

The Future of Orphan Drugs

<https://marketpublishers.com/r/FF8272E3283EN.html>

Date: August 2016

Pages: 0

Price: US\$ 695.00 (Single User License)

ID: FF8272E3283EN

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.

Contributors

Executive, biopharmaceutical company

Tom Croce, Head of Global Patient Advocacy, Shire

Anne Marie Finley, President, Biotech Policy Group

Tony Hall, Co-Founder of FindaCure

Chris Garabedian, Former President and CEO, Sarepta Therapeutics

Timothy J. Miller, Ph.D., President & CEO, Abeona Therapeutics

Francois Nader, MD, Chairman, Acceleron Pharma, Former CEO, NPS Pharma

Rare disease executive

ABOUT FIRSTWORD

FirstWord is an innovative industry intelligence leader serving over 240,000 Pharma and MedTech professionals worldwide. FirstWord offers a range of products and services designed to help your company gain a competitive edge by making key business decisions with speed and confidence.

FirstWord Pharma PLUS is a personalised and comprehensive intelligence service delivering up-to-the-minute pharma news, insight, analysis and expert views of importance to your company's success.

FirstWord Reports deliver timely, need-to-know intelligence about your products, your competitors and your markets. Covering biosimilars, market access, medical affairs, sales & marketing, technology and therapy areas, FirstWord Reports provide expert views and intelligence on the challenges facing pharma today.

Contents

1.EXECUTIVE SUMMARY

2.RESEARCH OBJECTIVES AND METHODOLOGY

3.CONTRIBUTORS

4.THE CURRENT MARKET FOR ORPHAN DRUGS

4.1 Key findings

4.2 Current market overview

4.2.1 The orphan drug market is increasing in value

4.2.1.1 There are a large number of orphan drugs being approved each year

4.2.2 The orphan drugs market is split between blockbuster products and niche therapies

4.2.2.1 New products will continue to drive growth

4.3 Orphan drug definition

4.4 Companies involved in the orphan drugs market

4.5 The orphan drugs market has benefitted from several positive trends in recent years

4.5.1 The orphan drug marketplace is maturing

4.5.2 The orphan drug market attracts strong investment

4.5.3 Interest in rare diseases has led to new technologies emerging

4.6 Current challenges in the orphan drug market

4.6.1 Lack of knowledge of rare diseases – a main challenge

4.6.2 Certain rare diseases are becoming increasingly competitive

4.6.3 Companies are facing increasing scrutiny from payers

5. THE FUTURE FOR ORPHAN DRUG DEVELOPMENT

5.1 Key findings

5.2 New pathways and technologies are expected to advance orphan drug R&D

5.2.1 Gene therapy is a promising area for rare disease

5.2.2 Academic research will continue to be pivotal for orphan drug development

5.3 Clinical trials will continue to be the main challenge in R&D

5.3.1 Defining endpoints will remain challenging for orphan drug trials

5.4 The positive direction for regulatory approvals is expected to continue

5.4.1 The FDA has well-established processes for rapid approval of orphan drugs

5.4.2 The EMA is following suit in establishing a pathway for rapid review of drugs

- 5.4.3 PDUFA VI will also boost the environment for orphan drugs
- 5.4.4 Greater dialogue between manufacturers and regulators is needed
- 5.5 Regulatory agency capacity and knowledge is still a challenge
 - 5.5.1 There may be greater scrutiny of products using the orphan drug pathway

6. THE FUTURE FOR ORPHAN DRUG MARKET ACCESS

- 6.1 Key findings
- 6.2 Overall, the orphan drug market access environment is positive
 - 6.2.1 Orphan drug prices
 - 6.2.2 Orphan drugs have generally enjoyed unrestricted reimbursement and access
- 6.3 Increasing scrutiny of orphan drug prices is expected
 - 6.3.1 Innovative pricing is seen as a potential differentiator
- 6.4 Reimbursement and access is expected to become increasingly challenging
 - 6.4.1 Demonstrating value is seen as critical for access in the future

7. THE FUTURE FOR ORPHAN DRUG COMMERCIALISATION

- 7.1 Key findings
- 7.2 Traditional sales and marketing strategies are not generally used for orphan drugs
 - 7.2.1 Education and awareness will continue to drive orphan drug marketing
 - 7.2.2 Increasing competition means orphan drug marketing will have to evolve
- 7.3 Stakeholder relationships critically important for commercial success
 - 7.3.1 Patient engagement is central
 - 7.3.2 Engagement with physicians and payers will continue to play an important role

8. THE FUTURE OUTLOOK FOR THE ORPHAN DRUG MARKET

- 8.1 Key findings
- 8.2 The orphan drug market offers significant opportunity
 - 8.2.1 Specialist companies will continue to lead the orphan drug market
 - 8.2.2 Big pharma's future in the orphan drug market is uncertain
- 8.3 Three areas of focus identified
 - 8.3.1 Knowledge and experience will be critical for future orphan drug success
 - 8.3.2 Collaboration with all stakeholders should be central to corporate strategy
 - 8.3.3 Innovation will continue to be rewarded
 - 8.3.4 SWOT analysis

I would like to order

Product name: The Future of Orphan Drugs

Product link: <https://marketpublishers.com/r/FF8272E3283EN.html>

Price: US\$ 695.00 (Single User License / Electronic Delivery)

If you want to order Corporate License or Hard Copy, please, contact our Customer Service:

info@marketpublishers.com

Payment

To pay by Credit Card (Visa, MasterCard, American Express, PayPal), please, click button on product page <https://marketpublishers.com/r/FF8272E3283EN.html>

To pay by Wire Transfer, please, fill in your contact details in the form below:

First name:
Last name:
Email:
Company:
Address:
City:
Zip code:
Country:
Tel:
Fax:
Your message:

****All fields are required**

Customer signature _____

Please, note that by ordering from marketpublishers.com you are agreeing to our Terms & Conditions at <https://marketpublishers.com/docs/terms.html>

To place an order via fax simply print this form, fill in the information below and fax the completed form to +44 20 7900 3970