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Pulmonary alveolar proteinosis – Pipeline Insight, 2020

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Abstracts

This report can be delivered to the clients within 48-72 Hours

DelveInsight's, "Pulmonary alveolar proteinosis – Pipeline Insight, 2020," report provides comprehensive insights about 2+ companies and 2+ pipeline drugs in Pulmonary alveolar proteinosis 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

Pulmonary alveolar proteinosis Understanding

Pulmonary alveolar proteinosis: Overview

Pulmonary alveolar proteinosis (PAP) is a rare pulmonary disease characterized by alveolar accumulation of surfactant-derived lipoproteinaceous material. This accumulation blocks air from entering alveoli and oxygen from passing through into the blood, which results in a feeling of breathlessness (dyspnea). Depending on the aetiology three main categories of PAP have been defined: auto-immune (previously named primary or idiopathic), secondary and genetic. Genetic PAP is seen especially in children, and radio-clinical presentation depends on the mutated gene. Auto-immune alveolar proteinosis is the most frequent form of PAP, representing 90% of cases.

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Patients with PAP are at an increased risk of developing an opportunistic infection.

Symptoms

The symptoms of Pulmonary alveolar proteinosis include:

Cyanosis

Chest pain or tightness

Shortness of breath, also called dyspnea

Fever

Weight loss

Cough (sometimes, but not always)

Low levels of oxygen in the blood

Nail clubbing (abnormal growth of toenails or fingernails)

Fatigue

Diagnosis

Diagnosis of PAP is initiated by computed tomography (CT) scan and confirmed by staining of bronchoalveolar lavage fluid (BALF). PAP diagnosis rarely requires lung biopsy. Autoimmune PAP can be identified by very sensitive and specific blood tests that identify the presence or absence of an increased level of GM-CSF autoantibody. Hereditary PAP can be diagnosed by a series of blood tests. The genetic risk factors for hereditary PAP and congenital PAP can be identified by genetic testing.

Treatment

The standard treatment for PAP is symptomatic whole lung lavage. Therapy for PAP varies depending upon what disease is present, disease severity, and the age of the

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patient. Treatment of congenital PAP is generally supportive. Lung transplantation can be used in infants and children with congenital PAP caused by genetic mutations and in patients who develop significant lung fibrosis. Some novel therapies targeting alveolar macrophages (recombinant GM-CSF therapy) or anti-GM-CSF antibodies (rituximab and plasmapheresis) are under investigation.

Pulmonary alveolar proteinosis Emerging Drugs Chapters

This segment of the Pulmonary alveolar proteinosis 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.

Pulmonary alveolar proteinosis Emerging Drugs

Molgradex: Savara

Molgradex is an investigational inhaled formulation of recombinant human GM-CSF. It is being evaluated for the treatment of autoimmune pulmonary alveolar proteinosis (aPAP). It has been granted Orphan Drug Designation for the treatment of aPAP in the U.S. and the European Union. A pivotal Phase 3 clinical study, called IMPALA, of Molgradex for the treatment of aPAP was completed in 2019.

Sargramostim: Bayer HealthCare Pharmaceuticals

Sargramostim is a yeast-derived recombinant human granulocyte-macrophage colony stimulating factor (rhu-GM-CSF). The therapy stimulates the production of white blood cells including neutrophils, monocytes/macrophages and myeloid-derived dendritic cell and promote their ability to function. The U.S. Food and Drug Administration (FDA) has granted orphan drug designation to sargramostim), for the treatment of pulmonary alveolar proteinosis (PAP).

Further product details are provided in the report

Pulmonary alveolar proteinosis: Therapeutic Assessment

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

Major Players in Pulmonary alveolar proteinosis

There are approx. 2+ key companies which are developing the therapies for Pulmonary alveolar proteinosis. The companies which have their Pulmonary alveolar proteinosis drug candidates in the most advanced stage, i.e. phase III include, Savara.

Phases

DelveInsight's report covers around 2+ 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

Pulmonary alveolar proteinosis 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

intravitreal

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Subretinal

Topical.

Molecule Type

Products have been categorized under various Molecule types such as

Monoclonal Antibody

Peptides

Polymer

Small molecule

Gene therapy

Product Type

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

Pulmonary alveolar proteinosis: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase III, II, preclinical and discovery stage. It also analyses Pulmonary alveolar proteinosis 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 Pulmonary alveolar proteinosis drugs.

Report Highlights

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

On December 30, 2019, Savara announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation for Molgradex, an inhaled formulation of recombinant human granulocyte-macrophage colony-stimulating factor (GM-CSF), for the treatment of aPAP.

Pulmonary alveolar proteinosis Report Insights

Pulmonary alveolar proteinosis Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Pulmonary alveolar proteinosis Report Assessment

Pipeline Product Profiles

Therapeutic Assessment

Pipeline Assessment

Inactive drugs assessment

Unmet Needs

Key Questions

Current Treatment Scenario and Emerging Therapies:

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How many companies are developing Pulmonary alveolar proteinosis drugs?

How many Pulmonary alveolar proteinosis drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Pulmonary alveolar proteinosis?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Pulmonary alveolar proteinosis 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 Pulmonary alveolar proteinosis and their status?

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

Key Players

Savara

Black Tie Medical

Sanofi Genzyme

Bayer HealthCare Pharmaceuticals

Key Products

Molgradex

Cellular stromal vascular fraction (cSVF)

Sargramostim

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