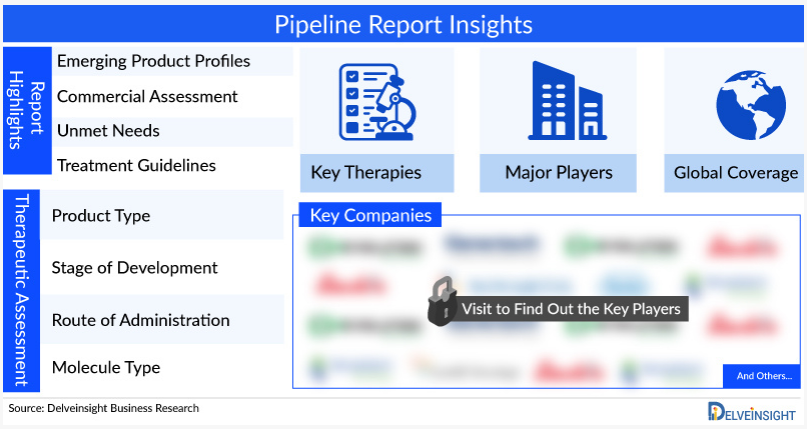
Fabry Disease Clinical Trial Pipeline, 18+ Companies Leading the Way in Advancing Treatment | DelveInsight

Major pharmaceutical companies are working to advance the pipeline space and unlock the future growth potential of the Fabry Disease treatment landscape.



Fabry Disease Clinical Trial Pipeline

LAS VEGAS , NV, UNITED STATES, February 13, 2025 /EINPresswire.com/ -- DelveInsight's 'Fabry Disease Pipeline Insight 2024' report provides comprehensive global coverage of pipeline Fabry Disease therapies in various stages of clinical development. Major pharmaceutical companies are working to advance the pipeline space and future growth potential of the Fabry Disease pipeline domain.

For Fabry Disease emerging drugs, the Fabry Disease pipeline analysis report provides a 360° view of the therapeutics landscape by development point, product type, route of administration, molecule type, and MOA. The pipeline research covers business opportunities, challenges, future partnerships, strong competitors, and growth strategies.

Key Takeaways from the Fabry Disease Pipeline Report
• DelveInsight's Fabry Disease Pipeline analysis depicts a robust space with 18+ active players working to develop 18+ pipeline drugs for Fabry Disease treatment.
• The leading Fabry Disease companies include Genzyme, Shire, Takeda, Protalix Biotherapeutics, Sangamo Therapeutics, Freeline Therapeutics, 4D Molecular Therapeutics, Idorsia Pharmaceuticals, Greenovation Biotech GmbH, and others are evaluating their lead assets to improve the Fabry Disease treatment landscape.
• Key Fabry Disease pipeline therapies in various stages of development include Venglustat (GZ402671), Isaralgagene civaparvovec (ST 920), PRX-102 (Elfabrio), and others.
• In December 2024, Exegenesis Bio announced that the US FDA granted Orphan Drug Designation to EXG110, a novel gene therapy for Fabry disease.
• In September 2024, uniQure N.V. announced that the US FDA granted Orphan Drug

Designation to AMT-191, its investigational gene therapy for Fabry disease.

Request a sample and discover the recent breakthroughs happening in the Fabry Disease pipeline landscape @ Fabry Disease Pipeline Outlook

Fabry Disease Overview
Fabry Disease is a rare genetic disorder caused by a deficiency of the enzyme alpha-galactosidase A, which is essential for breaking down globotriaosylceramide (Gb3 or GL-3) into usable components for the body's cells. This enzyme deficiency leads to the accumulation of Gb3 in various tissues, resulting in widespread symptoms.

Common symptoms of Fabry Disease include persistent burning or tingling pain in the hands and feet, clusters of small dark red skin lesions, cloudy corneas that affect vision, tinnitus, and hearing loss. The condition arises from mutations in the GLA gene, which encodes the alpha-galactosidase A enzyme. In severe cases, the enzyme is entirely nonfunctional, while milder cases may retain some activity.

Fabry Disease follows an X-linked dominant inheritance pattern, meaning the defective gene is located on the X chromosome. This inheritance pattern influences how the disease is passed down and affects individuals differently based on their genetic makeup.

Find out more about Fabry Disease medication @ https://www.delveinsight.com/report-store/fabry-disease-pipeline-insight?utm_source=einpresswire&utm_medium=pressrelease&utm_campaign=jpr

Fabry Disease Treatment Analysis: Drug Profile

Pegunigalsidase Alfa: Protalix Biotherapeutics
Pegunigalsidase alfa (PRX-102) is an investigational, plant cell culture-expressed, and chemically modified stabilized version of the recombinant α-Galactosidase-A enzyme. Protein sub-units are covalently bound via chemical cross-linking using short PEG moieties, resulting in a molecule with unique pharmacokinetic parameters. In clinical studies, PRX–102 has been observed to have a circulatory half-life of approximately 80 hours. PRX–102 has been designed to potentially address the continued unmet clinical need in Fabry patients. In May 2020, the companies filed an application with the U.S. Food and Drug Administration (FDA) seeking the accelerated approval of PRX-102, given at a dose of 1 mg/kg every other week, for the treatment of adults with Fabry. PRX-102 received orphan drug designation in Europe and fast-track designation in the U.S. Both designations are meant to speed up the therapy’s development and review process. After granting it priority review, the agency rejected the application in April 2022 due to issues with facility inspections and manufacturing processes, partially caused by travel restrictions during the COVID-19 pandemic. Protalix and Chiesi have requested a meeting with the FDA to discuss the regulatory path toward PRX-102’s approval in the U.S. The companies plan to file a similar regulatory application with the European Medicines Agency later this year to seek

the therapy's approval in the EU.

Venglustat: Sanofi
GSLs are cellular building blocks whose abnormal accumulation is implicated in several rare diseases, responsible for both cell dysfunction and disease progression. Venglustat is a novel, oral investigational therapy that has the potential to slow the progression of certain diseases by inhibiting abnormal GSL accumulation. Venglustat is currently under clinical investigation and its safety and efficacy have not been evaluated by any regulatory authority. Venglustat continues in Phase II for Gaucher, Fabry, Tay-Sachs and Sandhoff diseases. In 2015, the FDA fast-tracked this drug for Fabry disease.

Key Fabry Disease Therapies and Companies
• Pegunigalsidase Alfa: Protalix Biotherapeutics
• Venglustat (GZ402671) : Sanofi/Genzyme
• Isaralgagene civaparvovec (ST 920) : Sangamo Therapeutics
• PRX-102 (Elfabrio): Chiesi rare diseases/ Protalix Biotherapeutics

Learn more about the novel and emerging Fabry Disease pipeline therapies @ https://www.delveinsight.com/report-store/fabry-disease-pipeline-insight?utm_source=einpresswire&utm_medium=pressrelease&utm_campaign=jpr

Fabry Disease Therapeutics Assessment

By Product Type
• Mono
• Combination
• Mono/Combination.

By Stage
• 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

By Route of Administration
• Oral
• Parenteral
• Intravitreal
• Subretinal
• Topical.

By Molecule Type

• Recombinant fusion proteins
• Small molecule
• Monoclonal antibody
• Peptide
• Polymer
• Gene therapy

Scope of the Fabry Disease Pipeline Report
• Coverage: Global
• Key Fabry Disease Companies: Genzyme, Shire, Takeda, Protalix Biotherapeutics, Sangamo Therapeutics, Freeline Therapeutics, 4D Molecular Therapeutics, Idorsia Pharmaceuticals, Greenovation Biotech GmbH, and others.
• Key Fabry Disease Pipeline Therapies: Venglustat (GZ402671), Isaralgagene civaparvovec (ST 920), PRX-102 (Elfabrio), and others.

Dive deep into rich insights for drugs used for Fabry Disease treatment; visit @ Fabry Disease Drugs

Table of Contents

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