



Cystic Fibrosis Pipeline Analysis Report

February 2018

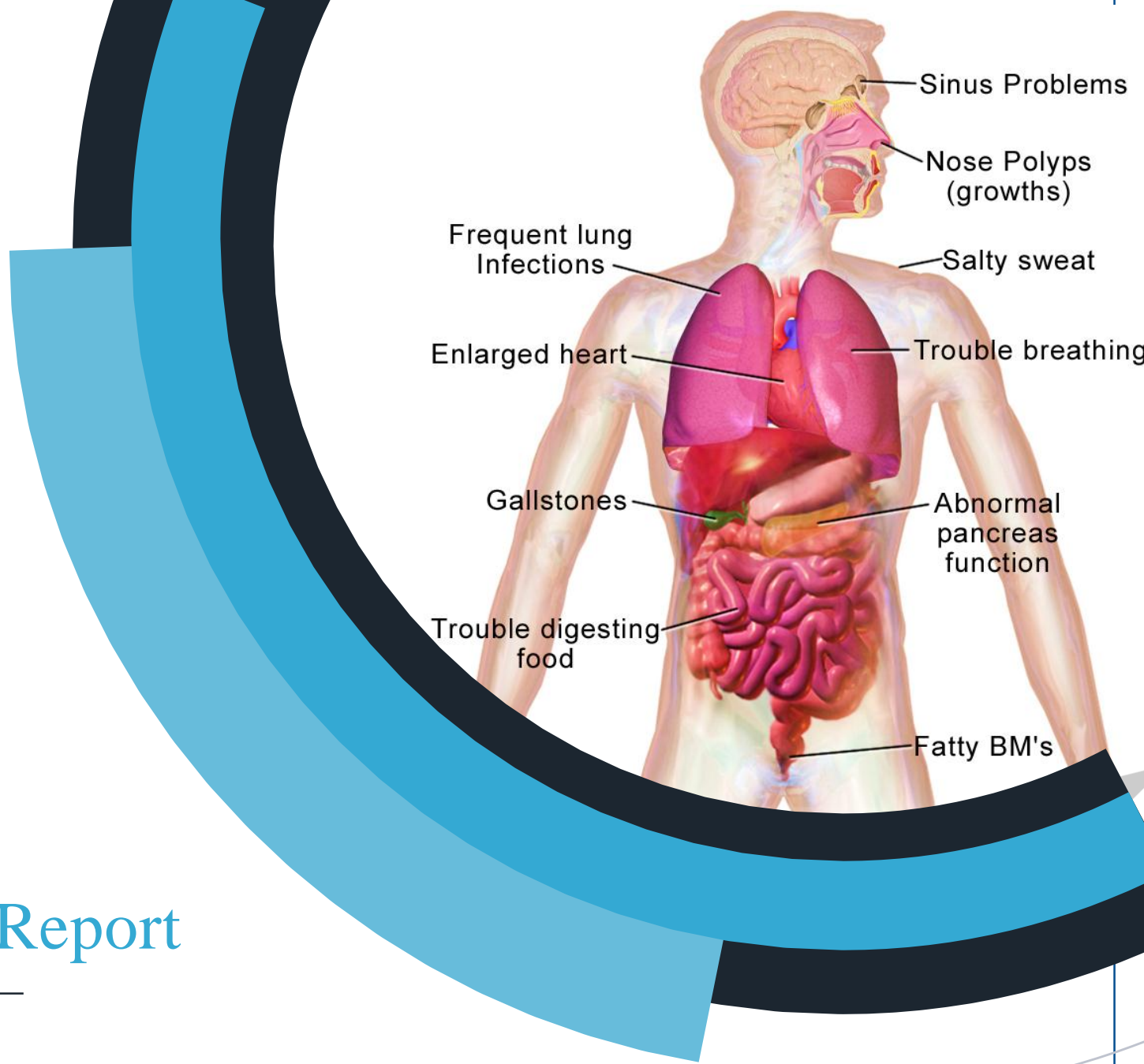


Table of Contents

Executive Summary

1. Cystic Fibrosis: Disease Overview

I. Clinical Manifestations and Complications of Cystic Fibrosis

II. Etiology and Genetics of Cystic Fibrosis

- a. Basics of Genetics and the CFTR Gene
- b. Role of CFTR Protein in Ion Transport
- c. The CFTR Gene Mutations
- d. Classification of Mutations of CFTR Gene

III. Diagnosis of Cystic Fibrosis

IV. Treatment Methodology of Cystic Fibrosis

- a. Treatment of Lung Complications
- b. Treatment for Advanced Lung Disease
- c. Treatment for Digestive Problems

2. Pipeline Analysis of Cystic Fibrosis

I. Cystic Fibrosis Drug Pipeline by Stage of Development

II. Cystic Fibrosis Drug Pipeline by Therapeutic Class

- a. Anti-Infective
- b. Anti-Inflammatory
- c. CFTR Modulator
- d. Mucociliary Clearance

e. Nutritional

f. Miscellaneous

III. Cystic Fibrosis Drug Pipeline by Molecule Type

IV. Cystic Fibrosis Drug Pipeline by Route of Administration

V. Cystic Fibrosis Drug Pipeline by Therapy Type

VI. Cystic Fibrosis Drug Pipeline by Company

List of Figures

Fig 1.1 Clinical Manifestations and Complications of Cystic Fibrosis

Fig 1.2 Mode of Inheritance of Cystic Fibrosis

Fig 2.1 Cystic Fibrosis Drug pipeline by Stage of Development

Fig 2.2 Cystic Fibrosis Drug Pipeline by Therapeutic Class

Fig 2.3 Cystic Fibrosis Drug Pipeline by Anti-Infective Class

Fig 2.4 Cystic Fibrosis Drug Pipeline by Anti-Inflammatory Class

Fig 2.5 Cystic Fibrosis Drug Pipeline by CFTR Modulator Class

Fig 2.6 Cystic Fibrosis Drug Pipeline by Mucociliary Clearance Class

Fig 2.7 Cystic Fibrosis Miscellaneous Drug Pipeline

Fig 2.8 Cystic Fibrosis Drug Pipeline by Molecule Type

Fig 2.9 Cystic Fibrosis Drug Pipeline by Route of Administration

Fig 2.10 Cystic Fibrosis Drug Pipeline by Therapy Type

Fig 2.11 Cystic Fibrosis Drug Pipeline by Company

List of Tables

Table 1.1 Classification of CFTR Mutations

Table 2.1 Cystic Fibrosis Drug Pipeline by Stage of Development

Table 2.2 Cystic Fibrosis Drug Pipeline by Anti-Infective Class

Table 2.3 Expanded Label Cystic Fibrosis Drug Pipeline by Anti-Infective Class

Table 2.4 Cystic Fibrosis Drug Pipeline by Anti-Inflammatory Class

Table 2.5 Cystic Fibrosis Drug Pipeline by CFTR Modulator Class

Table 2.6 Expanded Label Cystic Fibrosis Drug Pipeline by CFTR Modulator Class

Table 2.7 Cystic Fibrosis Drug Pipeline by Mucociliary Clearance Class

Table 2.8 Cystic Fibrosis Drug Pipeline by Mucociliary Clearance Class for Different Geographies

Table 2.9 Cystic Fibrosis Drug Pipeline by Nutritional Class

Table 2.10 Cystic Fibrosis Miscellaneous Drug Pipeline

Table 2.11 Cystic Fibrosis Drug Pipeline by Company

Executive Summary

Cystic fibrosis is a rare genetic disorder caused by the mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene located on chromosome 7. It largely affects the lungs and digestive system, and causes the secretions to become thick and sticky. The individuals with cystic fibrosis develop lung disease, resulting from a cycle of mucus retention, infection and inflammation, and pancreatic dysfunction results in calorie malabsorption. The sweat glands and reproductive organs are also get affected. The sequence of the CFTR gene was identified in 1989 and it encodes a 1480 amino acid protein. There are approximately 250 known disease-causing mutations that intervene various stages of CFTR synthesis and function. Every year approximately 70,000 children and young adults are affected with CF across the world.

The current treatment of CF comprises of symptomatic therapy mainly. The objective of the treatment includes as follows:

- prevent and control lung infections
- loosen and eliminate thick, sticky mucus from the lungs
- prevent or treat blockages in the intestines
- maintain good nutrition
- avert dehydration
- early identification and treatment of the comorbidities associated with the disease

The cystic fibrosis pipeline is comprised of diverse sets of molecules with a majority of products in early-stage development. In total 124 molecules are in development either alone or in combination with other molecules. Majority of those are in the pre-clinical development stage followed by Phase II and Phase I. Earlier the CF treatment was largely focused on the downstream effects of CFTR dysfunction (mucus retention, infection, and inflammation of the airways) and there have been many advances on those problems. However, new therapies such as CFTR modulators are able to address the underlying abnormality rather than its downstream effects. The efficacy of these treatments has been established recently. At present there are just three FDA approved molecules are commercially available in the market that targets the specific mutation types.

Majority of the companies are focusing on the modulators of CFTR gene followed by anti-infectives. The cystic fibrosis drug market is very competitive; many small players are working in the same. Vertex Pharmaceuticals, Galapagos, Flatley Discovery Lab, Ockham Biotech, Proteostasis Therapeutics and Druggability Technologies Holdings are leading the space.

About EffeMarket

EffeMarket provides market and business research solutions to clients in over 20 industries through syndicated studies, custom research, consulting engagements and newsletters. Our experienced analyst and widespread network of experts ensures accuracy within studies and provides our patrons with extensively researched, fact-based insights that helps you to take better decisions.

Disclaimer

EffeMarket reports and their contents, including all the analysis and research containing valuable market information, are provided to a select group of customers in response to orders. Our customers acknowledge when ordering that EffeMarket reports including all research and insights in their entirety are for our customers' internal use and not for general publication or disclosure to third parties. The market information is based primarily on secondary research and interviews and therefore, is subject to changes. EffeMarket takes no responsibility for any incorrect information supplied to us through such sources.

No part of this report may be given, lent, resold or disclosed to non-customers without written permission. Reproduction and/ or transmission in any form and by any means including photocopying, mechanical, electronic, recording or otherwise, without the permission of the publisher are prohibited.

Contact Us

For any questions and updates please contact:

Sales@effemarket.com

Support@effemarket.com

Website: www.effemarket.com

Phone:

US: +1-972-256-8133

UK: +44-203-286-8233

IN: +91 120 4561797

Address: Effectual Knowledge Services Pvt.

Ltd. B-55, Sector 2, Noida – 201301, U.P.,

India

US Office – New York

Effectual Knowledge Services, Inc. 425

Broadhollow Road, Suite 427, Melville | NY

11747