

The Future of Orphan Drugs

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Abstracts

Orphan Drugs: Future opportunities and challenges

The orphan drug sector is booming. Record product approvals, robust pipelines and regulatory incentives. But will the good times last? As the market matures expect increasing competition, clinical research pressures and mounting payer scrutiny. It's time to plan for the future.

The Future of Orphan Drug provides a 360-degree scan of the horizon for rare disease therapies. Through primary research with leading industry experts, the report identifies key strategic and tactical pressure points for companies seeking to consolidate or enter this high-growth space.

"You will see more companies getting into this space. We will see more novel treatments come out for diseases that never had a treatment. There will be more patient involvement in the process as we go forward."

Tom Croce

Head of Global Patient Advocacy, Shire

Leading Pharma Experts Answer Key Questions:

Market drivers: What's driving investor and Pharma interest in orphan drugs?

Rising competition: What impact could rising competition in key sectors such as Duchenne's Muscular Dystrophy have on prices, market access and innovation?

Education is key: How can orphan drug developers help payers and regulators determine the value of drugs for rare disease for which clinical knowledge is limited?

Pricing: Payer scrutiny is expected to increase as orphan drug price continue to rise. How can industry proactively respond to demonstrate value and support pricing?

Clinical trials: As the orphan drug market matures what are the growing challenges for the design of, and recruitment to, clinical trials and why is patient engagement so critical?

Gene therapy: With the first products coming to market, will gene therapy unlock the full potential of the orphan drug market

Key Topics Explored

The drivers of pharma and investor interest in the potential of orphan drugs are demonstrated, as the sector is expected to achieve total annual sales of US\$178 billion by 2020 - a 2014-2020 CAGR of 10.5%.

The importance of KOLs is expected to increase in research, usage and payer/regulator advocacy.

Rising competition means orphan drug marketing will have to evolve; but how and what impact could this have on smaller companies/biotechs who lack commercial infrastructure?

Big Pharma will have to become more flexible if they are to remain competitive in the orphan drugs market in the future

Delivering innovative products that make a meaningful difference to patients, physicians and payers are critical “must haves” for orphan drug commercial success in the future.

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