Rare Diseases: The Pharmaceutical Landscape and Opportunity Areas

Description:

The service oriented and dynamic nature of the healthcare industry has led to the discovery of a plethora of treatment options for a wide range of ailments. However, there are certain diseases, termed as rare, that continue to remain untreated and neglected. Rare / orphan diseases, as the name suggests, are those diseases which are relatively infrequent in occurrence. Many orphan diseases are life-threatening and progressive in nature. Absence of potential treatment regimes catering to these diseases is a big unmet need in the market.

The prevalence and adversity of these ailments has gained significant attention in the global healthcare industry. Stakeholders have realized the necessity and importance of urgently addressing the widening concerns and the need for discovering treatment options for several rare diseases. There is a tremendous scope offered by the innovation driven research being executed by various organizations in an effort to bridge gaps in the current market landscape.

The enactment of the Orphan Drugs Act in the US and EU emerged as ground breaking and provided the necessary support to guide research focused on rare diseases worldwide. The orphan designated drugs are granted important incentives which include market exclusivity and fees reductions. This regulatory assistance has provided a strong impetus to the pharmaceutical companies and led to their active participation in the market. Collaborative agreements and partnerships, amongst the pharmaceutical firms and other active organizations (such as EURORDIS, NORD and Global Genes), are also playing a key role in addressing the needs of this market.

The wide gap between the demand and supply of drugs is likely to provide a major boost to the market in the coming decade. Several drugs for various rare diseases are currently under development and, once approved, are likely to be readily accepted by the untreated patient population.

The ‘Rare Diseases: The Pharmaceutical Landscape and Opportunity Areas’ report is a comprehensive study of the market of drugs being developed by the top pharmaceutical companies for rare diseases. The focus of this study is primarily to uncover several initiatives currently underway to harness the potential presented in different therapeutic areas. One of the basic building blocks for analysing the market was to prepare an extensive pipeline of drugs for rare diseases. This effort encompassed reviewing several databases, company websites and other information available in the public domain.

We confined the focus of our study to 50 large pharmaceutical companies which invest a proportion of their R&D spend on drugs for rare diseases. These pharmaceutical companies were profiled to get an in-depth view of the work being done by them in this domain. The profiles include a general overview of the firms, financial performance over the last five to six years, and the current marketed and clinical pipeline products specific to rare diseases.

With a robust pipeline in place, we then analysed where the industry's current vision lies and highlighted a number of efforts which have been taken by pharmaceutical companies, healthcare organizations and other industry stakeholders. Specifically, our market potential analysis outlines key patient demographics and underscores potential areas anticipated to emerge as major revenue generators for the pharmaceutical companies. In addition to the above, we have also provided our own independent perspective on the key drivers behind the growth of this market, and the associated challenges.

Contents:

1. Preface
   1.1. Overview
   1.2. Research Methodology
   1.3. Chapter Outlines

2. Introduction
   2.1. Overview
   2.2. Scope of the Study
   2.3. Methodology
2.4. Key Highlights

3. Rare Disease: Pipeline Analysis
3.1. Chapter Overview
3.2. Drugs for Rare Diseases
3.2.1. Drugs for Rare Diseases: Distribution by Highest Phase of Development
3.2.2. Drugs for Rare Diseases: Distribution by Therapeutic Area
3.2.3. Drugs for Oncological Rare Diseases: Distribution by Highest Phase of Development
3.2.4. Drugs for Rare Diseases: Distribution by Nature of Drug
3.3. Regulatory Committees: Impetus to the Rare Disease Drug Market
3.3.1. US Orphan Drug Designations: Timeline
3.3.2. EU Orphan Drug Designations: Timeline

4. Rare Diseases: Market Potential
4.1. Chapter Overview
4.2. Market Potential
4.3. Rare Indications: Market Potential
4.3.1. Ovarian Cancer
4.3.1.1. Overview
4.3.1.2. Facts
4.3.1.3. Example Marketed Drugs
4.3.1.4. Pipeline Drugs
4.3.1.5. Our Perspective
4.3.2. Chronic Lymphocytic Leukemia
4.3.2.1. Overview
4.3.2.2. Facts
4.3.2.3. Example Marketed Drugs
4.3.2.4. Pipeline Drugs
4.3.2.5. Our Perspective
4.3.3. Multiple Myeloma
4.3.3.1. Overview
4.3.3.2. Facts
4.3.3.3. Example Marketed Drugs
4.3.3.4. Pipeline Drugs
4.3.3.5. Our Perspective
4.3.4. Gastric Cancer
4.3.4.1. Overview
4.3.4.2. Facts
4.3.4.3. Example Marketed Drugs
4.3.4.4. Pipeline Drugs
4.3.4.5. Our Perspective
4.3.5. Acute Myeloid Leukemia
4.3.5.1. Overview
4.3.5.2. Facts
4.3.5.3. Example Marketed Drugs
4.3.5.4. Pipeline Drugs
4.3.5.5. Our Perspective
4.3.6. Metastatic Breast Cancer
4.3.6.1. Overview
4.3.6.2. Facts
4.3.6.3. Example Marketed Drugs
4.3.6.4. Pipeline Drugs
4.3.6.5. Our Perspective

5. Rare Diseases: Company Profiles
5.1. Chapter Overview
5.2. Key Contributors: Potential Analysis
5.3. Key Contributors: R&D Spend
5.4. Key Contributors: Research Spend on Rare Diseases
5.5. Key Contributors: Distribution by Therapeutic Area
5.6. Company Profiles
5.6.1. Roche Laboratories
5.6.1.1. Company Overview
5.6.48.4. Recent Developments
5.6.49. Teijin Pharmaceuticals
5.6.49.1. Company Overview
5.6.49.2. Financial Performance
5.6.49.3. Rare Disease Drug Portfolio
5.6.49.4. Partnerships
5.6.49.5. Recent Developments
5.6.50. Lundbeck
5.6.50.1. Company Overview
5.6.50.2. Financial Performance
5.6.50.3. Rare Disease Drug Portfolio
5.6.50.4. Partnerships
5.6.50.5. Recent Developments
5.6.50.6. Company Initiatives

6. Conclusion
6.1. The Emerging Focus in a Widely Untapped Market
6.2. Several Stakeholders have Collaborated to Lead the Initiatives
6.3. Big Pharma are the Major Contributors
6.4. Industry- Academia Coalition: Expunge the MarketConstraints
6.5. Rare Disease Market: Overall Opportunity is Big

7. Appendix: List of Companies

List of Tables:
Table 3.1 Rare Disease Drugs: Clinical Pipeline
Table 4.1 Ovarian Cancer: Key Facts
Table 4.2 Ovarian Cancer: Example Marketed Rare Disease Drugs
Table 4.3 Ovarian Cancer: Pipeline Rare Disease Drugs
Table 4.4 CLL: Key Facts
Table 4.5 CLL: Example Marketed Rare Disease Drugs
Table 4.6 CLL: Pipeline Rare Disease Drugs
Table 4.7 Multiple Myeloma: Factual Peep
Table 4.8 Multiple Myeloma: Example Marketed Rare Disease Drugs
Table 4.9 Multiple Myeloma: Pipeline Rare Disease Drugs
Table 4.10 Gastric Cancer: Key Facts
Table 4.11 Gastric Cancer: Example Marketed Rare Disease Drugs
Table 4.12 Gastric Cancer: Pipeline Rare Disease Drugs
Table 4.13 Acute Myeloid Leukaemia: Key Facts
Table 4.14 AML: Example Marketed Rare Disease Drugs
Table 4.15 AML: Pipeline Rare Disease Drugs
Table 4.16 Breast Cancer: Key Facts
Table 4.17 Metastatic Breast Cancer: Marketed Rare Disease Drugs
Table 4.18 Metastatic Breast Cancer: Pipeline Rare Disease Drugs
Table 5.1 Roche: Rare Disease Marketed Drugs
Table 5.2 Roche: Rare Disease Pipeline Drugs
Table 5.3 BMS: Rare Disease Marketed Drugs
Table 5.4 BMS: Rare Disease Pipeline Drugs
Table 5.5 Celgene: Rare Disease Marketed Drugs
Table 5.6 Celgene: Rare Disease Pipeline Drugs
Table 5.7 AstraZeneca/ MedImmune: Rare Disease Marketed Drugs
Table 5.8 AstraZeneca/ MedImmune: Rare Disease Pipeline Drugs
Table 5.9 Eli Lilly: Rare Disease Marketed Drugs
Table 5.10 Eli Lilly: Rare Disease Pipeline Drugs
Table 5.11 Pfizer: Rare Disease Marketed Drugs
Table 5.12 Pfizer: Rare Disease Pipeline Drugs
Table 5.13 GSK: Rare Disease Marketed Drugs
Table 5.14 GSK: Rare Disease Pipeline Drugs
Table 5.15 Boehringer Ingelheim: Rare Disease Pipeline Drugs
Table 5.16 Amgen: Rare Disease Marketed Drugs
Table 5.17 Amgen: Rare Disease Pipeline Drugs
Table 5.18 AbbVie: Rare Disease Marketed Drugs
Table 5.19 AbbVie: Rare Disease Pipeline Drugs
Table 5.20 Novartis: Rare Disease Marketed Drugs
Table 5.21 Novartis: Rare Disease Pipeline Drugs
Table 5.22 Sanofi/Genzyme: Rare Disease Marketed Drugs
Table 5.23 Sanofi/Genzyme: Rare Disease Pipeline Drugs
Table 5.24 Takeda/Millennium: Rare Disease Pipeline Drugs
Table 5.25 Ono Pharmaceuticals: Rare Disease Pipeline Drugs
Table 5.26 Otsuka: Rare Disease Pipeline Drugs
Table 5.27 Eisai: Rare Disease Marketed Drugs
Table 5.28 Eisai: Rare Disease Pipeline Drugs
Table 5.29 Daiichi Sankyo: Rare Disease Pipeline Drugs
Table 5.30 Shire: Rare Disease Marketed Drugs
Table 5.31 Shire: Rare Disease Pipeline Drugs
Table 5.32 Johnson & Johnson: Rare Disease Marketed Drugs
Table 5.33 Johnson & Johnson: Rare Disease Pipeline Drugs
Table 5.34 Kyowa Hakko Kirin: Rare Disease Pipeline Drugs
Table 5.35 Astellas: Rare Disease Marketed Drugs
Table 5.36 Astellas: Rare Disease Pipeline Drugs
Table 5.37 Biogen Idec: Rare Disease Marketed Drugs
Table 5.38 Biogen Idec: Rare Disease Pipeline Drugs
Table 5.39 Chugai: Rare Disease Pipeline Drugs
Table 5.40 Gilead Sciences: Rare Disease Marketed Drugs
Table 5.41 Gilead Sciences: Rare Disease Pipeline Drugs
Table 5.42 Mitsubishi Tanabe: Rare Disease Pipeline Drugs
Table 5.43 Sumitomo Dainippon: Rare Disease Pipeline Drugs
Table 5.44 Chiesi: Rare Disease Pipeline Drugs
Table 5.45 Teva: Rare Disease Marketed Drugs
Table 5.46 Teva: Rare Disease Pipeline Drugs
Table 5.47 Baxter: Rare Diseases Marketed Drugs
Table 5.48 Baxter: Rare Diseases Pipeline Drugs
Table 5.49 Grifols: Rare Disease Marketed Drugs
Table 5.50 Grifols: Rare Disease Pipeline Drugs
Table 5.51 Bayer: Rare Disease Marketed Drugs
Table 5.52 Bayer: Rare Disease Pipeline Drugs
Table 5.53 Actelion: Rare Disease Marketed Drugs
Table 5.54 Actelion: Rare Disease Pipeline Drugs
Table 5.55 Ipsen: Rare Disease Pipeline Drugs
Table 5.56 Nippon Shinyaku: Rare Disease Pipeline Drugs
Table 5.57 Merck KGaA: Rare Disease Marketed Drugs
Table 5.58 Merck KGaA: Rare Disease Pipeline Drugs
Table 5.59 UCB: Rare Disease Pipeline Drugs
Table 5.60 CSL: Rare Disease Marketed Drugs
Table 5.61 CSL: Rare Disease Pipeline Drugs
Table 5.62 Merck: Rare Disease Pipeline Drugs
Table 5.63 Merck Partnerships
Table 5.64 Sigma-Tau: Rare Disease Marketed Drugs
Table 5.65 Sigma-Tau: Rare Disease Pipeline Drugs
Table 5.66 Alexion: Rare Disease Marketed Drugs
Table 5.67 Alexion: Rare Disease Pipeline Drugs
Table 5.68 Recordati: Rare Disease Marketed Drugs
Table 5.69 Recordati: Rare Diseases Pipeline Drugs
Table 5.70 Novo Nordisk: Rare Disease Marketed Drugs
Table 5.71 Novo Nordisk: Rare Disease Pipeline Drugs
Table 5.72 Shionogi: Rare Disease Pipeline Drugs
Table 5.73 Vertex: Rare Disease Pipeline Drugs
Table 5.74 Meiji Seika: Rare Disease Pipeline Drugs
Table 5.75 Ferring: Rare Disease Pipeline Drugs
Table 5.76 Teijin: Rare Diseases Pipeline Drugs
Table 5.77 Santen: Rare Disease Pipeline Drugs
Table 5.78 Teijin: Rare Disease Pipeline Drugs
Table 5.79 Lundbeck: Rare Disease Marketed Drugs

List of Figures:
Figure 2.1 Rare Disease Drugs Market: Key Takeaways
Ordering:  
Order Online - http://www.researchandmarkets.com/reports/3291861/ 
Order by Fax - using the form below 
Order by Post - print the order form below and send to 

Research and Markets, 
Guinness Centre, 
Taylors Lane, 
Dublin 8, 
Ireland.
Fax Order Form
To place an order via fax simply print this form, fill in the information below and fax the completed form to 646-607-1907 (from USA) or +353-1-481-1716 (from Rest of World). If you have any questions please visit http://www.researchandmarkets.com/contact/

Order Information
Please verify that the product information is correct and select the format(s) you require.

Product Name: Rare Diseases: The Pharmaceutical Landscape and Opportunity Areas
Web Address: http://www.researchandmarkets.com/reports/3291861/
Office Code: SCPLROPI

Product Formats
Please select the product formats and quantity you require:

<table>
<thead>
<tr>
<th>Format</th>
<th>Price</th>
</tr>
</thead>
<tbody>
<tr>
<td>Electronic (PDF) - Single User:</td>
<td>USD 2799</td>
</tr>
<tr>
<td>Electronic (PDF) - Site License:</td>
<td>USD 6499</td>
</tr>
<tr>
<td>Electronic (PDF) - Enteprisewide:</td>
<td>USD 10999</td>
</tr>
</tbody>
</table>

Contact Information
Please enter all the information below in BLOCK CAPITALS

Title:  
Mr ☐  Mrs ☐  Dr ☐  Miss ☐  Ms ☐  Prof ☐  
First Name: ______________________________________  Last Name: ______________________________________
Email Address: * ______________________________________
Job Title: ______________________________________
Organisation: ______________________________________
Address: ______________________________________
City: ______________________________________
Postal / Zip Code: ______________________________________
Country: ______________________________________
Phone Number: ______________________________________
Fax Number: ______________________________________

* Please refrain from using free email accounts when ordering (e.g. Yahoo, Hotmail, AOL)
Payment Information

Please indicate the payment method you would like to use by selecting the appropriate box.

☐ Pay by credit card: You will receive an email with a link to a secure webpage to enter your credit card details.

☐ Pay by check: Please post the check, accompanied by this form, to:
Research and Markets,
Guinness Center,
Taylors Lane,
Dublin 8,
Ireland.

☐ Pay by wire transfer:
Please transfer funds to:
Account number 833 130 83
Sort code 98-53-30
Swift code ULSBIE2D
IBAN number IE78ULSB98533083313083
Bank Address Ulster Bank,
27-35 Main Street,
Blackrock,
Co. Dublin,
Ireland.

If you have a Marketing Code please enter it below:

Marketing Code: _______________________

Please note that by ordering from Research and Markets you are agreeing to our Terms and Conditions at http://www.researchandmarkets.com/info/terms.asp

Please fax this form to:
(646) 607-1907 or (646) 964-6609 - From USA
+353-1-481-1716 or +353-1-653-1571 - From Rest of World