries in Asia are at various stages of progress in terms of the awareness of rare diseases and provision of access to orphan drug treatments. Japan and Korea are deemed to be amongst the most advanced countries in Asia, largely owing to the support provided by their respective governments.

Early access to orphan drug treatments was a recurrent topic at the congress, and delegates expressed a strong desire and urgent need to reduce the time lag between the launch of orphan drugs in Europe and USA, and countries in Asia. It was recognized that this is primarily a challenge for the pharmaceutical industry and establishment of a clearer understanding of local regulations, awareness-raising initiatives and increased recruitment of Asian patients into clinical trials of orphan drugs were among the potential solutions recommended.

A clear theme that emerged from the meeting was the appetite of Asian PAGs for greater collaboration with other associations that share similar experiences, objectives and challenges. Together, they might be able to learn from each other and be better-placed to drive improvements in the outlook for rare disease patients across the region.

COMRADIS®
Rare disease expertise

For further information,
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