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Commercial Excellence in Rare Diseases and Orphan Drugs

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Abstracts

Developing therapies for orphan diseases is attracting much industry interest with the promise of profits. But what are the specific R&D, regulatory, stakeholder and market access issues you need to understand and practically address?

Commercial Excellence in Rare Diseases and Orphan Drugs is a highly-detailed report for industry management who must evaluate, plan, execute, manage and deliver profit from orphan drug programmes. Enriched with case studies, the report reveals the real-world experience and actionable insights of eight senior industry experts who possess a deep knowledge of the challenging and highly-demanding operating environment for orphan drug development and commercialisation.

Gain Answers to Key Questions

How can patients and patient advocacy groups play an enhanced role throughout a product's development cycle from research funding to market access?

Why is early engagement with payers and KOLs critical in ensuring wide market access and uptake?

To what extent is the accurate assessment of unmet clinical need and establishing potential patient population critical and what are the benchmarks?

With approximately 7,000 identified orphan diseases, how do you select research targets for development?

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Clinical trial design and recruitment are challenges for orphan drug developers: what needs to be taken into account and how have companies approached this?

Orphan drug regulation is relatively relaxed, but what more could regulators do and how can you influence them?

Small/Big pharma collaborations: when is the optimum time for engagement and what can each learn from each other?

Top Benefits

Understand how patient support is a must-have for industry and identify the areas where they are most influential

Formulate communication strategies to ensure wide clinical buy-in and support

Address effectively the anxieties of payers who must justify orphan drug spend in their budgets

Examine critical orphan drug candidate selection and overcome challenges of designing and conducting clinical trials

Understand what is considered to be commercial excellence for orphan drugs and what are the hurdles for success

Gain insight into current company structures for orphan drugs and the way in which rare disease teams should be structured in order to maximise commercial success

Understand the regulatory structures that determine orphan drug research/approval and learn where and how regulators can be influenced

Key Takeaways

Orphan drugs offer a positive opportunity for Pharma, but companies must be realistic about their potential and the challenges – old models of assessment

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used for drugs targeting large patient populations are not fit for purpose

Engagement with key stakeholders at an early stage is critical to ultimate commercial success

Patient Groups, more than any other area, are critical to success and hugely influential in realising a product's commercial potential

Orphan drugs are often very expensive: make your case to payers, and make it early to ensure buy in

Identifying appropriate research candidates for late phase investment requires a clear understanding of unmet needs and patient population – and how you will access them

Regulators may look positively on orphan drug applications but there are still challenges to overcome

The internal organisation of orphan drug teams varies and the assessment of different models is critical

Report Features – Not Available Elsewhere

Knowledgeable and detailed insights and opinions of eight industry experts working daily in developing and commercialising orphan disease drugs in the US and Europe

Extensive case studies demonstrating different approaches and experiences for ensuring commercial effectiveness

Critical insights for corporate, research, planning, marketing and communications management

Experts Interviewed

Business Unit Head, Rare diseases, Top 10 pharmaceutical company

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Senior Director, Top 10 pharmaceutical company

Marketing Director, Mid-size European pharmaceutical company

Sales Director, European pharmaceutical company

Senior Product Manager, Top 10 pharmaceutical company

Director, Rare disease pharmaceutical company

Marketing Director, Top 10 pharmaceutical company

Marketing Director, Top 10 pharmaceutical company

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