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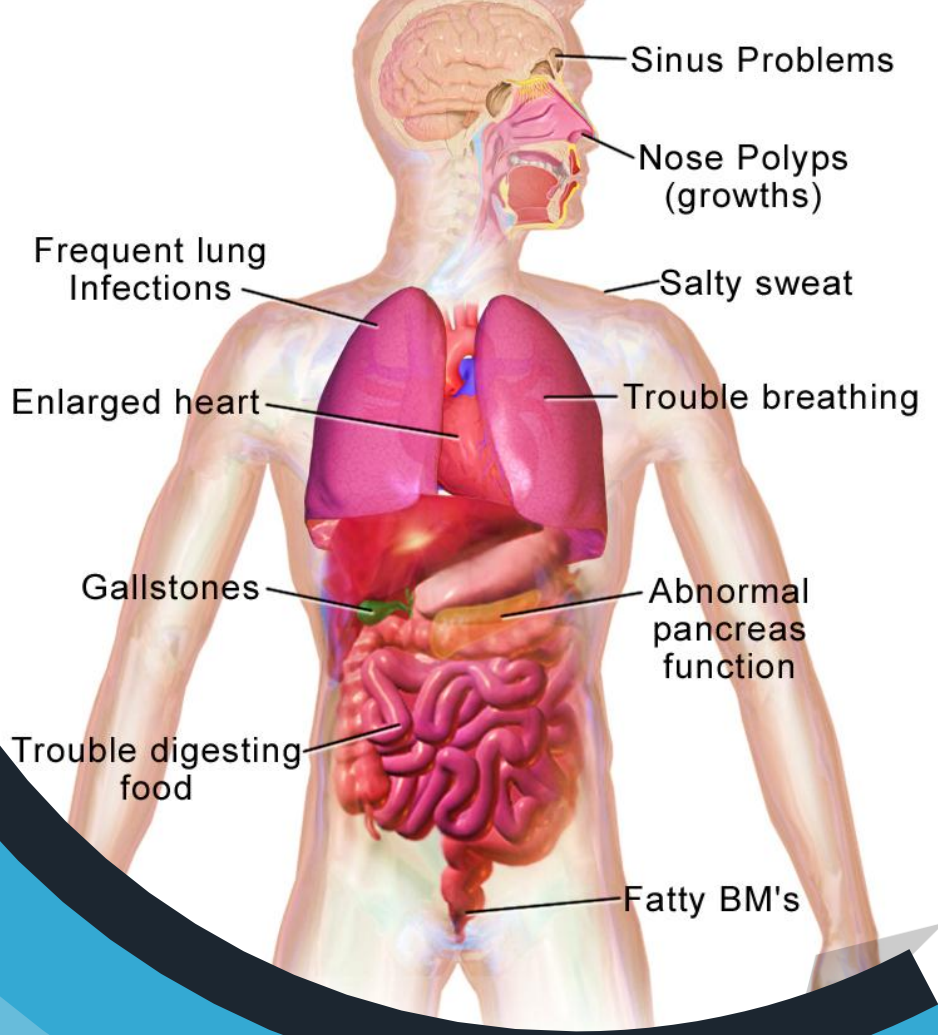

Global Cystic Fibrosis Market Report: 2028

April 2018

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Executive Summary

Cystic Fibrosis (CF) is a rare genetic disorder caused by a mutation in the Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) gene located on chromosome 7. This disorder primarily affects the lungs and digestive system and causes secretions to become thick and sticky. Individuals with Cystic Fibrosis develop lung disease resulting from a cycle of mucus retention, infection, and inflammation, as well as pancreatic dysfunction resulting in calorie malabsorption. The sweat glands and reproductive organs are also 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 with various stages of CFTR synthesis and function. Every year, approximately 70,000 to 100,000 children and young adults are affected with CF across the world.

The Cystic Fibrosis market is primarily driven by increasing CF prevalence, the rise in R&D expenditure by pharmaceutical companies and increase in funding by government bodies. The rise in the prevalence of Cystic Fibrosis is further attributed to reduced mortality rate due to treatment advancements and better disease management. Drugs such as Orkambi, Kalydeco, Symdeko etc. are expected to boost the market growth, while factors such as the high cost of treatment, and limited availability of medicine for all mutation type's patients are expected to hamper market growth.

Key players in the Cystic Fibrosis market are Vertex Pharmaceuticals, Roche, Allergan, Novartis, AbbVie, Pharmaxis, Chiesi Farmaceutici, PARI Medical Holding and Gilead Sciences. Vertex Pharmaceuticals is the leading player in the market. The global Cystic Fibrosis market is expected to witness substantial growth during the forecast period. The market is anticipated to reach a value of $12.2 billion by 2028 from the estimated value of $3.8 billion in 2018, at a CAGR of 12.3 percent. This can be attributed to the great investment by market players and many non-profit organizations in the research and development of Cystic Fibrosis therapy. Another factor is the growing penetration of drugs including CFTR modulators during the forecast period and the existence of a strong pipeline.

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. The majority of these molecules are in the pre-clinical development stage followed by Phase II and Phase I. Earlier, 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 advancements in the treatment of those problems. However, new therapies such as CFTR modulators are able to address the underlying abnormalities of Cystic Fibrosis rather than its downstream effects. The effectiveness of these treatments has been established recently. Currently, there are just three FDA-approved molecules targeting specific mutation types that are commercially available in the market.

The majority of companies are focused on the modulators of the CFTR gene, followed by anti-infectives. The Cystic Fibrosis drug market is very competitive and many small players are researching and developing products in this field. Vertex Pharmaceuticals, Galapagos, Flatley Discovery Lab, Ockham Biotech, Proteostasis Therapeutics and Druggability Technologies Holdings are leading the research space.

Key questions answered and insights delivered in the report:

1. Global Cystic Fibrosis market size by value from 2016 to 2028
2. Global Cystic Fibrosis market size by product sales; 2016 and 2017
3. Global Cystic Fibrosis market by therapeutic approach; 2017
4. Comprehensive product landscape by therapeutic class, company, originator, approval date, pharma class, molecule type, mutation type and therapy type
5. Pipeline analysis by stage of development, therapeutic class, molecule type, therapy type, route of administration and company
6. Merger and acquisition, and collaboration by the key players in the market
7. Growth drivers, future opportunities and challenges in the market
8. Earliest priority country filing trend in Cystic Fibrosis market; 2007-2018
9. Cystic Fibrosis patent trend by focus area, route of administration and composition; 2007-2018
10. Cystic Fibrosis patent trend by key patent holder; 2007-2018
11. Key patents in Cystic Fibrosis market; 2007-2018
12. Top inventors in Cystic Fibrosis market; 2007-2018
13. Detailed company profiling of key market players

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