

# **Cystic Fibrosis Market - A Global and Regional Analysis: Focus on Country and Region - Analysis and Forecast, 2025-2035**

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## **Abstracts**

Cystic fibrosis is a genetic disorder that primarily affects the lungs and digestive system, characterized by the production of thick, sticky mucus that can clog airways and lead to respiratory infections, digestive issues, and a range of other health problems. It is one of the most common life-limiting inherited diseases in children and young adults. Cystic fibrosis is caused by mutations in the CFTR gene (Cystic Fibrosis Transmembrane Conductance Regulator), which is responsible for the regulation of salt and water movement in and out of cells.

The cystic fibrosis market is driven by increasing prevalence of cystic fibrosis, advances in targeted therapies, and expanding focus on precision medicine. Cystic fibrosis is a genetic disease with significant prevalence in Western populations, especially in countries like the U.S. and Europe. According to the Cystic Fibrosis Foundation, there are over 30,000 people with cystic fibrosis in the U.S. alone. The steady increase in the number of diagnosed patients is a key driver for the growing demand for cystic fibrosis drugs, especially as the population ages and new treatments become available.

Moreover, the introduction of CFTR modulator therapies has dramatically transformed the treatment landscape for cystic fibrosis. Drugs have proven to significantly improve the quality of life and lung function for patients by targeting the underlying cause of cystic fibrosis mutations in the CFTR gene. These drugs have led to improved life expectancy and reduced hospital admissions, driving further market growth.

Precision medicine is becoming increasingly important in the cystic fibrosis market. Genetic testing and personalized therapies are on the rise, especially in understanding which mutations are causing cystic fibrosis in patients. This trend is likely to boost

demand for drugs tailored to specific mutations (e.g., CFTR modulators designed for different genotypes). This focus allows for highly personalized treatment plans, increasing the effectiveness of therapy. Also, greater awareness among healthcare providers and patients about cystic fibrosis, coupled with advancements in newborn screening programs, has led to earlier diagnosis and treatment. Early treatment can delay disease progression, reduce the severity of symptoms, and improve patient outcomes. The result is an expanded patient pool and a growing demand for cystic fibrosis therapies.

The introduction of novel drugs for cystic fibrosis has brought hope for patients but also raised concerns due to their high costs. These costs pose a significant barrier to accessibility, particularly in low- and middle-income countries and among uninsured or underinsured populations. The high price tag of cystic fibrosis drugs is a major challenge for both patients and healthcare systems.

Also, patients with cystic fibrosis require complex, multi-drug regimens, which can involve pulmonary therapies, enzyme replacement, and CFTR modulator therapies. Adherence to these treatments can be difficult, leading to non-compliance and treatment failures. Maintaining a consistent therapy routine can be physically, emotionally, and financially exhausting for patients. While CFTR modulators have brought significant improvements for many cystic fibrosis patients, they are not effective for all cystic fibrosis mutations. This means that certain patient populations still have limited treatment options, particularly those with rarer mutations that do not respond to available CFTR modulators. Drug development for these rare mutations remains a challenge.

Gene therapy is emerging as a groundbreaking treatment approach for cystic fibrosis, offering the potential to directly correct the genetic mutations that cause the disease. The development of CRISPR-based therapies and other gene-editing technologies is being explored to provide long-term or permanent solutions. Also, the orphan drug market for cystic fibrosis is growing, with regulatory agencies like the FDA and EMA granting fast-track approvals and market exclusivity to drugs targeting cystic fibrosis. This is because cystic fibrosis is classified as a rare disease, allowing drug manufacturers to enjoy financial incentives, including extended patent protection and tax credits. This has encouraged more companies to invest in cystic fibrosis drug development, leading to innovative treatments.

While cystic fibrosis is more common in Western populations, global awareness and diagnostic capabilities are increasing in emerging markets. As healthcare infrastructure

improves in regions like Asia-Pacific and Latin America, these regions represent significant growth opportunities for cystic fibrosis drug manufacturers. Increased access to genetic testing and CFTR modulator therapies could unlock a large, untapped market.

Moreover, numerous companies are developing drugs to address gaps in the cystic fibrosis treatment landscape, such as novel antibiotics for respiratory infections and drugs targeting rare cystic fibrosis mutations. These innovative solutions offer new growth prospects for both small biotech firms and large pharmaceutical companies. Investment in biologics and targeted therapies presents high revenue potential.

The competitive landscape of the cystic fibrosis market is shaped by strong competition from a few key pharmaceutical and biotechnology firms. These companies have the potential to disrupt the cystic fibrosis market, especially if gene therapies become a viable long-term solution.

## Contents

Executive Summary  
Scope and Definition  
Market/Product Definition  
Inclusion and Exclusion  
Key Questions Answered  
Analysis and Forecast Note

### **1. GLOBAL CYSTIC FIBROSIS MARKET: INDUSTRY OUTLOOK**

1.1 Market Overview  
1.2 Epidemiological Analysis  
1.3 Regulatory Landscape  
1.4 Key Trends  
1.5 Clinical Trial Analysis  
1.6 Market Dynamics  
    1.6.1 Impact Analysis  
    1.6.2 Market Drivers  
    1.6.3 Market Restraint  
    1.6.4 Market Opportunities

### **2. GLOBAL CYSTIC FIBROSIS MARKET, BY REGION, \$MILLION, 2023-2035**

2.1 North America  
    2.1.1 Market Dynamics  
    2.1.2 Market Size and Forecast  
        2.1.2.1 North America Cystic Fibrosis Market, by Country  
            2.1.2.1.1 U.S.  
2.2 Europe  
    2.2.1 Market Dynamics  
    2.2.2 Market Size and Forecast  
        2.2.2.1 Europe Cystic Fibrosis Market, by Country  
            2.2.2.1.1 Germany  
            2.2.2.1.2 U.K.  
            2.2.2.1.3 France  
            2.2.2.1.4 Italy  
            2.2.2.1.5 Spain  
2.3 Asia-Pacific

- 2.3.1 Market Dynamics
- 2.3.2 Market Size and Forecast
  - 2.3.2.1 Asia-Pacific Cystic Fibrosis Market, by Country
    - 2.3.2.1.1 Japan

### **3. GLOBAL CYSTIC FIBROSIS MARKET - COMPETITIVE LANDSCAPE AND COMPANY PROFILES**

- 3.1 Key Strategies and Developments by Company
  - 3.1.1 Funding Activities
  - 3.1.2 Mergers and Acquisitions
  - 3.1.3 Regulatory Approvals
  - 3.1.4 Partnerships, Collaborations, and Business Expansions
- 3.2 Company Profiles
  - 3.2.1 Vertex Pharmaceuticals Incorporated
    - 3.2.1.1 Overview
    - 3.2.1.2 Product Portfolio
    - 3.2.1.3 Target Customers
    - 3.2.1.4 Key Personnel
    - 3.2.1.5 Analyst View
  - 3.2.2 Gilead Sciences, Inc
    - 3.2.2.1 Overview
    - 3.2.2.2 Product Portfolio
    - 3.2.2.3 Target Customers
    - 3.2.2.4 Key Personnel
    - 3.2.2.5 Analyst View
  - 3.2.3 Novartis AG
    - 3.2.3.1 Overview
    - 3.2.3.2 Product Portfolio
    - 3.2.3.3 Target Customers
    - 3.2.3.4 Key Personnel
    - 3.2.3.5 Analyst View
  - 3.2.4 Sumitomo Pharma (Spirovant Sciences, Inc.)
    - 3.2.4.1 Overview
    - 3.2.4.2 Product Portfolio
    - 3.2.4.3 Target Customers
    - 3.2.4.4 Key Personnel
    - 3.2.4.5 Analyst View
  - 3.2.5 Boehringer Ingelheim International GmbH

- 3.2.5.1 Overview
- 3.2.5.2 Product Portfolio
- 3.2.5.3 Target Customers
- 3.2.5.4 Key Personnel
- 3.2.5.5 Analyst View
- 3.2.6 Krystal Biotech, Inc.
  - 3.2.6.1 Overview
  - 3.2.6.2 Product Portfolio
  - 3.2.6.3 Target Customers
  - 3.2.6.4 Key Personnel
  - 3.2.6.5 Analyst View
- 3.2.7 Others

#### **4. RESEARCH METHODOLOGY**

## List Of Figures

### LIST OF FIGURES

Figure: Cystic Fibrosis Market (by Scenario), \$Million, 2024, 2030, and 2035

Figure: Global Cystic Fibrosis Market, 2024 and 2035

Figure: Global Cystic Fibrosis Market Key Trends, Impact Analysis, 2023-2035

Figure: North America Cystic Fibrosis Market, \$Million, 2023-2035

Figure: Europe Cystic Fibrosis Market, \$Million, 2023-2035

Figure: Asia-Pacific Cystic Fibrosis Market, \$Million, 2023-2035

## List Of Tables

### LIST OF TABLES

Table: Market Snapshot

Table: Global Cystic Fibrosis Market, Market Dynamics, Impact Analysis

Table: Global Cystic Fibrosis Market (by Region), \$Million, 2023-2035

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