

Familial Lipoprotein Lipase Deficiency – Pipeline Insight, 2021

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Abstracts

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DelveInsight's, "Familial Lipoprotein Lipase Deficiency - Pipeline Insight, 2021," report provides comprehensive insights about 7+ companies and 7+ pipeline drugs in Familial Lipoprotein Lipase Deficiency 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

Familial Lipoprotein Lipase Deficiency Understanding

Familial Lipoprotein Lipase Deficiency: Overview

Familial Lipoprotein Lipase Deficiency (LPLD), also known as Familial Chylomicronemia Syndrome (FCS) is a rare inherited condition, in which the normal breakdown of fats in the body is affected. The condition is inherited in an autosomal recessive pattern. In the past, familial lipoprotein lipase deficiency has also been called hyperlipoproteinemia type I. This deficiency is usually caused by a defect in the LPL gene, which encodes for an enzyme called lipoprotein lipase. A diagnosis of Familial Lipoprotein Lipase Deficiency may be done based upon identification of characteristic symptoms, a detailed patient history, and a thorough clinical evaluation. Blood tests can reveal



reduced activity of the lipoprotein lipase enzyme in the plasma. Treatment for Familial Lipoprotein Lipase Deficiency aims to control the symptoms and blood triglyceride levels with a very low-fat diet. Drugs that lower lipid levels in the body are not effective in reducing fat levels.

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

Familial Lipoprotein Lipase Deficiency Emerging Drugs Chapters

This segment of the Familial Lipoprotein Lipase Deficiency report encloses its detailed analysis of various drugs in different stages of clinical development, including phase III, 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.

Familial Lipoprotein Lipase Deficiency Emerging Drugs

IONIS-APOCIII-LRx: Ionis Pharmaceuticals

IONIS-APOCIII-LRx, formerly known as AKCEA-APOCIII-LRx, is a ligand-conjugated



(LICA) investigational antisense medicine designed to inhibit the production of apoC-III, for patients who are at risk of disease due to elevated triglyceride levels. The drug is in Phase III clinical evaluation for the treatment of familial lipoprotein lipase deficiency.

ARO-APOC3: Arrowhead Pharmaceuticals

ARO-APOC3 is a subcutaneously administered RNAi therapeutic that targets apolipoprotein C-III (apoC-III), and reduces VLDL synthesis and assembly, enhances the breakdown of triglyceride rich lipoproteins, and improve clearance of VLDL and chylomicron remnants. The drug is in Phase II clinical studies for the treatment of familial lipoprotein lipase deficiency.

Further product details are provided in the report.

Familial Lipoprotein Lipase Deficiency: Therapeutic Assessment

This segment of the report provides insights about the different Familial Lipoprotein Lipase Deficiency drugs segregated based on following parameters that define the scope of the report, such as:

Major Players in Familial Lipoprotein Lipase Deficiency

There are approx. 7+ key companies which are developing the therapies for Familial Lipoprotein Lipase Deficiency. The companies which have their Familial Lipoprotein Lipase Deficiency drug candidates in the most advanced stage, i.e. Phase III include, Ionis Pharmaceuticals.

Phases

DelveInsight's report covers around 7+ 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

Familial Lipoprotein Lipase Deficiency 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

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.

Familial Lipoprotein Lipase Deficiency: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase III, II, I, preclinical and discovery stage. It also analyses Familial Lipoprotein Lipase Deficiency 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 Familial Lipoprotein Lipase Deficiency drugs.

Familial Lipoprotein Lipase Deficiency Report Insights

Familial Lipoprotein Lipase Deficiency Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Familial Lipoprotein Lipase Deficiency Report Assessment

Pipeline Product Profiles

Therapeutic Assessment

Pipeline Assessment

Inactive drugs assessment

Unmet Needs

Familial Lipoprotein Lipase Deficiency – Pipeline Insight, 2021



Key Questions

Current Treatment Scenario and Emerging Therapies:

How many companies are developing Familial Lipoprotein Lipase Deficiency drugs?

How many Familial Lipoprotein Lipase Deficiency drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Familial Lipoprotein Lipase Deficiency?

What are the key collaborations (Industry-Industry, Industry-Academia), Mergers and acquisitions, licensing activities related to the Familial Lipoprotein Lipase Deficiency 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 Familial Lipoprotein Lipase Deficiency and their status?

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

Key Players

Amryt Pharma

Arrowhead Pharmaceuticals

Ionis Pharmaceuticals

iMetabolic Biopharma

Precision Biosciences



Pfizer

Lipigon Pharmaceuticals

Key Products

ARO-APOC3

iMBP-001

ApoC3 gene editing therapeutic

IONIS APOCIII LRx

Lomitapide

Vupanorsen

Lipisense



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