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Primary Ciliary Dyskinesia - Pipeline Insight, 2021

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Abstracts

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DelveInsight's, "Primary Ciliary Dyskinesia – Pipeline Insight, 2021," report provides comprehensive insights about 4+ companies and 4+ pipeline drugs in Primary Ciliary Dyskinesia 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

Primary Ciliary Dyskinesia Understanding

Primary Ciliary Dyskinesia: Overview

Primary ciliary dyskinesia (PCD) is usually an autosomal recessive genetic condition in which the microscopic organelles (cilia) in the respiratory system have defective function. Ciliary dysfunction prevents the clearance of mucous from the lungs, paranasal sinuses and middle ears. Bacteria and other irritants in the mucous lead to frequent respiratory infections. Symptoms often begin shortly after birth and can include coughing, gagging, choking and lung atelectasis (neonatal respiratory distress). Affected individuals often experience chronic sinus, middle ear and lung infections as well as chronic coughing, excess mucus and hearing loss. Primary ciliary dyskinesia is diagnosed definitively through examination of lung or sinus tissue obtained from a biopsy or through genetic testing. Specific structural defects that are present in these

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tissues can be detected under an electron microscope. Airway clearance therapy is used to keep the lung tissue healthy for as long as possible. This therapy may include routine washing and suctioning of the sinus cavities and ear canals. Antibiotics, bronchodilators, steroids and mucus thinners (mucolytics) are also used to treat PCD.

'Primary Ciliary Dyskinesia - Pipeline Insight, 2021' report by DelveInsight outlays comprehensive insights of present scenario and growth prospects across the indication. A detailed picture of the Primary Ciliary Dyskinesia pipeline landscape is provided which includes the disease overview and Primary Ciliary Dyskinesia treatment guidelines. The assessment part of the report embraces, in depth Primary Ciliary Dyskinesia 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, Primary Ciliary Dyskinesia 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 Primary Ciliary Dyskinesia R&D. The therapies under development are focused on novel approaches to treat/improve Primary Ciliary Dyskinesia.

Primary Ciliary Dyskinesia Emerging Drugs Chapters

This segment of the Primary Ciliary Dyskinesia 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.

Primary Ciliary Dyskinesia Emerging Drugs

> P 1037: Parion Sciences

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Epithelial sodium channel (ENaC) inhibitors are designed to block the sodium channels on the airway surfaces. In pulmonary diseases, such as chronic obstructive pulmonary disease, cystic fibrosis and primary ciliary dyskinesia, where there is a build-up of excessively concentrated mucus, preclinical models have demonstrated that blocking ENaC hydrates the mucus on the lung surface. Hydration of airway mucus restores airway clearance and improves lung function. P-1037 is a novel, long acting ENaC Inhibitor that was well tolerated at the doses tested in multiple clinical trials in healthy volunteers and patients with either cystic fibrosis or primary ciliary dyskinesia. The company has reached an agreement with Vertex Pharmaceuticals, Inc. to reacquire the pulmonary rights to epithelial sodium channel (ENaC) inhibitors developed under a collaboration announced between the parties in 2015. Under this agreement, Vertex is eligible to receive future undisclosed royalties based upon commercial success. FDA has also Granted Orphan Drug Designation, Fast Track Designation and Rare Pediatric Disease Designation to Parion Sciences P-1037 Inhalation Solution for the Treatment of Primary Ciliary Dyskinesia.

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

Primary Ciliary Dyskinesia: Therapeutic Assessment

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

Major Players in Primary Ciliary Dyskinesia

There are approx. 4+ key companies which are developing the therapies for Primary Ciliary Dyskinesia. The companies which have their Primary Ciliary Dyskinesia drug candidates in the most advanced stage, i.e. Phase II include, Parion Sciences

Phases

DelveInsight’s report covers around 4+ products under different phases of clinical development like

Late stage products (Phase III)

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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

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

Oral

Parenteral

Intravenous

Subcutaneous

Topical.

Molecule Type

Products have been categorized under various Molecule types such as

Monoclonal Antibody

Peptides

Polymer

Small molecule

Gene therapy

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Product Type

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

Primary Ciliary Dyskinesia: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase II, I, preclinical and discovery stage. It also analyses Primary Ciliary Dyskinesia 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 Primary Ciliary Dyskinesia drugs.

Primary Ciliary Dyskinesia Report Insights

Primary Ciliary Dyskinesia Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Primary Ciliary Dyskinesia Report Assessment

Pipeline Product Profiles

Therapeutic Assessment

Pipeline Assessment

Inactive drugs assessment

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Unmet Needs

Key Questions

Current Treatment Scenario and Emerging Therapies:

How many companies are developing Primary Ciliary Dyskinesia drugs?

How many Primary Ciliary Dyskinesia drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Primary Ciliary Dyskinesia?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Primary Ciliary Dyskinesia therapeutics?

What are the recent trends, drug types and novel technologies developed to overcome the limitation of existing therapies?

What are the clinical studies going on for Primary Ciliary Dyskinesia and their status?

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

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