

# COMRADIS

## RARE RESOURCE 1

**Communication strategies  
for rare diseases: uncommon  
opportunities**



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COMRADIS is an AMICULUM agency dedicated to the provision  
of support for the development of, and promotion of access to,  
effective treatments for rare diseases.

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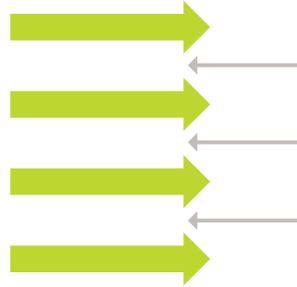
# Overview



## Traditional pharma 'broadcast' model



Pharmaceutical company



Predominantly unidirectional information flow for 'blockbuster' drug



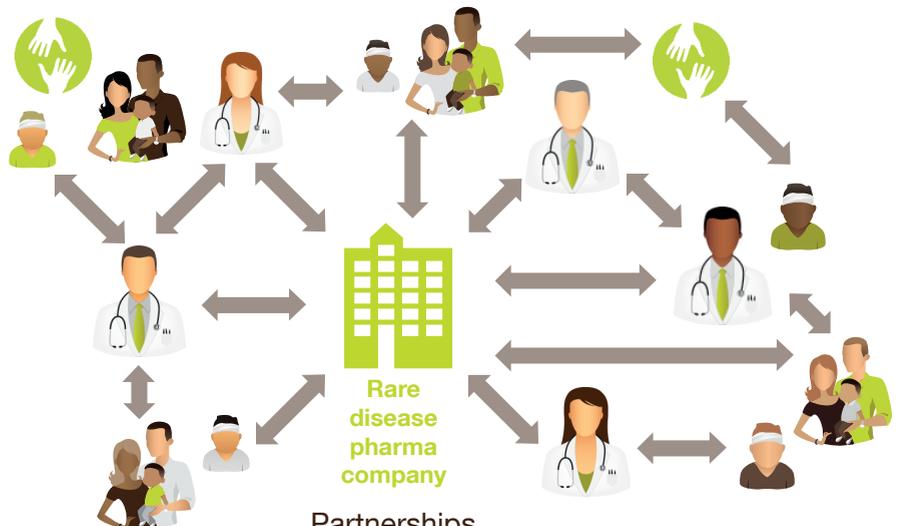
Large HCP 'audience'



## Rare diseases network model

Fewer but multiple partners:

- HCPs
- Patients
- Families
- PAGs



Partnerships  
Networked communications  
Bidirectional information flows



## The **RARE** communications model



### **R**esearch excellence

Global centre of expertise in rare disease R&D and treatment.  
Education rather than promotion. A core communications value



### **A**dvocacy

Lobbying HAs and government, PR, liaison with PAGs, media.  
Disease awareness



### **R**each

Beyond HCPs. Partnership with patients, carers and families.  
Health literacy and disease education



### **E**ngagement

Partnerships with leading HCPs, bidirectional communication.  
Networks, learning and medical education

# Introduction

Rare diseases present distinct communication requirements and opportunities. While fundamental medical communications principles are universal, the specific characteristics of rare diseases demand a new, highly tailored approach. This briefing document dissects those characteristics of rare diseases that shape effective communication with key audiences and recommends appropriate and effective methods.

Rare diseases are typically defined as those that affect no more than 5 in 10,000 patients in the EU, or fewer than 200,000 patients in the USA. Similar definitions apply in Asian countries. Collectively, however, rare diseases are far from rare. There are over 7000 of them, estimated to affect 1 in 17 people, with a prevalence of over 55 million in the EU and USA combined. So what are the key aspects of rare diseases that should influence communications strategy?

It is known that rare conditions are often characterized by:

- Delays in diagnosis
- Misdiagnosis
- Limited information
- Lack of familiarity and clinical expertise
- Fragmented care pathways
- Over-representation in children and minorities
- Few clinical management guidelines, and
- Limited treatment options

Early diagnosis and intervention is known to improve clinical outcomes; nevertheless, research conducted in the UK found that 46% of rare disease patients had to wait over a year for a final diagnosis following onset of symptoms. Of these, 20% had to wait over 5 years, and 12% over 10 years. More than 45% of patients were given an incorrect diagnosis at some stage. This situation is replicated globally, even in countries with advanced healthcare systems. Tragically, it is estimated that 27% of patients with the top 350 rare diseases will not reach their first birthday.

These challenging features of rare diseases demand highly specific and well-crafted communications strategies.

# The river in reverse: a new communications model

The traditional pharmaceutical industry blockbuster drug communication model, regardless of the channels adopted, is essentially one of a one-way stream or 'broadcast' to large 'audiences', mainly consisting of healthcare professionals (HCPs). The term 'audience' implies a willingness to listen and act, which is a major and often unfounded assumption. As George Bernard Shaw put it, "*The single biggest problem in communication is the illusion that it has taken place*". Interactive digital channels aim to improve this situation, but these are still dependent on the utility of the content being delivered and personalization of materials remains difficult. Communications on common conditions, such as arthritis, cardiovascular disease and diabetes, suffer in this regard and many HCPs experience information overload and receive divergent or contradictory messaging from multiple organizations competing for their attention.

A river of information flowing in a single direction is not true communication. The river also needs to flow in reverse. The 'RARE' communications model (see page 3) seeks to deliver naturally engaging content focused on acute unmet needs. The information is educational rather than promotional in nature, with clear clinical utility. Such communications engender two-way conversations and a networked flow. The scale factor also allows personalization, and tailored educational content can be designed to reach beyond HCPs, to patients, families and carers. HCPs who treat rare diseases have been relatively neglected in the past and as such are highly appreciative of authoritative educational materials. In parallel, useful dialogue needs to occur with health policy makers, such as health authorities, governments and regulators, as well as a generally receptive media.

## A focus on diagnosis

As already noted, the widespread absence of prompt and accurate diagnoses is a huge problem affecting health outcomes for rare diseases. Education and training aimed at transforming this situation has tremendous clinical and societal value – a real unmet need. This is a clear example of 'when preparation meets opportunity' because of the recent advances in genomics, genetic testing and other diagnostic technologies. Many rare diseases are genetically based and are amenable to molecular characterization. Educational communications and training on diagnosis and screening methods are very cost-effective for pharmaceutical companies because the incremental commercial value of each newly diagnosed patient (or each patient diagnosed several months earlier than otherwise) is considerable. This is due to the high-margin, low-volume pricing model for orphan drugs, often coupled with market exclusivity. Similarly, personalized follow-up, with education on compliance and adherence, is feasible and likewise cost effective. Strategies designed to support prompt diagnosis of rare diseases therefore present clear opportunities for collaboration between patient support organizations, healthcare providers and the pharmaceutical industry.

## Communications on trial

Clinical trials, patient registries and compassionate-use programmes potentially offer great benefits to patients with rare diseases, often at no cost to the recipient. Communication and publicity from trial sponsors is vital to secure adequate patient enrolment from the limited patient pools available. Although regulators do not require large-scale randomized studies for rare disease agents (orphan drugs) in most cases, a sufficient body of evidence needs to be gathered to indicate an adequate risk:benefit ratio,

confirmed by post-marketing follow-up data collection from patient registries, adverse-event reporting and additional trials. Patient and HCP details in registries provide a ready-made contact database to identify partners for ongoing educational communications, professional networking and dialogue. Furthermore, data from patient registries is valuable in characterizing the progression of rare diseases. Such information is often hard to gather by other means and can be highly valuable for optimization of management with existing therapies and in the search for new interventions.

## Micro-targeting and micro-messaging

The limited numbers of disease experts and patients allow for 'micro-targeting' and 'micro-messaging', which are very difficult to achieve with large-scale communications on traditional molecules for the treatment of common conditions. This is essentially a reversal of the rule of economies of scale, in which personalization to the recipients' needs and individualized delivery becomes cost-effective. Today, even Henry Ford, in such a marketplace, would ask, "*Exactly which shade of black do you require?*" The clinical journey undertaken by every patient is different and companies marketing therapies for rare diseases can tailor their educational and informational content accordingly. In some respects, communication strategies designed for rare diseases today may be regarded as models for those required to support 'personalized medicine' for more common disorders. Personalized medicine necessitates personalized communication.

## Education, not promotion

Potential market exclusivity and the absence of competing therapies and comparative studies essentially means that no comparative claims or competitive messaging is necessary for pharmaceutical companies marketing orphan drugs. Indeed, the companies have an opportunity to establish themselves as respected global centres of excellence and clinical expertise on the rare diseases in which they specialize. Communications can therefore be almost exclusively educational, rather than promotional. As such, they may be coordinated through Medical Affairs and research scientists rather than marketing channels. As has already been stated, two-way rather than 'broadcast' communications are vital in this sector.

## Partners for life

The reach of communications for rare diseases must be broad. Clearly, the roles of research bodies, patient advocacy groups (PAGs), charities, fundraisers, as well as patients, carers, families and payers are vital in this area. Consensual, mutually beneficial partnerships can readily be forged for all parties. The pharmaceutical company, as a global expert, is a rich oasis of specialized knowledge. In this case, the audience is a true, receptive audience and potential partner.

In respect of patients and families, a 'partnership for life' can be formed, either directly via educational outreach or via the HCP. The relationship can start from initial diagnosis and carry on through treatment and for the duration of the illness. This represents a longitudinal model of communication throughout the patient journey, absent in more traditional therapeutic arenas. The expected increased longevity of patients suffering from rare diseases resulting from earlier diagnosis and more effective treatment, whilst clearly of great benefit to patients and their families, is likely to be accompanied by new challenges for all involved.

## Speaking the same language

Given the diverse range of partnerships and audiences for rare disease communications, it is vital that the language and media adopted in each case is audience appropriate. Some of the content can be user-generated by interested educational partners, such as PAGs and HCPs, based on resources and assistance provided by the pharmaceutical company. Clearly, the language of scientists and healthcare professionals is distinct from that of patients, as is the context and focus.

A related aspect is the different languages spoken in different global regions, alongside local cultural and educational characteristics, which can form barriers. This is especially true for small companies and communities of medical experts who reside in a single region and culture. Rare disease communities are usually global and communications need to reflect this.

## The 'information prescription'

Apart from drug therapy, there is an acute need for patients, carers and families to receive authoritative information on rare diseases to promote 'health literacy'. This has been termed the 'information prescription' and should be tailored to individual needs. It could include:

- Medical information, including treatment options
- Care plan schedule
- Information for carers and family members
- Social support and welfare information
- Medicines access and financial information
- Disease education
- Access to information on research into the condition
- Sources of further information
- HCP contacts

## Is there such a thing as a free launch?

It is feasible to launch an orphan drug without major marketing expenditure and infrastructure. A representative-free launch can be envisaged, based instead on education and awareness campaigns. These can be accomplished digitally using disease portals and professional networking software, or coordinated through Medical Affairs personnel, such as MSLs. As such, small pharmaceutical companies (or large companies with small rare disease units) can compete on a level playing field with companies who have access to large marketing infrastructures and budgets. We are already witnessing the emergence of a new type of company that does not develop its own drugs but focuses on providing access to medicines through collaborations or licensing arrangements with R&D organizations, including universities, and contract manufacturers.

## Access all areas

In an ideal world, access to treatments for rare diseases should be available to all patients who might benefit. Overall, individual rare disease drug costs are unlikely to form a large part of healthcare budgets, although individual courses of therapy can appear comparatively expensive per patient versus other therapies, assuming high-margin, low-volume pricing structures. This situation necessitates clear and persuasive communication beyond traditional cost–benefit analysis and value per quality-adjusted life year, with more of an emphasis on total impact on the healthcare budget and benefits for public health. Such conversations may need to take place at government level and involve PAGs and HCPs as advocates.

Interestingly, the ability to monitor individual patients post-treatment offers the possibility of innovative benefit-based pricing. In such a pricing model, payers for patients who benefit most would incur the full cost, while those who suffer adverse events or disease relapse would trigger a discount. This model has the capacity to transform the cost–benefit equation.

## Good pharma?

The field of rare diseases and orphan drugs presents the global pharmaceutical industry with an opportunity to clearly demonstrate the societal benefits which are derived from their activities in developing innovative medicines. Communications activities based on a clear foundation of using science to address significant unmet medical needs will provide a beacon for the sector, which will guide future practice and help to establish the true value of pharmaceutical innovation even amongst the industry's sceptics and detractors.

## Conclusion: uncommon opportunities

Rare diseases offer opportunities for innovative communication strategies. Collectively, rare diseases are not rare. Moreover, even widespread conditions, such as various cancers, rheumatoid arthritis, diabetes, hypertension and depression, are now the subject of research that is segmenting them at the genomic level. Soon it might be a truism that everybody has a rare disease. Personalized medicine involving highly targeted drugs is clearly the future. Considered and appropriate communication strategies as outlined above will be required and may gradually become the norm in healthcare.

It would require an uncommon lack of foresight to dismiss this trend.

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