

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

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## Abstracts

The 7 major Fragile X syndrome markets reached a value of US\$ 43.1 Million in 2023. Looking forward, IMARC Group expects the 7MM to reach US\$ 66.9 Million by 2034, exhibiting a growth rate (CAGR) of 4.07% during 2024-2034.

The Fragile X syndrome market has been comprehensively analyzed in IMARC's new report titled "Fragile X Syndrome Market: Epidemiology, Industry Trends, Share, Size, Growth, Opportunity, and Forecast 2024-2034". Fragile X syndrome refers to an X-linked genetic disorder that causes a variety of intellectual disabilities, such as learning problems and cognitive impairment. This condition is caused by variations in the fragile X messenger ribonucleoprotein 1 (FMR1) gene, responsible for brain growth. Affected individuals typically have delayed speech and language development by the age of two. The disease is prominently characterized by an elongated face, flat feet, large or protruding ears, low muscle tone, larger testes, etc. In addition, patients suffering from Fragile X syndrome may experience hyperactivity, anxiety, seizures, a low intelligence quotient, aggression, irritation, sleep problems, etc. The other behavioral indications include atypical social development, memory deficits, stereotypic movements (e.g., hand flapping), limited eye contact, shyness, difficulty with face encoding, etc. This ailment is generally diagnosed by evaluating the patient's medical history and clinical presentation. The common investigations to identify the condition are genetic testing, polymerase chain reaction (PCR), and methylation status through southern blot analysis. Various other diagnostic tools may include physical and neurological exams, developmental assessments, and behavioral evaluations.

The rising prevalence of genetic disorders on account of DNA abnormalities, gene mutations, and chromosomal damage is primarily driving the Fragile X syndrome

market. Furthermore, the expanding male population, who are prone to single X chromosome gene variations, is also augmenting the market growth. Apart from this, the widespread adoption of efficient drugs, such as benzodiazepines and atypical antipsychotic agents, to target irritability and aggression by causing sedation and reducing anxiety among patients is further creating a positive outlook for the market. Additionally, the inflating application of prenatal diagnostic procedures, including amniocentesis and chorionic villus sampling, which identify the FMR1 gene mutation in the fetus and provide early diagnosis of the syndrome, is also bolstering the market growth. Moreover, the emerging popularity of speech-language and behavioral therapy, alongside pharmacological treatment, to support the management of the underlying condition is acting as another significant growth-inducing factor. In addition to this, the increasing utilization of gene therapy for delivering a functional FMR1 gene into cells using a safe and modified adeno-associated viral vector to promote the synthesis of FMR protein is expected to drive the Fragile X syndrome market in the coming years.

IMARC Group's new report provides an exhaustive analysis of the Fragile X 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 Fragile X syndrome and also represents the largest market for its treatment. Furthermore, the current treatment practice/algorithm, market drivers, challenges, opportunities, reimbursement scenario, 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 Fragile X syndrome market in any manner.

### Recent Developments

In September 2023, Shionogi & Co., Ltd. reported that the United States FDA has granted Tetra Therapeutics Inc., a Rare Pediatric Disease Designation for zatolmilast (BPN14770), which is an experimental medication being evaluated for Fragile X syndrome.

In July 2023, Acadia Pharmaceuticals Inc. disclosed that it had expanded its existing licensing agreement with Neuren Pharmaceuticals for trofinetide to obtain ex-North American rights to the medicine as well as global rights in Fragile X syndrome to Neuren's research candidate NNZ-2591.

In May 2023, Allos Pharma Inc. revealed that it has met with the United States FDA to

improve the design of its Phase 3 trial. This trial is intended to support a New Drug Application (NDA) for Fragile X Syndrome. Allos' investigational medication, arbaclofen, targets the molecular pathogenesis of Fragile X Syndrome and has shown significant efficacy in an FDA-approved Phase 3 trial.

In February 2023, KGK Science announced that it had secured an exemption under Canada's Controlled Drugs and Substances Act, beginning with its partner Nova Mentis Life Science. According to the firm, this is the first Phase IIa clinical trial research to evaluate the efficacy of repeated, oral microdosing of psilocybin as a Fragile X syndrome treatment.

#### Key Highlights:

According to the CDC, around 46% of males and 16% of females with Fragile X syndrome also have autism.

Fragile X syndrome often affects males more than females.

According to research studies, around one in every 7,000 men and one in every 11,000 women have been diagnosed with Fragile X syndrome.

Individuals with Fragile X syndrome are more likely to develop seizures, with rates ranging from 10% to 40% reported in the literature.

Based on multiple prevalence studies, it is estimated that about one million Americans carry the Fragile X premutation.

#### Drugs:

Zatolmilast is an investigational medicine for the treatment of Fragile X syndrome. The drug is designed to act by altering a signaling molecule called cyclic AMP (cAMP), which may enhance the maturation of connections between neurons that are deficient in individuals with Fragile X syndrome.

ZYN002 cannabidiol (CBD) gel is the first and only pharmaceutically manufactured CBD made as a patent-protected permeation-enhanced gel, and it is being tested on children with Fragile X Syndrome. ZYN002 is a transparent, permeation-enhanced gel intended to administer controlled drugs transdermally with once- or twice-daily dosing.

Acamprosate is an innovative, clinically discovered medication that functions as an NMDA receptor inhibitor and GABA receptor agonist. It is intended to treat the core social and communicative problems associated with Fragile X syndrome. The medicine aims to target vital capabilities, such as speech, thinking, social engagement, and self-care.

## 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 Fragile X syndrome market

Historical, current, and future performance of various therapeutic categories in the market

Sales of various drugs across the Fragile X syndrome market

Reimbursement scenario in the market

In-market and pipeline drugs

## Competitive Landscape:

This report also provides a detailed analysis of the current Fragile X 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

## Late-Stage Pipeline Drugs

Drug Overview

Mechanism of Action

Regulatory Status

Clinical Trial Results

Regulatory Status

\*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 Fragile X 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 Fragile X syndrome market across the seven major markets in 2023 and what will it look like in 2034?

What is the growth rate of the Fragile X 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 Fragile X syndrome across the seven major markets?

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

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

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

What is the size of the Fragile X 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 Fragile X syndrome?

What will be the growth rate of patients across the seven major markets?

Fragile X 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 Fragile X 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 Fragile X syndrome market?

What are the key regulatory events related to the Fragile X syndrome market?

What is the structure of clinical trial landscape by status related to the Fragile X syndrome market?

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

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

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