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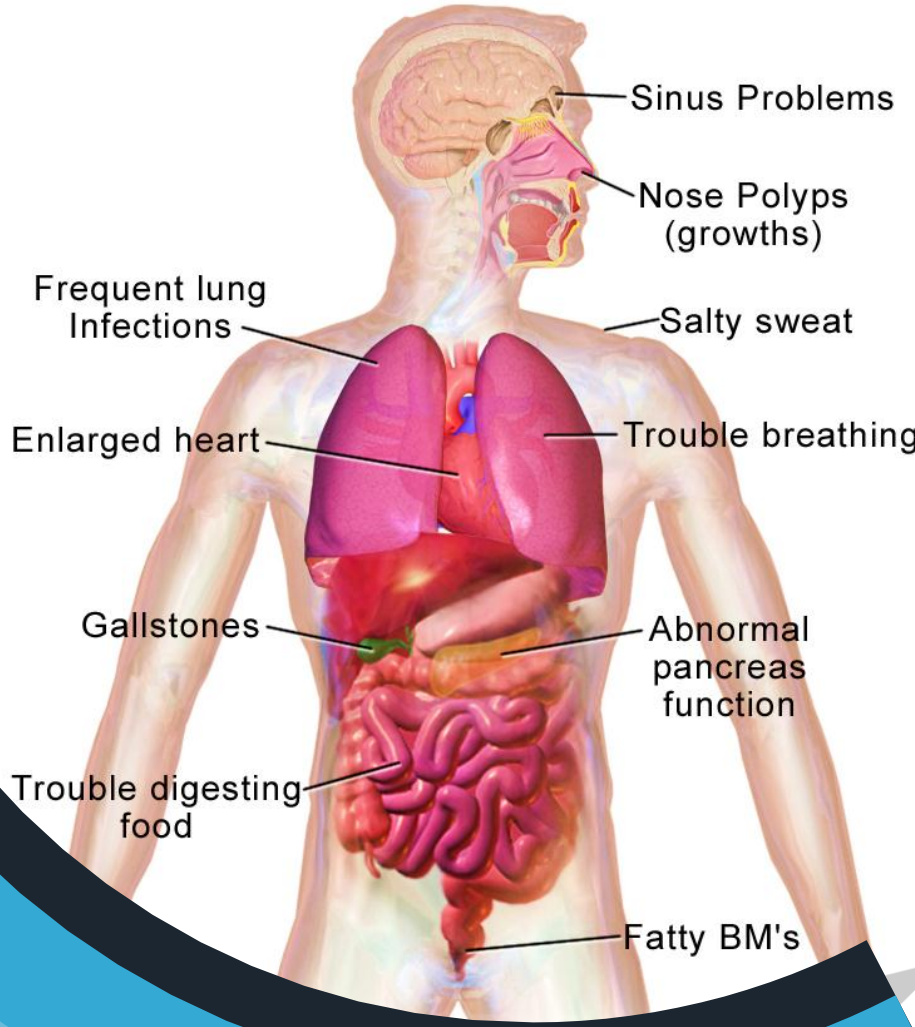

Cystic Fibrosis Pipeline Analysis Report

February 2018

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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.

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