Cystic Fibrosis - Global Strategic Business Report

Description: This report analyzes the worldwide markets for Cystic Fibrosis in US$ Million. The report provides separate comprehensive analytics for the US, Canada, Europe, and Rest of World. Annual estimates and forecasts are provided for the period 2014 through 2022. Market data and analytics are derived from primary and secondary research.

Company profiles are primarily based on public domain information including company URLs. The report profiles 56 companies including many key and niche players such as -

- Advanced Inhalation Therapies (AIT) Ltd.
- AbbVie Inc.
- Galapagos NV
- Genentech, Inc.
- Gilead Sciences, Inc.

Contents:

I. INTRODUCTION, METHODOLOGY & PRODUCT DEFINITIONS
   Study Reliability and Reporting Limitations
   Disclaimers
   Data Interpretation & Reporting Level
   Quantitative Techniques & Analytics
   Product Definitions and Scope of Study

II. EXECUTIVE SUMMARY

1. MARKET OVERVIEW
   Cystic Fibrosis
   A Prelude
   CF Disease Prevalence: A Brief Note
   Table 1: Prevalence of Cystic Fibrosis across Select Countries
   Treatment Modalities for Cystic Fibrosis
   CFTR Modulators: Key Drug Category
   Competitive Scenario
   Table 2: Leading CF Drugs on the Market (2016): Percentage Share Breakdown by Sales (includes corresponding Graph/Chart)
   Vertex: At the Forefront of Cystic Fibrosis Therapeutics

2. RESEARCH IN CYSTIC FIBROSIS SPACE
   Areas of Research & Development
   Gene Therapy
   A Step Towards Cure
   Research Findings & Innovations
   Updated CF Diagnosis Guidelines to Aid in Diagnosis and Personalized Treatment
   Pipeline Drugs
   An Overview
   Select Pipeline Drugs for Cystic Fibrosis Treatment by Development Stage, 2016
   Description of Select Pipeline Drugs
   Ataluren
   VX-661 + Ivacaftor
   Riociguat
   QBW251
   CTP-656
   Cavosonstat (N91115)
   VX-440
   QR-010
   pGM169/GL67A
Acebilustat
Resunab
Inhaled Mannitol
VX-371
Inhaled Levoflaxacin (Quinsair)
AeroVanc (Vancomycin Inhalation Powder)

3. DISEASE OVERVIEW
Cystic Fibrosis
A Hereditary Disease
Mutation in CFTR Gene
A Major Cause of CF
?F508
The Most Common Mutation that Affects CFTR Protein

Table 3: Most Common Mutations and Worldwide Frequency (includes corresponding Graph/Chart)
Hereditary Risk of the Disease
Pathophysiology of the Disease
Symptoms of Cystic Fibrosis
Common Symptoms in Early Childhood
Common Symptoms in Grownups or Adults:
Complications Associated with Cystic Fibrosis
Sinus and Lung Illness
Pancreatic, Gastrointestinal, and Liver Disease
Endocrine Disorders
Infertility
Diagnosis of Cystic Fibrosis
Newborn Test
Sweat Test
The IRT Test
Carrier Test
Antenatal Test
Other Common Tests
Prenatal Diagnosis of Cystic Fibrosis
Tests to Diagnose CF-Related Complications
Cystic Fibrosis in Children

4. CYSTIC FIBROSIS TREATMENT: AN OVERVIEW
Existing Treatment Options for Cystic Fibrosis
A Quick Glance at Common Drugs for Cystic Fibrosis Management
Analysis of Drugs Indicated for Management of Cystic Fibrosis
Antibiotic Therapy for Lung Infections
Select Antibiotics Approved for CF Treatment
TOBI (Tobramycin Inhalation Solution)
Other Common Cystic Fibrosis Infections and Recommended Antibiotics
Cayston (Aztreonam for Inhalation Solution)
Combination Antibiotic Therapy
Mucolytics
Pulmozyme
The Effective Mucolytic Agent
Pancreatic Enzyme Replacement Therapy (PERT) for EPI
Leading PE Drugs Worldwide for Treating Pancreatic Insufficiency Associated with Cystic Fibrosis
CTFR Modulators
Bronchodilators
Additional Treatment for Patients with CF
Surgical and Other Techniques
Lung Transplantation
Recommended for Rare Cases of CF
Feeding Tube
For Extra Nutrition
Bowel Surgery
To Remove any Blockage in Bowel

5. CLINICAL STUDIES
Vertex Pharmaceuticals Announces Positive Phase 3 Study Results of ORKAMBI
ProQR Therapeutics Announces Positive Proof-of-Concept Study for QR-010
Concert Pharmaceuticals Presents Positive Food Effect Results for CTP-656
Vertex Pharmaceuticals Initiates Phase 2 Studies for VX-440 & VX-152
Vertex Pharmaceuticals Provides Update on Phase 3 Study of VX 661 with ivacaftor
Protalix BioTherapeutics Announces Start of Phase II Trial of AIR DNase™ (PRX-110)
Nivalis Therapeutics Announces Completion of Enrollment for Phase 2 Study of N91115
Alaxia SAS Announces Agreement and Grant from CFFT for ALX-009 Studies
Polyphor Completes Successful Phase I Study of Inhaled POL6014
AlgiPharma Receives Grant from CFFT for Phase 2 Trials of OligoG
Health Canada Grants Clearance to ProMetic for its PBI-4050
Cystic Fibrosis Canada Finances Laurent Pharmaceuticals’ LAU-7b Phase 2 Trial
Galapagos NV Begins Phase 2 Study of GLPG1837
Pharmaxis Announces Positive Phase II Trial Results of Bronchitol®
PCTerapeutics Completes Enrollment for second Phase 3 clinical trial of Translarna™
Anthera Pharmaceuticals Begins Phase 3 Study of Oral Liprotamase
Parion Sciences Announces Expanded Enrollment Criteria for CLEAN-CF Trial
Laurent Announces Positive Phase 1b Trial Results for LAU-7b
Celtaxsys Announces Positive Phase 1 Clinical Trial Data of CTX-4430
Corbus Pharmaceuticals Receives USFDA Clearance to Initiate Phase 2 Study of Resunab™
Savara Pharmaceuticals Unveils Positive Phase 2 Trial Results of AeroVanc
KalosBios Reports Top-line Data from Phase 2 Study of KB001-A
Galapagos NV Nominates GLPG2222 for Combination Therapy with GLPG1837
Cystic Fibrosis Trust and NovaBiotics Partner for the Phase IIa Study of Lynovex
PTC Therapeutics Initiates Phase 3 Trial of Translarna/ataluren
Kamada to Start Clinical Study of Alpha-1 Antitrypsin (AAT) for Cystic Fibrosis

6. PRODUCT LAUNCHES/APPROVALS
EC Grants Orphan Drug Designation to Corbus Pharmaceuticals’ Resunab
FDA Approves Vertex’s ORKAMBI® for Extended Age Group
Pharmaxis Announces Approval of Bronchitol® in Russia
FDA Grants Orphan Drug Status to Pulmatrix’s CF Drug PUR1900
ProQR Therapeutics Announces FDA Fast Track Designation for QR-010
Alcresta Therapeutics Launches Relizorb Cartridge
EC Grants Orphan Medicinal Product Designation to Arch Biopartners’ AB569
TGV-Inhalonix Receives Orphan Drug Designation for its Mul-1867
RespirTech Launches Development of Remote Monitoring System for CF
Vertex Pharmaceuticals Receives TGA Approval of ORKAMBI®
AlgiPharma’s OligoG Receives Orphan Drug Designation by FDA
Proteostasis Therapeutics Receives FDA Fast Track designation for PTI-428
Proteostasis Therapeutics Expands CF Drug Pipeline
Vertex Pharmaceuticals Receives Two EU Approvals for KALYDECO®
USPTO Issues Patent to OrPro Therapeutics’ Theradux™
FDA Approves Vertex’s ORKAMBI
Advanced Inhalation Therapies Receives Orphan Medicinal Product Designation for AIT-CF from EMA
FDA Approves New Age Group Indication for Vertex Pharmaceuticals’ KALYDECO
Sandoz Launches Tobramycin Inhalation Solution in the US
FDA Approves sNDA for KALYDECO™
EC Grants Orphan Designation to Macrolide CF Drug by Synovo GmbH

7. RECENT INDUSTRY ACTIVITY
Pharmaxis Enters into Research Collaboration with Woolcock Institute
Horizon Pharma Acquires Raptor Pharmaceutical
AstraZeneca to Sell Drug Commercialization Rights to Pfizer
Vertex Pharmaceuticals and Moderna Therapeutics Enter into Research Collaboration
Editas Medicine Enters into Agreement with CFFT for CRISPR/ Cas9-based CF Drugs
Galapagos and AbbVie Expand Agreement for CF Portfolio
Raptor Pharmaceutical Acquires Quinsair from Tripex Pharmaceuticals
Galapagos NV Enters into Licensing Agreement with the HUB Foundation
Zambon SpA Acquires Nigaard Pharma
ManRos and Cyclacel Enter into Licensing Agreement for Seliciclib
Vertex and Parion Sciences Collaborate to Develop Investigational CF Treatment
Ruthigen and Pulmatrix Enter into Merger Agreement
N30 Pharmaceuticals Changes Name to Nivalis Therapeutics
Anthera Pharmaceuticals Acquires Sollpura/liprotamase from Eli Lilly
OPKO Health Acquires Inspirostat™ dry powder inhaler from Inspiro Medical
CURx Pharmaceuticals Enters into Global License Agreement with Gilead Sciences

8. SELECT CLINICAL-STAGE PLAYERS
Advanced Inhalation Therapies (AIT) Ltd (Israel)
Alaxia SAS (France)
AlgiPharma AS (Norway)
Anthera Pharmaceuticals, Inc. (USA)
Arch BioPartners (Canada)
Celtaxsys, Inc. (USA)
Concert Pharmaceuticals, Inc. (USA)
Corbus Pharmaceuticals Holdings, Inc.(USA)
CURx Pharmaceuticals (USA)
Nivalis Therapeutics, Inc. (USA)
NovaBiotics Ltd (UK)
Parion Sciences, Inc. (USA)
Pharmaxis Ltd (Australia)
PTC Therapeutics, Inc. (USA)
Pulmatrix, Inc. (USA)

9. FOCUS ON SELECT GLOBAL PLAYERS
Abbvie Inc. (USA)
Galapagos NV (Belgium)
Genentech, Inc. (USA)
Gilead Sciences, Inc. (USA)
Novartis AG (Switzerland)
Vertex Pharmaceuticals (USA)

10. GLOBAL MARKET PERSPECTIVE
Table 4: World Recent Past, Current and Future Analysis for Cystic Fibrosis Therapeutics by Geographic Region
US, Canada, Europe, and Rest of World Markets Independently Analyzed with Annual Sales Figures in US$ Million for Years 2014 through 2022 (includes corresponding Graph/Chart)
Table 5: World 7-Year Perspective for Cystic Fibrosis Therapeutics by Geographic Region
Percentage Breakdown of Dollar Sales for US, Canada, Europe, and Rest of World Markets for Years 2016 and 2022 (includes corresponding Graph/Chart)
Table 6: World Recent Past, Current and Future Analysis for Cystic Fibrosis Therapeutics by Drug Type
Mucolytics, CFTR Modulators, Anti-Infectives, PERT, and Bronchodilators Markets Independently Analyzed with Annual Sales Figures in US$ Million for Years 2014 through 2022 (includes corresponding Graph/Chart)
Table 7: World 7-Year Perspective for Cystic Fibrosis Therapeutics by Drug Type
Percentage Breakdown of Dollar Sales for Mucolytics, CFTR Modulators, Anti-Infectives, PERT, and Bronchodilators Markets for Years 2016 and 2022 (includes corresponding Graph/Chart)

III. MARKET
1. THE UNITED STATES
A. Market Analysis
Snapshots of Cystic Fibrosis (CF) Prevalence in the US
CFFT: NGO Dedicated to Cystic Fibrosis
List of Drugs (Companies) in the US Funded by CFFT for Cystic Fibrosis (2016)
Drug Pricing in Wake of Orkambi’s High Price
Clinical Studies
Product Launches
Strategic Corporate Developments
Select Key Players
Clinical-Stage Players
B. Market Analytics
Table 8: US Recent Past, Current & Future Analysis for Cystic Fibrosis Therapeutics Analyzed with Annual Sales Figures in US$ Million for Years 2014 through 2022 (includes corresponding Graph/Chart)
2. CANADA
A. Market Analysis
Clinical Studies
Product Launch
Arch BioPartners: Player Review
B. Market Analytics
Table 9: Canadian Recent Past, Current & Future Analysis for Cystic Fibrosis Therapeutics Analyzed with Annual Sales Figures in US$ Million for Years 2014 through 2022 (includes corresponding Graph/Chart)

3. EUROPE
A. Market Analysis
Table 10: Number of Cystic Fibrosis Patients in Europe by Country (2014) (includes corresponding Graph/Chart)
Table 11: Proportion of CF Patients in Europe (Adults Vs Children) by Country (2014) (includes corresponding Graph/Chart)
Clinical Studies
Product Launches
Strategic Corporate Developments
Select Key Players
B. Market Analytics
Table 12: European Recent Past, Current & Future Analysis for Cystic Fibrosis Therapeutics Analyzed with Annual Sales Figures in US$ Million for Years 2014 through 2022 (includes corresponding Graph/Chart)

4. REST OF WORLD
A. Market Analysis
Australia
High Out-of-Pocket Expenditure by CF Patients
Clinical Studies
Product Launch
Strategic Corporate Development
Select Key Players
B. Market Analytics
Table 13: Rest of World Recent Past, Current & Future Analysis for Cystic Fibrosis Therapeutics Analyzed with Annual Sales Figures in US$ Million for Years 2014 through 2022 (includes corresponding Graph/Chart)

IV. COMPETITIVE LANDSCAPE
Total Companies Profiled: 56 (including Divisions/Subsidiaries 58)
- The United States (30)
- Canada (4)
- Europe (20)

- France (2)
- Germany (2)
- The United Kingdom (3)
- Italy (2)
- Rest of Europe (11)

- Asia-Pacific (Excluding Japan) (1)
- Middle East (3)

Ordering:
Order Online - http://www.researchandmarkets.com/reports/1244803/
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.

<table>
<thead>
<tr>
<th>Product Name:</th>
<th>Cystic Fibrosis - Global Strategic Business Report</th>
</tr>
</thead>
<tbody>
<tr>
<td>Web Address:</td>
<td><a href="http://www.researchandmarkets.com/reports/1244803/">http://www.researchandmarkets.com/reports/1244803/</a></td>
</tr>
<tr>
<td>Office Code:</td>
<td>SC</td>
</tr>
</tbody>
</table>

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

<table>
<thead>
<tr>
<th>Quantity</th>
<th>Price</th>
</tr>
</thead>
<tbody>
<tr>
<td>Electronic (PDF) -</td>
<td></td>
</tr>
<tr>
<td>Single User:</td>
<td>USD 4500</td>
</tr>
<tr>
<td>1 - 5 Users:</td>
<td>USD 6300</td>
</tr>
<tr>
<td>1 - 10 Users:</td>
<td>USD 8550</td>
</tr>
<tr>
<td>1 - 15 Users:</td>
<td>USD 10800</td>
</tr>
</tbody>
</table>

* The price quoted above is only valid for 30 days. Please submit your order within that time frame to avail of this price as all prices are subject to change.

Contact Information
Please enter all the information below in BLOCK CAPITALS

<table>
<thead>
<tr>
<th>Title:</th>
<th>Mr</th>
<th>Mrs</th>
<th>Dr</th>
<th>Miss</th>
<th>Ms</th>
<th>Prof</th>
</tr>
</thead>
<tbody>
<tr>
<td>First Name:</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Last Name:</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Email Address: *</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Job Title:</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Organisation:</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Address:</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>City:</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Postal / Zip Code:</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Country:</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Phone Number:</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Fax Number:</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

* 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: Bank details will be provided on the invoice which you will receive after you place your order with us.

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