

# Medical Affairs in Orphan Drugs

<https://marketpublishers.com/r/M7DAEF6193FEN.html>

Date: May 2017

Pages: 0

Price: US\$ 695.00 (Single User License)

ID: M7DAEF6193FEN

## Abstracts

Orphan drugs: Is your medical affairs team leading the fray or lagging behind?

Traditional commercial models simply don't work for orphan drugs, not least because the physician and patient populations are so much smaller – and the value of individual relationships is consequently higher as a result. But some players are achieving success with high-performing medical affairs teams and sophisticated strategies. Getting medical affairs right has emerged as the key success factor for drugs companies gaining traction in rare diseases markets. So, what exactly are they doing? What works and why?

### Report Overview

Medical Affairs in Orphan Drugs delves into the brave new world of rare diseases to find out what the new 'super-breed' of medical affairs specialists are doing to edge ahead. Eight leading experts in the US and Europe give their views on the evolving orphan drugs market and how medical affairs is adapting.

### Report Features

A detailed assessment of the role of medical affairs at multiple touchpoints throughout the orphan drug lifecycle.

Details of current and potential medical affairs structures including what works well for orphan drugs, and what elements need further development.

Insight into the future of medical affairs with recommendations on how to respond to the distinct challenges present in orphan drug markets and the differing approaches of big versus small pharma.

## Key Benefits

Understand what changes to make: See how medical affairs should be evolving and understand where to focus resources for maximum results.

Realise the potential: Make the case for increased investment in medical affairs and greater influence with internal and external orphan drugs stakeholders.

Recognise vital skillsets: What are the key characteristics of high-performing medical affairs specialists in the orphan drugs sector? Know what to look for and who to nurture.

Plan with confidence: Gain clarity on the evolving role of medical affairs as markets shift from traditional commercial models to the uncharted waters of rare diseases.

## Why is it so important for medical affairs to adapt?

All eyes are on medical affairs as more and more drug manufacturers throw their hats into the ring and enter the orphan drugs marketplace. But the tried and tested approaches that delivered results in traditional markets are not right in this new arena. Reinvention is the key, and medical affairs teams need to prepare their strategies with care and precision. Only a handful of highly influential physicians combined with a tiny patient population means there's no room for trial and error. Get it right and the rewards are there. Get it wrong and it's impossible to hide in the smaller and rather more personal world of rare diseases

## Key Questions Answered

What difference will intense price scrutiny make to the development of orphan drugs?

Which touchpoints across the orphan drug lifecycle should medical affairs focus on?

What specific role can medical affairs play in development and

commercialisation?

How should medical affairs engage with internal and external stakeholders on rare diseases?

What are the three 'E's of medical affairs?

Should pharma-economic evidence generation sit with medical affairs or market access?

What role does medical affairs have in educating patients, payers and regulators?

## Expert Views

The eight influencers interviewed for this report are senior medical affairs and rare diseases experts based in the US and Europe. All have shared their views and perspectives on this growth area; some are identified and some have chosen to remain anonymous.

Global Medical Lead Director, European Rare Disease Company

Francois Nader, MD, MBA, former President & CEO, NPS Pharma

Hartmann Wellhoefer, MD, VP, Head Genetic Diseases Medical, Shire

Clinical Affairs Lead, Biotechnology Company

Vice President, Head of Medical Affairs, Biotechnology Company

Michael Zaiac, VP, Medical Affairs, Hematology/Oncology EMEA, Celgene

Steven Danehy on behalf of the Pfizer Rare Disease business unit

Scott Schliebner, Vice President, Scientific Affairs – Rare Diseases, PRA Health Sciences

### 3 Key Quotes

“The role of medical affairs is even more critical and more deep than in these larger indications. Part of it is that you're basically dealing with a disease that might affect a couple of hundred or 1000 patients across the globe. That means you have maybe a dozen of real experts in that field, so there is a much more individual collaboration with these world-class expert centres in a given disease that we need to engage with. It's a much closer relationship in the sense that we continue to support the research in these centres; we continue to support medical education and we continue to support disease awareness, which is very critical.”

Hartmann Wellhoefer

“I think a really important thing is the cultivation of creative ways to continuously generate data. The key challenge with an orphan condition is you have a small population. For any drug that comes out, there's lots of questions outstanding - even when it's approved - on long-term safety, real world safety, real world efficacy, efficacy in subpopulations, efficacy in combination with other drugs, efficacy looking at more of these real world endpoints that may or may not have been included in your pivotal trial.”  
Vice President, Head of Medical Affairs, Biotechnology Company

“[You need to] get people with the right level of skills. You need to have people who can learn quickly where technical expertise is concerned but who are excellent communicators, who are having a good level of soft skills and the mental flexibility to deal with the fast pacing change in our industry. To attract and retain and develop those people will be the key challenge for medical affairs but not just medical affairs in the industry.” Michael Zaiac

### Who Would Benefit from This Report?

Medical affairs teams seeking new ways to engage orphan drug markets or to put the case for additional resources and evolved structures

Senior leadership teams assessing investment requests and performance of medical affairs

Payer liaison experts specialising in orphan drugs

Marketing and market access wishing to understand shifting internal focus on

medical affairs

Clinical development engaging with medical affairs at the early stages of orphan drug design

## Content Highlights

Research objectives and methodology

Experts interviewed

Executive summary

The current orphan drug market

Summary

Orphan drug definition

Current market overview

The orphan drug market will continue to be dominated by oncology products

Increasing scrutiny of prices is the key challenge within the orphan drug market

The nature of rare diseases means that education is needed for payers to be able to make a fully informed decision

Regulatory agencies are beginning to take a harder stance on orphan drug approvals

The growth in the orphan drug market is offering a number of opportunities for companies

The increasing interest in rare diseases has raised awareness considerably

The role of medical affairs in orphan drug development and commercialisation

## Summary

The medical affairs team tends to play a pivotal role in orphan drug R&D

Medical affairs has a critical role in identifying and understanding unmet need

Establishing relevant endpoints is a critical element of the medical affairs role

Communication of clinical development is an area for medical affairs involvement

Medical affairs will become involved in the regulatory and approval process

Engagement with both internal and external stakeholders is an important element of the medical affairs role

The medical affairs team is often the main interface between physicians and the company

Other HCPs are also an important stakeholder group for medical affairs

Medical affairs is frequently becoming involved in patient engagement activities

In some cases, payer engagement can also fall to medical affairs

Education is a fundamental element of the medical affairs role

Physician education is conducted through several different means

Medical affairs has a role to play in patient education

Payers need to understand each rare disease in order to make decisions

Medical affairs will be responsible for the majority of evidence generation

Clinical evidence generation is a traditional remit of the medical affairs team

Although traditionally the remit of market access, medical affairs is becoming involved in pharmaco-economic evidence generation

Big data has not had a significant impact on the orphan drugs market yet

Current medical affairs structures

Summary

A dedicated, focused medical affairs team is seen as the ideal

Medical affairs tends to become involved at an early stage

Most rare disease medical affairs teams have a high level of expertise

The medical affairs team will work closely with other teams to ensure alignment

The increase in resources for medical affairs highlights its importance for orphan drugs

A different approach to non-orphan indications is required

The future for medical affairs in orphan drugs

Summary

Medical affairs will continue to play a critical role

Engagement, education and evidence will continue to be the fundamental pillars through which medical affairs will deliver success

Work with stakeholders to ensure a full understanding

Be proactive in data generation

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