

# The ROI of Orphan Drugs- Ensuring optimal returns (2017)

<https://marketpublishers.com/r/RF58932E982EN.html>

Date: November 2017

Pages: 0

Price: US\$ 745.00 (Single User License)

ID: RF58932E982EN

## Abstracts

Orphan drugs are at the forefront of medical science and offer a chance for the pharma industry to make a real difference to people's lives; there's also the lure of potentially lucrative commercial benefits for those that succeed. But finding the right balance between return on investment (ROI) and optimum patient access is proving difficult. And with only 415 orphan drugs in the marketplace, it's still early days.

The ROI of Orphan Drugs: Ensuring Optimal Returns centres on the commercial perspective and provides the inside view from experts already wrestling with the issues arising in the emerging world of rare disease treatments

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Research objectives and methodologies employed producing in the report

Reasons to buy this report

The orphan drugs market is lucrative and growing, however its particular challenges require 'go to market' approaches that differ substantially from the norm – especially if there is any hope of ROI being achieved. This places the pharma industry at a

crossroads on many issues. Should orphan drug prices reflect the level of unmet need, or be built from the bottom up to recoup R&D investment? What ethical stance should the industry be adopting? Are protracted legal battles about exclusivity setting the right tone? Launching an orphan drug and then repurposing it may make sense commercially but is it the right path in the longer term?

Ultimately, how the fledging orphan drugs market resolves these issues will do plenty to influence the reputation of pharma. Will that be for better or worse?

This report will enable you to:

Get up to speed on how the orphan drugs market has developed since The Orphan Drug Act came into force.

Understand in detail why ROI is so difficult to achieve for rare disease treatments.

Explore the commercial potential of orphan drugs including financial and non-financial market incentives.

Breakdown the key challenges to market success and hear expert views on how these are being overcome.

Understand where the patient perspective sits alongside the commercial push to meet ROI targets.

Discover how market frontrunners are using stakeholder engagement strategies to meet ROI targets.

Review the various business models that drive ROI and gain insight into how these may evolve in the future.

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This report puts the commercial focus on orphan drugs and looks at how return on investment can be achieved whilst still driving optimum patient access.

Insights are drawn from in-depth interviews with 8 pharmaceutical company experts with a particular interest in orphan drugs who are currently working in commercial strategy, market access or senior management. The interviews were conducted between September and November 2017. One interviewee chose to remain anonymous.

Danil Blinov, Marketing Lead, Rare Disease - International Developed Markets,  
Pfizer, UK

Brian Go, Senior Manager, Alnylam Pharmaceuticals, US

Patrik Grandits, Head of Commercial Operations and Prokurist, Oncology Europe,  
Daiichi Sankyo Oncology, Europe GmbH

Philippe Ledru, Senior Director, Global Business Lead, Novartis, US

Luigi Longinotti, Head of Portfolio Management - Orphan Drugs, Recordati, Italy

Rick Polio, BS, CMR, MBA, Senior Regional Manager, Pulmonary & Biologics of  
Pulmonary, Autoimmune Disorders, Rare Disease, and Orphan & Biologics at a  
major biotech company, US

Gaël Le Rouzo, Oncology Scientific & Market Access Manager, EUSA Pharma,  
France

Anonymous, Pharma executive within a global pharmaceutical company

Key questions explored in this report include:

Are there particular disease areas where it is easier to achieve ROI?

What impact have orphan drug regulations had on ROI?

Do investors value the FDA orphan drug designation?

Do payers value rarity?

What is the best pricing model to ensure ROI?

What are the obstacles to achieving ROI and do these vary geographically?

How do ethical issues come into play within orphan drug ROI?

What evidence is needed to demonstrate the value of orphan drugs?

What is the future of orphan drugs given the difficulty in achieving ROI?

Is a new business model needed for orphan drugs and what might it look like?

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