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Cystic Fibrosis - Pipeline Insight, 2022

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Abstracts

This report can be delivered to the clients within 72 hours

DelveInsight's, "Cystic Fibrosis - Pipeline Insight, 2022," report provides comprehensive insights about 75+ companies and 80+ pipeline drugs in Cystic Fibrosis pipeline landscape. It covers the pipeline drug profiles, including clinical and nonclinical stage products. It also covers the therapeutics assessment by product type, stage, route of administration, and molecule type. It further highlights the inactive pipeline products in this space.

Geography Covered

Global coverage

Cystic Fibrosis Understanding

Cystic Fibrosis: Overview

Cystic fibrosis is a progressive, genetic disease that causes long-lasting lung infections and limits the ability to breathe over time. More than 30,000 children and adults in the United States have CF (70,000 worldwide) and CF affects people of every racial and ethnic group. In people with CF, mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene cause the CFTR protein to become dysfunctional. When the protein is not working correctly, it's unable to help move chloride -- a component of salt -- to the cell surface. Without the chloride to attract water to the cell surface, the mucus in various organs becomes thick and sticky. In the lungs, the mucus clogs the airways and traps germs, like bacteria, leading to infections, inflammation, respiratory failure, and other complications. For this reason, avoiding germs is a top

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concern for people with CF. In men, CF can affect their ability to have children. People with CF can have a variety of symptoms, including: Very salty-tasting skin, persistent coughing, at times with phlegm, frequent lung infections including pneumonia or bronchitis, wheezing or shortness of breath, poor growth or weight gain in spite of a good appetite, frequent greasy, bulky stools or difficulty with bowel movements, nasal polyps , chronic sinus infections and many more. Cystic fibrosis is a complex disease. The types of symptoms and how severe they are can differ widely from person to person.

'Cystic Fibrosis - Pipeline Insight, 2021' report by DelveInsight outlays comprehensive insights of present scenario and growth prospects across the indication. A detailed picture of the Cystic Fibrosis pipeline landscape is provided which includes the disease overview and Cystic Fibrosis treatment guidelines. The assessment part of the report embraces, in depth Cystic Fibrosis commercial assessment and clinical assessment of the pipeline products under development. In the report, detailed description of the drug is given which includes mechanism of action of the drug, clinical studies, NDA approvals (if any), and product development activities comprising the technology, Cystic Fibrosis collaborations, licensing, mergers and acquisition, funding, designations and other product related details.

Report Highlights

The companies and academics are working to assess challenges and seek opportunities that could influence Cystic Fibrosis R&D. The therapies under development are focused on novel approaches to treat/improve Cystic Fibrosis.

Cystic Fibrosis Emerging Drugs Chapters

This segment of the Cystic Fibrosis report encloses its detailed analysis of various drugs in different stages of clinical development, including phase II, I, preclinical and Discovery. It also helps to understand clinical trial details, expressive pharmacological action, agreements and collaborations, and the latest news and press releases.

Cystic Fibrosis Emerging Drugs

OligoG: Algi Pharma

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OligoG is formulated as a dry powder for inhalation and is AlgiPharma's lead drug candidate. It is an alginate oligosaccharide derived from seaweed, and is a new class of drug which is modulating mucus and normalizing mucus rheology. It is being developed to help people with cystic fibrosis clear mucus from their lungs. It is anticipated that OligoG may help to slow the progression of the disease. OligoG has been shown to disrupt the infectious biofilm often present in the lungs of individuals with CF. This biofilm disruption is believed to improve antibiotic effectiveness by increasing exposure of bacteria to antibiotics. It is in Phase 2 stage of development for the treatment of Cystic Fibrosis.

Ensifentrine: Verona Pharma

Ensifentrine combines bronchodilator and anti-inflammatory properties in one compound and has the potential to be an effective treatment for COPD and other respiratory diseases, including asthma and cystic fibrosis. It is designed to maximize its effectiveness and reduce adverse events through: high selectivity for PDE3 and PDE4 over other enzymes and receptors to minimize off-target effects; direct delivery to the lungs by inhalation to maximize pulmonary exposure to ensifentrine while minimizing systemic distribution and potential adverse events. Ensifentrine also activates the Cystic Fibrosis Transmembrane Conductance Regulator (CFTR), which is beneficial in reducing mucous viscosity and improving mucociliary clearance. This potentially makes it an attractive therapy for the treatment of cystic fibrosis. It is in Phase 2 stage of development for the treatment of Cystic Fibrosis.

MRT5005: Translate Bio

MRT5005 is the first clinical-stage mRNA product candidate designed to address the underlying cause of CF by delivering mRNA encoding fully functional cystic fibrosis transmembrane conductance regulator (CFTR) protein to the lung epithelial cells through nebulization. MRT5005 is being developed to treat all patients with CF, regardless of the underlying genetic mutation, including those with limited or no CFTR protein. The U.S. Food and Drug Administration (FDA) has granted MRT5005 Orphan Drug, Fast Track and Rare Pediatric Disease designation. It is in Phase1/2 stage of development for the treatment of Cystic Fibrosis

CB280: Calithera Biosciences

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CB-280 is a potent and selective oral inhibitor of arginase. Arginase plays an important role in the pathophysiology of CF airway disease. Sputum from patients with CF has elevated arginase activity leading to diminished arginine levels. Reduced arginine is thought to exacerbate pulmonary disease in CF by impairing the production of nitric oxide, leading to a diminished anti-microbial immune response and impaired airway function. It is known that the airways of patients with CF have lower than normal nitric oxide production, and lower nitric oxide levels directly correlate with worsened lung function and increased colonization with pathogens, including Pseudomonas aeruginosa. It is in Phase1 stage of development for the treatment of Cystic Fibrosis.

KB407 : Krystal Biotech

KB407 is a redosable gene therapy designed to correct the underlying cause of CF by delivering two copies of the CFTR gene directly to the airway epithelial cells when delivered via a nebulizer. By inducing expression of full length, normal CFTR protein in the lung, treatment with KB407 has potential to restore ion and water flow into and out of lung cells to correct the lung manifestations of the disease in patients regardless of their underlying genetic mutation. It is in Pre-Clinical stage of development for the treatment of Cystic Fibrosis.

SPL84231: SpliSense

SPL84-23-1, is designed to be mainly delivered via inhalation, effectively penetrates the target cells in the lungs, and binds to the target region, thereby preventing the inclusion of 84 intronic nucleotides as a cryptic exon, and generating a fully functioning CFTR protein. It is in Pre-Clinical stage of development for the treatment of Cystic Fibrosis.

Further product details are provided in the report……..

Cystic Fibrosis: Therapeutic Assessment

This segment of the report provides insights about the different Cystic Fibrosis drugs segregated based on following parameters that define the scope of the report, such as:

Major Players in Cystic Fibrosis

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There are approx. 75+ key companies which are developing the therapies for Cystic Fibrosis. The companies which have their Cystic Fibrosis drug candidates in the most advanced stage, i.e. phase II include, Algi Pharma.

Phases

DelveInsight's report covers around 80+ products under different phases of clinical development like

Late stage products (Phase III)

Mid-stage products (Phase II)

Early-stage product (Phase I) along with the details of

Pre-clinical and Discovery stage candidates

Discontinued & Inactive candidates

Route of Administration

Cystic Fibrosis pipeline report provides the therapeutic assessment of the pipeline drugs by the Route of Administration. Products have been categorized under various ROAs such as

Intra-articular

Intraocular

Intrathecal

Intravenous

Ophthalmic

Oral

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Parenteral

Subcutaneous

Topical

Transdermal

Molecule Type

Products have been categorized under various Molecule types such as

Oligonucleotide

Peptide

Small molecule

Product Type

Drugs have been categorized under various product types like Mono, Combination and Mono/Combination.

Cystic Fibrosis: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase II, I, preclinical and discovery stage. It also analyses Cystic Fibrosis therapeutic drugs key players involved in developing key drugs.

Pipeline Development Activities

The report covers the detailed information of collaborations, acquisition and merger, licensing along with a thorough therapeutic assessment of emerging Cystic Fibrosis drugs.

Cystic Fibrosis Report Insights

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- Cystic Fibrosis Pipeline Analysis
- Therapeutic Assessment
- Unmet Needs
- Impact of Drugs

Cystic Fibrosis Report Assessment

- Pipeline Product Profiles
- Therapeutic Assessment
- Pipeline Assessment
- Inactive drugs assessment
- Unmet Needs

Key Questions

Current Treatment Scenario and Emerging Therapies:

- How many companies are developing Cystic Fibrosis drugs?
- How many Cystic Fibrosis drugs are developed by each company?
- How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Cystic Fibrosis?
- What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Cystic Fibrosis therapeutics?
- What are the recent trends, drug types and novel technologies developed to overcome the limitation of existing therapies?

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What are the clinical studies going on for Cystic Fibrosis and their status?

What are the key designations that have been granted to the emerging drugs?

Key Players

Krystal Biotech

Vertex Pharmaceuticals

Translate Bio

Novartis

Algi Pharma

Verona Pharma

Atlantic Healthcare

Calithera Biosciences

Horizon Therapeutics

Santhera Pharmaceuticals

Reveragen Biopharma

Spli Sense

GlaxosmithKline

EmphyCorp

Abbvie

Galapagos NV

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Vertex Pharmaceuticals

PathBio Analytics

AstraZeneca

AxentisPharma AG

Key Products

KB407

MRT5005

P1037

Cavosonstat

QBW276

VX121

OligoG

Ensifentrine

Renzapride

CB280

SPL84231

Bacteriophage therapeutics

Renzapride

Sodium pyruvate

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GLPG 3067

GLPG2737

P1037

Cavosonstat

Brensocatib

Ravicti

Tobramycin

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Contents

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List Of Tables

LIST OF TABLES

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List Of Figures

LIST OF FIGURES

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