

# Adrenoleukodystrophy (ALD) - Market Insights, Epidemiology and Market Forecast-2030

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# **Abstracts**

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DelveInsight's 'Adrenoleukodystrophy (ALD) - Market Insights, Epidemiology and Market Forecast-2030' report delivers an in-depth understanding of the Adrenoleukodystrophy, historical and forecasted epidemiology as well as the Adrenoleukodystrophy market trends in the United States, EU5 (Germany, Spain, Italy, France, and the United Kingdom) and Japan.

The Adrenoleukodystrophy (ALD) market report provides current treatment practices, emerging drugs, and Adrenoleukodystrophy market share of the individual therapies, current and forecasted Adrenoleukodystrophy market size from 2017 to 2030 segmented by seven major markets. The Report also covers current Adrenoleukodystrophy treatment practice, market drivers, market barriers and unmet medical needs to curate the best of the opportunities and assesses the underlying potential of the market.

Geography Covered

The United States

EU5 (Germany, France, Italy, Spain, and the United Kingdom)

Japan

Study Period: 2017–2030



Adrenoleukodystrophy (ALD) Disease Understanding and Treatment Algorithm

# Adrenoleukodystrophy Overview

Adrenoleukodystrophy (ALD) is an X-linked recessive genetic disorder caused by the abnormality in the ABCD1 gene present on the X chromosome that leads to the accumulation of very-long-chain fatty acids (VLCFAs) in the brain, nervous system, and adrenal gland. The accumulation of VLCFAs leads to the deterioration of the myelin sheath, and without the sheath, neurons cannot conduct action potentials, this leads to the development of seizures and hyperactivity. Currently, no drug is approved for the treatment of Adrenoleukodystrophy and the available treatment options include hormone replacement therapy, dietary therapy with Lorenzo's oil and Hematopoietic stem cell transplantation, using either umbilical cord or bone marrow stem cells.

# Adrenoleukodystrophy Diagnosis

The diagnosis of Adrenoleukodystrophy is difficult as its symptoms are similar to other diseases such as attention deficit hyperactivity disorder, epilepsy, autism, and other learning disabilities. The diagnosis is done by the use of diagnostic assays, imaging tests, adrenal function testing, and prenatal diagnosis. In recent years various countries started the newborn screening programs to identify the conditions that may affect a baby's long-term health and survival. The newborn screening programs also included the screening of Adrenoleukodystrophy and gained huge success in early detection and cost-saving.

# Adrenoleukodystrophy Treatment

The DelveInsight Adrenoleukodystrophy market report gives a thorough understanding of Adrenoleukodystrophy by including details such as disease definition, clinical aspects, symptoms, causes, inheritance pattern, pathophysiology, and diagnosis. It covers the details of conventional and current medical therapies available in the Adrenoleukodystrophy market for the treatment of the condition. It also provides treatment guidelines for Adrenoleukodystrophy in the US and Europe.

### Adrenoleukodystrophy Epidemiology

The disease epidemiology covered in the report provides historical as well as forecasted epidemiology segmented by Total Prevalent Population of Adrenoleukodystrophy in the



7MM, Gender-specific Prevalence of Adrenoleukodystrophy in the 7MM, Gender-specific Diagnosed Prevalence of Adrenoleukodystrophy in the 7MM, and Type-specific Diagnosed Prevalence of ALD in Males in the 7MM. The epidemiology section covers the 7MM countries, the United States, EU5 countries (Germany, France, Italy, Spain, and the United Kingdom), and Japan from the year 2017 to 2030.

# **Key Findings**

As per DelveInsight's analysis, in 2017, the total prevalent population of Adrenoleukodystrophy in the 7MM was 55,242. Based on the type of ALD, it was also assessed that majority of the patients suffer from Cerebral ALD (CALD), with 1,160 cases observed in the US, in 2017, while Adrenomyeloneuropathy (AMN) and Addison Disease account for nearly 774, and 314 cases of the total diagnosed male symptomatic ALD population. Out of the total diagnosed male cases, CALD includes 1,160 cases, which is further divided based on age, i.e., Childhood CALD (894 cases), Adolescent CALD (169 cases), and Adult CALD (97 cases) in 2017 in the US.

Among the European five countries, Germany had the highest diagnosed prevalent population of ALD with 1,895 cases, followed by France and the United Kingdom. On the other hand, Italy had the lowest diagnosed prevalent population of 1,126 in 2017.

Country Wise- Adrenoleukodystrophy Epidemiology

The epidemiology segment also provides the Adrenoleukodystrophy epidemiology data and findings across the United States, EU5 (Germany, France, Italy, Spain, and the United Kingdom) and Japan.

Adrenoleukodystrophy Drug Chapters

The drug chapter segment of the Adrenoleukodystrophy report encloses the detailed analysis of Adrenoleukodystrophy late stage (Phase III and Phase II) pipeline drugs. It also helps to understand the Adrenoleukodystrophy clinical trial details, expressive pharmacological action, agreements and collaborations, approval and patent details, advantages and disadvantages of drugs, and the latest news and press releases around them.

Adrenoleukodystrophy Emerging Drugs

Lenti D: Bluebird Bio



Lenti D is an investigational gene therapy that is in Phase II/III stage of development for the treatment of cerebral ALD (CALD). Currently, bluebird bio is enrolling patients for a Phase III study (ALD-104) designed to assess the efficacy and safety of Lenti-D after myeloablative conditioning using busulfan and fludarabine in patients with CALD. Additionally, the company is conducting a long-term safety and efficacy follow-up study (LTF-304) for patients who have participated in ALD-102 and were treated with Lenti-D.

Leriglitazone: Minoryx Therapeutics, S.L.

Leriglitazone (MIN-102) is a metabolite of pioglitazone which shows an excellent brain penetration and safety profile, allowing PPAR gamma engagement in the CNS above the level that can be safely achieved with pioglitazone and other glitazones. The drug candidate is in Phase II/III stage of development for the treatment of both cerebral ALD and Adrenomyeloneuropathy (AMN).

Products detail in the report...

Adrenoleukodystrophy Market Outlook

The Adrenoleukodystrophy market outlook of the report helps to build a detailed comprehension of the historic, current and forecasted Adrenoleukodystrophy market trends by analyzing the impact of current and emerging therapies on the market, unmet needs, drivers and barriers and demand of better technology.

This segment gives a thorough detail of Adrenoleukodystrophy market trend of each current available and late-stage pipeline therapy by evaluating their impact based on annual cost of therapy, inclusion and exclusion criteria's, mechanism of action, compliance rate, growing need of the market, increasing patient pool, covered patient segment, expected launch year, competition with other therapies, brand value, their impact on the market and view of the key opinion leaders. The calculated market data are presented with relevant tables and graphs to give a clear view of the market at first sight.

According to DelveInsight, Adrenoleukodystrophy market in 7MM is expected to change in the study period 2017–2030.

**Key Findings** 



This section includes a glimpse of the Adrenoleukodystrophy market in the 7MM.

The United States Market Outlook

This section provides the total Adrenoleukodystrophy market size and market size by therapies in the United States.

The market size for ALD was USD 993 million in 2017 in the Seven Major Markets. DelveInsight's analysts estimate that the market is expected to experience a significant rise in the coming years. Although, a noticeable growth can be seen in the upcoming year, owing to the launch of Lenti-D (Gene therapy; Bluebird Bio), and Leriglitazone (MIN-102) (Minoryx Therapeutics).

EU-5 Countries: Market Outlook

The total Adrenoleukodystrophy market size and market size by therapies in Germany, France, Italy, Spain, and the United Kingdom are provided in this section.

Japan Market Outlook

The total market size of Adrenoleukodystrophy in Japan was USD 111.57 million in 2017.

Adrenoleukodystrophy Drugs Uptake

This section focusses on the rate of uptake of the therapies in the market and potential drugs expected to get launched in the market during the study period 2017–2030. The analysis covers Adrenoleukodystrophy market uptake by drugs; patient uptake by therapies; and sales of each drug.

This helps in understanding the drugs with the most rapid uptake, reasons behind the maximal use of new drugs and allows the comparison of the drugs based on market share and size which again will be useful in investigating factors important in market uptake and in making financial and regulatory decisions.

Adrenoleukodystrophy Pipeline Development Activities

The report provides insights into different therapeutic candidates in Phase II, and Phase III stage. It also analyses Adrenoleukodystrophy key players involved in developing



targeted therapeutics.

Pipeline Development Activities

The report covers the detailed information of collaborations, acquisition, merger, licensing, and patent details for Adrenoleukodystrophy emerging therapies.

Reimbursement Scenario in Adrenoleukodystrophy

Approaching reimbursement proactively can have a positive impact both during the late stages of product development and well after product launch. In the report we consider reimbursement to identify economically attractive indications and market opportunities. When working with finite resources, the ability to select the markets with the fewest reimbursement barriers can be a critical business and price strategy.

**KOL-Views** 

To keep up with current market trends, we take KOLs and SME's opinion working in the Adrenoleukodystrophy domain through primary research to fill the data gaps and validate our secondary research. Their opinion helps to understand and validate current and emerging therapies treatment patterns or the Adrenoleukodystrophy market trend. This will support the clients in potential upcoming novel treatment by identifying the overall scenario of the market and the unmet needs.

Competitive Intelligence Analysis

We perform Competitive and Market Intelligence analysis of the Adrenoleukodystrophy Market by using various Competitive Intelligence tools that includes – SWOT analysis, PESTLE analysis, Porter's five forces, BCG Matrix, Market entry strategies, etc. The inclusion of the analysis entirely depends upon the data availability.

Scope of the Report

The report covers the descriptive overview of Adrenoleukodystrophy, explaining its clinical aspects, symptoms, causes, inheritance pattern, pathophysiology, diagnosis, and currently available therapies.

Comprehensive insight has been provided into the Adrenoleukodystrophy epidemiology and treatment in the 7MM.



Additionally, an all-inclusive account of both the current and emerging therapies for Adrenoleukodystrophy is provided, along with the assessment of new therapies, which will have an impact on the current treatment landscape.

A detailed review of Adrenoleukodystrophy market; historical and forecasted is included in the report, covering drug outreach in the 7MM

The report provides an edge while developing business strategies, by understanding trends shaping and driving the 7MM Adrenoleukodystrophy market

# Report Highlights

In the coming years, Adrenoleukodystrophy market is set to change due to the increased number of potential therapies in the pipeline, awareness about newborn screening programs as well as an increase in the number of CALD cases.

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

Major players are involved in developing therapies for Adrenoleukodystrophy. Launch of emerging therapies will significantly impact the Adrenoleukodystrophy market.

A better understanding of disease pathogenesis will also contribute to the development of novel therapeutics for Adrenoleukodystrophy

Our in-depth analysis of the pipeline assets across different stages of development (Phase III and Phase II), different emerging trends and comparative analysis of pipeline products with detailed clinical profiles, key cross-competition, launch date along with product development activities will support the clients in the decision-making process regarding their therapeutic portfolio by identifying the overall scenario of the research and development activities



# Adrenoleukodystrophy Report Insights

Patient Population

Therapeutic Approaches

Adrenoleukodystrophy Pipeline Analysis

Adrenoleukodystrophy Market Size and Trends

Market Opportunities

Impact of upcoming Therapies

# Adrenoleukodystrophy Report Key Strengths

11 Years Forecast

7MM Coverage

Adrenoleukodystrophy Epidemiology Segmentation

**Key Cross Competition** 

Highly Analyzed Market

**Drugs Uptake** 

Adrenoleukodystrophy Report Assessment

**Current Treatment Practices** 

**Unmet Needs** 

Pipeline Product Profiles



Market Attractiveness

Market Drivers and Barriers

# **Key Questions**

# Market Insights:

What was the Adrenoleukodystrophy Market share (%) distribution in 2017 and how it would look like in 2030?

What would be the Adrenoleukodystrophy total market size as well as market size by therapies across the 7MM during the forecast period (2017–2030)?

What are the key findings of the market across 7MM and which country will have the largest Adrenoleukodystrophy market size during the forecast period (2017–2030)?

At what CAGR, the Adrenoleukodystrophy market is expected to grow in 7MM during the forecast period (2017–2030)?

What would be the Adrenoleukodystrophy market outlook across the 7MM during the forecast period (2017–2030)?

What would be the Adrenoleukodystrophy market growth till 2030 and what will be the resultant market size in the year 2030?

How would the market drivers, barriers and future opportunities affect the market dynamics and subsequent analysis of the associated trends?

### **Epidemiology Insights:**

What is the disease risk, burden and unmet needs of the Adrenoleukodystrophy?

What is the historical Adrenoleukodystrophy patient pool in seven major markets



covering the United States, EU5 (Germany, Spain, France, Italy, UK) & Japan?

What would be the forecasted patient pool of Adrenoleukodystrophy in seven major markets covering the United States, EU5 (Germany, Spain, France, Italy, UK) & Japan?

What will be the growth opportunities in the 7MM concerning the patient population of Adrenoleukodystrophy?

Out of all 7MM countries, which country would have the highest Incident population of Adrenoleukodystrophy during the forecast period (2017–2030)?

At what CAGR the population is expected to grow in 7MM during the forecast period (2017–2030)?

Current Treatment Scenario, Marketed Drugs, and Emerging Therapies:

What are the current options for the treatment of Adrenoleukodystrophy along with the approved therapy?

What are the current treatment guidelines for the treatment of Adrenoleukodystrophy in the US and Europe?

What are the Adrenoleukodystrophy marketed drugs and their MOA, regulatory milestones, product development activities, advantages, disadvantages, safety, and efficacy, etc.?

How many companies are developing therapies for the treatment of Adrenoleukodystrophy?

How many therapies are developed by each company for the treatment of Adrenoleukodystrophy?

How many emerging therapies are in the mid-stage and late stage of development for the treatment of Adrenoleukodystrophy?

What are the key collaborations (Industry–Industry, Industry-Academia), Mergers and acquisitions, licensing activities related to the



Adrenoleukodystrophy therapies?

What are the recent novel therapies, targets, mechanisms of action and technologies developed to overcome the limitation of existing therapies?

What are the clinical studies going on for Adrenoleukodystrophy and their status?

What are the key designations that have been granted for the emerging therapies for Adrenoleukodystrophy?

What are the 7MM historical and forecasted market of Adrenoleukodystrophy?

# Reasons to buy

The report will help in developing business strategies by understanding trends shaping and driving the Adrenoleukodystrophy market.

To understand the future market competition in the Adrenoleukodystrophy market and insightful review of the key market drivers and barriers.

Organize sales and marketing efforts by identifying the best opportunities for Adrenoleukodystrophy in the US, Europe (Germany, Spain, Italy, France, and the United Kingdom) and Japan.

Identification of strong upcoming players in the market will help in devising strategies that will help in getting ahead of competitors

Organize sales and marketing efforts by identifying the best opportunities for Adrenoleukodystrophy market

To understand the future market competition in the Adrenoleukodystrophy market



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