

MELAS Syndrome Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 2024-2034

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Abstracts

The MELAS Syndrome market reached a value of US\$ 154.8 Million across the top 7 markets (US, EU4, UK, and Japan) in 2023. Looking forward, IMARC Group expects the 7MM to reach US\$ 447.7 Million by 2034, exhibiting a growth rate (CAGR) of 10.14% during 2024-2034.

The MELAS syndrome market has been comprehensively analyzed in IMARC's new report titled "MELAS Syndrome Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 2024-2034". MELAS syndrome, an acronym for mitochondrial encephalopathy, lactic acidosis, and stroke-like episodes, is a rare and complex mitochondrial disorder that affects multiple organ systems. It is primarily caused by mutations in mitochondrial DNA, leading to impaired energy production within cells. The hallmark symptoms of the ailment usually emerge during childhood or early adulthood. Patients often experience recurrent stroke-like episodes characterized by neurological indications, such as seizures, migraines, and focal neural deficits. Additionally, lactic acidosis, an accumulation of lactic acid in the bloodstream, may cause muscle weakness, fatigue, and metabolic disturbances. Several other common features include cognitive decline, hearing loss, and heart abnormalities. Diagnosing MELAS syndrome can be challenging due to its variable presentation and similarity to other conditions. The healthcare provider often relies on a combination of clinical history, physical examination, and specialized tests, such as genetic testing, to detect mutations in mitochondrial DNA. Brain imaging and muscle biopsies may also aid in confirming the diagnosis.

The growing prevalence of somatic mutations in the mitochondrial DNA, particularly in the MT-TL1 and MT-ND5 genes, which can result in disruption of cells and tissues is



primarily driving the MELAS syndrome market. In addition to this, the increasing usage of effective medications, such as antioxidants, coenzyme Q10 supplements, L-arginine, etc., to manage the symptoms of the illness and improve mitochondrial function is also fostering a positive outlook for the market. These therapeutic agents aim to alleviate the severity of indications and enhance the overall quality of life for patients. Furthermore, the widespread adoption of physical and occupational therapies, which play a crucial role in managing the disease's debilitating effects by strengthening muscles, enhancing coordination, and improving gross motor skills, is further bolstering the market growth. Apart from this, the continuous advancements in gene therapy approach that hold promising potential for the prevention and treatment of complex and challenging disorders are acting as another significant growth-inducing factor. Additionally, the emerging popularity of innovative procedures, like mitochondrial replacement therapy, since it involves replacing defective mitochondrial genes with healthy donor DNA to correct the underlying genetic abnormalities responsible for the condition, is expected to drive the MELAS syndrome market during the forecast period.

IMARC Group's new report provides an exhaustive analysis of the MELAS syndrome market in the United States, EU4 (Germany, Spain, Italy, and France), United Kingdom, and Japan. This includes treatment practices, in-market, and pipeline drugs, share of individual therapies, market performance across the seven major markets, market performance of key companies and their drugs, etc. The report also provides the current and future patient pool across the seven major markets. According to the report, the United States has the largest patient pool for MELAS syndrome and also represents the largest market for its treatment. Furthermore, the current treatment practice/algorithm, market drivers, challenges, opportunities, reimbursement scenario and unmet medical needs, etc. have also been provided in the report. This report is a must-read for manufacturers, investors, business strategists, researchers, consultants, and all those who have any kind of stake or are planning to foray into the MELAS syndrome market in any manner.

Recent Developments:

In February 2024, PTC Therapeutics confirmed the phase III status for the pipeline drug of MELAS (Mitochondrial Encephalomyopathy, Lactic Acidosis, and Stroke-Like Episodes)

In October 2023, Tisento Therapeutics revealed the initiation of a patient interview study. This study seeks to uncover and describe the most burdensome signs, symptoms, and impacts on the quality of life experienced by adults and adolescents living with MELAS, a rare mitochondrial disease.

In May 2023, Cyclerion Therapeutics, Inc. disclosed in a press release that it had



finalized a definitive contract valued at US\$ 81 Million with a recently established private entity, referred to as "NewCo." This agreement was geared towards advancing the development of zagociguat (formerly identified as CY6463) for addressing MELAS* and various other conditions associated with mitochondrial dysfunction, alongside furthering the progress of CY3018.

In March 2023, Cyclerion Therapeutics, Inc. announced that Zagociguat (formerly known as CY6463) had been granted orphan drug designation by the U.S. Food and Drug Administration (FDA) for the treatment of mitochondrial diseases.

Key Highlights:

MELAS (Mitochondrial Encephalopathy, Lactic Acidosis, and Stroke-like episodes) syndrome is a rare condition typically onset in childhood, occurring between the ages of two and fifteen.

MELAS is recognized as one of the prevalent mitochondrial diseases, affecting approximately 1 in 5,000 individuals worldwide.

It occurs with equal frequency among both women and individuals assigned female at birth (AFAB), as well as men and individuals assigned male at birth (AMAB). MELAS is commonly diagnosed during childhood, typically between the ages of 2 and 15.

Drugs:

CY 6463, previously known as IW 6463, is an orally administered small molecule that stimulates soluble guanylate cyclase (sGC). Developed by Cyclerion Therapeutics, it serves as an sGC stimulator, functioning as a positive allosteric modulator to amplify the signaling of endogenous nitric oxide (NO). Cyclerion Therapeutics has outlined its intentions to proceed with a phase IIa trial for MELAS, utilizing CY-6463 as the investigational compound.

The development of the KL1333 project has reached the Phase I stage, aiming to establish its efficacy for the chronic oral treatment of primary mitochondrial disorders, particularly those within the MELAS-MIDD spectrum. KL1333 functions as a potent modulator of cellular NAD+ levels, essential for cell energy metabolism. Currently, patient studies are ongoing to assess KL1333, which has received orphan drug designation in both the United States and Europe.

Vatiquinone, under development for the treatment MELAS (Mitochondrial Encephalomyopathy, Lactic Acidosis, and Stroke-like episodes), is an orally administered small molecule, it serves as 15-lipoxygenase inhibitors, Antioxidants and NQO1 modulators.



Time Period of the Study

Base Year: 2023 Historical Period: 2018-2023 Market Forecast: 2024-2034

Countries Covered

United States Germany France United Kingdom Italy Spain Japan

Analysis Covered Across Each Country

Historical, current, and future epidemiology scenario Historical, current, and future performance of the MELAS syndrome market Historical, current, and future performance of various therapeutic categories in the market Sales of various drugs across the MELAS syndrome market Reimbursement scenario in the market In-market and pipeline drugs

Competitive Landscape:

This report also provides a detailed analysis of the current MELAS syndrome marketed drugs and late-stage pipeline drugs.

In-Market Drugs

Drug Overview Mechanism of Action Regulatory Status Clinical Trial Results Drug Uptake and Market Performance

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Late-Stage Pipeline Drugs

Drug Overview Mechanism of Action Regulatory Status Clinical Trial Results Drug Uptake and Market Performance

*Kindly note that the drugs in the above table only represent a partial list of marketed/pipeline drugs, and the complete list has been provided in the report.

Key Questions Answered in this Report: Market Insights

How has the MELAS syndrome market performed so far and how will it perform in the coming years?

What are the markets shares of various therapeutic segments in 2023 and how are they expected to perform till 2034?

What was the country-wise size of the MELAS syndrome market across the seven major markets in 2023 and what will it look like in 2034?

What is the growth rate of the MELAS syndrome market across the seven major markets and what will be the expected growth over the next ten years? What are the key unmet needs in the market?

Epidemiology Insights

What is the number of prevalent cases (2018-2034) of MELAS syndrome across the seven major markets?

What is the number of prevalent cases (2018-2034) of MELAS syndrome by age across the seven major markets?

What is the number of prevalent cases (2018-2034) of MELAS syndrome by gender across the seven major markets?

How many patients are diagnosed (2018-2034) with MELAS syndrome across the seven major markets?

What is the size of the MELAS syndrome patient pool (2018-2023) across the seven major markets?

What would be the forecasted patient pool (2024-2034) across the seven major



markets?

What are the key factors driving the epidemiological trend of MELAS syndrome? What will be the growth rate of patients across the seven major markets?

MELAS Syndrome: Current Treatment Scenario, Marketed Drugs and Emerging Therapies

What are the current marketed drugs and what are their market performance? What are the key pipeline drugs and how are they expected to perform in the coming years?

How safe are the current marketed drugs and what are their efficacies?

How safe are the late-stage pipeline drugs and what are their efficacies?

What are the current treatment guidelines for MELAS syndrome drugs across the seven major markets?

Who are the key companies in the market and what are their market shares? What are the key mergers and acquisitions, licensing activities, collaborations, etc. related to the MELAS syndrome market?

What are the key regulatory events related to the MELAS syndrome market? What is the structure of clinical trial landscape by status related to the MELAS syndrome market?

What is the structure of clinical trial landscape by phase related to the MELAS syndrome market?

What is the structure of clinical trial landscape by route of administration related to the MELAS syndrome market?



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