

# Focal Segmental Glomerulosclerosis (FSGS) – Pipeline Insight, 2020

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## Abstracts

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DelveInsight's, "Focal Segmental Glomerulosclerosis (FSGS) – Pipeline Insight, 2020," report provides comprehensive insights about 15+ companies and 15+ pipeline drugs in Focal Segmental Glomerulosclerosis 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

### Focal Segmental Glomerulosclerosis Understanding

#### Focal Segmental Glomerulosclerosis: Overview

Focal Segmental glomerulosclerosis is a type of glomerular disease and describes scarring (sclerosis) in your kidney. The scarring of FSGS only takes place in small sections of each glomerulus (filter), and only a limited number of glomeruli are damaged at first. Focal Segmental Glomerulosclerosis affects both children and adults. Males are affected slightly more often than females. FSGS is not caused by a single disease. It can have many different causes. The scarring may happen because of an infection, or drug, or a disease that affects the entire body, like diabetes, HIV infection, sickle cell disease or lupus. FSGS can also be caused by another glomerular disease that you had

before you got FSGS. FSGS has different types based on the cause.

Below are the types of FSGS:

**Primary FSGS:** This type of FSGS means that the disease happened on its own without a known or obvious cause.

**Secondary FSGS:** This type is caused by another disease or a drug. Examples include: viruses such as HIV or drugs such as anabolic steroids that some people use to speed up their muscle growth

## Symptoms

Early stages may not cause any symptoms. Signs and symptoms of FSGs include:

Swelling in body parts like your legs, ankles and around your eyes (called edema)

Weight gain due to extra fluid building in your body

Foamy urine caused by high protein levels in the urine (called proteinuria)

High fat levels in the blood (high cholesterol)

Low levels of protein in the blood

## Diagnosis

A blood test, urine test, and a kidney biopsy will determine if one have FSGS.

**Urine test:** A urine test will help find protein and blood in urine.

**Blood test:** A blood test will help find levels of protein, cholesterol, and wastes in blood.

**Glomerular filtration rate (GFR):** A blood test will be done to know how well kidneys are filtering the wastes from body.

**Kidney biopsy:** In this test, a tiny piece of kidney is removed with a special needle, and looked at under a microscope.

**Genetic testing:** A genetic test may be done to see if the patient was born with genes that caused kidney disease. This information may help doctor decide what type of treatment is best for the patient.

## Treatment

The type of treatment depends on the cause. Everyone is different and the doctor will make a treatment plan that is right for the type of FSGS. Usually, treatments for FSGS include:

Corticosteroids

Immunosuppressive drugs

Plasmapheresis

ACE inhibitors and ARBs

Diuretics

Diet change

## Focal Segmental Glomerulosclerosis Emerging Drugs Chapters

This segment of the Focal Segmental Glomerulosclerosis 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.

## Focal Segmental Glomerulosclerosis Emerging Drugs

Sparsentan: Retrophin

Sparsentan is a novel small-molecule candidate in phase III development for the treatment of focal segmental glomerulosclerosis (FSGS). Retrophin is examining the ability of sparsentan to slow the decline of kidney function in patients with FSGS and IgAN. It is a single molecule designed to be a dual endothelin receptor type A (ETA) and angiotensin II receptor type 1 (AT1) antagonist. The investigational approach is to attempt to reduce proteinuria, a characteristic marker of kidney injury in both FSGS and IgAN, with sparsentan. Lowering proteinuria levels is associated with better outcomes.

#### CXA-10: Complexa

CXA-10 is an oral nitrated fatty acid compound that acts through a constellation of actions including:

Upregulation of Nrf2 pathways, a key pro-reparative and metabolic pathway

Inhibition of NF-Kappa B and TLR4, key drivers of inflammatory molecules and pathways

Inhibition of Xanthine Oxidoreductase, a key intracellular producer of Reactive Oxygen Species add that result in cellular oxidative stress

Increasing the expression of heat shock proteins, which act as chaperones during cellular stress to restore cellular homeostatic mechanisms

#### GFB-887: Goldfinch Bio

GFB-887 is a TRPC5 ion channel inhibitor in development for the treatment of FSGS. TRPC5 is a calcium-permeable ion channel that has been implicated in the pathogenesis of FSGS by triggering a Rac1-TRPC5 disease pathway. TRPC5 represents a target for therapeutic intervention to halt progression of FSGS to kidney failure. It is currently in phase II stage of development.

Research programme: focal segmental glomerulosclerosis therapeutics - Delta 4

Delta 4 is working on its lead program which aims at developing a therapy for Focal Segmental Glomerulosclerosis (FSGS). Delta 4 concluded pre-clinical development with promising results. The Company initiated patent filing and is currently preparing for clinical testing of selected drug candidates.

Further product details are provided in the report

## Focal Segmental Glomerulosclerosis: Therapeutic Assessment

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

### Major Players in Focal Segmental Glomerulosclerosis

There are approx. 15+ key companies which are developing the therapies for Focal Segmental Glomerulosclerosis. The companies which have their Focal Segmental Glomerulosclerosis drug candidates in the most advanced stage, i.e. phase III include Retrophin and others

### Phases

DelveInsight's report covers around 15+ products under different phases of clinical development like

Late-stage products (Phase II and Phase II/III)

Mid-stage products (Phase II and Phase II/III)

Early-stage products (Phase I/II and Phase I) along with the details of

Pre-clinical and Discovery stage candidates

Discontinued & Inactive candidates

Route of Administration

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

Infusion

Intravenous

Intradermal

Intramuscular

Intranasal

Oral

Parenteral

Subcutaneous

Topical.

Molecule Type

Products have been categorized under various Molecule types such as

Gene therapies

Small molecule

Vaccines

Polymers

Peptides

Monoclonal antibodies

## Product Type

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

### Focal Segmental Glomerulosclerosis: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase II, I, preclinical and discovery stage. It also analyses Focal Segmental Glomerulosclerosis 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 Focal Segmental Glomerulosclerosis drugs.

### Report Highlights

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

In November 2015, Retrophin announced that the European Commission (EC) has granted sparsentan orphan drug designation for the treatment of focal segmental glomerulosclerosis (FSGS).

In January 2015, Retrophin announced that sparsentan received orphan drug designation from the US Food and Drug Administration (FDA) for the treatment of FSGS.

In October 2018, Variant Pharmaceuticals announced that it has filed an application with the FDA for orphan drug designation for VAR 200 for Focal Segmental Glomerulosclerosis.

## Focal Segmental Glomerulosclerosis Report Insights

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Current Treatment Scenario and Emerging Therapies:

How many companies are developing Focal Segmental Glomerulosclerosis drugs?

How many Focal Segmental Glomerulosclerosis drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Focal Segmental Glomerulosclerosis?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Focal Segmental Glomerulosclerosis 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 Focal Segmental Glomerulosclerosis and their status?

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

## Key Players

Dimerix Bioscience Pty Ltd

Astellas Pharma

Complexa

Aurinia Pharmaceuticals

FibroGen

Retrophin

Genzyme, a Sanofi Company

Vertex Pharmaceuticals

ChemoCentryx

Goldfinch

Delta 4

## Key Products

Propagermanium

Bleselumab

CXA-10

Voclosporin

FG-3019

VX-147

Fresolimumab

CCX140-B

RE-021

GFB-887

Research programme: focal segmental glomerulosclerosis therapeutics

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