

# Mucopolysaccharidosis Type Vii - Market Access, Reimbursement Outlook, Competitive Landscape, Epidemiology and Forecast Report - 2015 To 2030

<https://marketpublishers.com/r/ME8827AF554EN.html>

Date: December 2018

Pages: 152

Price: US\$ 6,550.00 (Single User License)

ID: ME8827AF554EN

## Abstracts

Thelansis Knowledge Partners 'Mucopolysaccharidosis Type VII' - Market Access, Reimbursement Outlook, Competitive Landscapet, Epidemiology and Forecast Report – 2015 to 2030' report comprises of the Disease overview, classifications, stages or severity level, pathogenesis, identified and emerging biomarkers, pipeline assessment, competitive landscape, KOLs perspective, Market access - Country Specific Regulatory scenario s, Patent landscape, reimbursement scenario; Public, Private, Medicare, Medicaid, Government, Private, Co-payment, Out of pocket, Epidemiology; Incidence, Prevalence, Mortality, and current therapy market share, Order of entry, patient share, Market uptake, Peak patient share, Disease burden, Annual cost of therapy, market forecast for USA, Germany, France, Italy, Spain, UK, Japan and China 2015 to 2030

### Geographic Coverage

United States

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

Japan

China

Market estimation: 2015 – 2030

## Report brief:

### Epidemiology

This section provide the insights about historical and current patient pool (Incidence, Prevalence and mortality) and forecasted trend for 8 major markets (US, Germany, France, Italy, Spain, UK, Japan and China). The epidemiology research is one the key element of the report driven by meta-analysis, Systemic literature review and insights from KOL's

### Treatment Algorithm

The comprehensive details on country specific treatment and disease management for the patients in the US, Europe, Japan, and China. Future treatment paradigm and KOL's perspective (HCP's, Payers and Researcher) are the key strength of the report

### Competitive Landscapet

A thorough analysis of reported revenue, patient share and marketed trend for approved therapies however for pipeline assets we consider Phase I, Phase II and Phase III including their major milestones, study result, interim result, safety & efficacy data, key drivers, mechanism of action designation (Fast Track, Breakthrough Therapy, Accelerated Approval, Priority Review) geography level prioritization, asset level profile and probable launch date estimation

### Market Outlook & Market Forecast

Historic, current and forecasted market trend (High, Base case, Low) of the market by analyzing the impact of current therapies on the market, unmet needs, drivers and barriers and demand of better diagnosis of biomarkers. Key patient segment and market based on type, severity and stages of the disease. Disease burden, compliance rate, annual cost of therapy, reimbursement scenario, Patient share, Patient access

### Market uptake and benchmarking

The market penetration for pipeline assets being derived on the basis of benchmarking the sales of launched assets specifically targeting the patient segment or overall indication or therapy area or country or region specific. Order of entry benchmark works as a critical element for defining the uptake curve for the pipeline assets. Many drug

utilization study are being benchmarked to arrive at the current patient share for marketed therapies as well as off-label products. Market events are the major key factors which applied to the forecast model to optimize the year on year revenue estimation

#### Regulatory scenario

Country specific healthcare structure, regulatory bodies, HTA bodies, approval process for diagnostic and therapies, and work flow

#### Patent landscape

Country specific patent filings for approved therapies as well as pipeline assets, formulation patent, process patent (FDA, EMA, EPO, Orange book, JPO; Japan Patent Office) and fillings

#### Reimbursement Scenario

Country specific reimbursement Scenario; Public, Private, Medicare, Medicaid, Government, Private, Co-payment, Out of pocket

#### Key Report highlights

4 years of historical epidemiology data Epidemiology; Incidence, Prevalence, Mortality

11 years of epidemiology and market projections

Incidence/ Prevalence patient population

Mortality, survival and diagnosed patient pool

Treated patient pool

Treatment guidelines

Competitive landscape (Marketed therapies, Off-label therapies, Pipeline assessment, Phase – I, II, III)

Patient share

Country specific revenue share and trend

Market Opportunities assessment

Key market and product event analysis

Disease burden and annual cost of therapy

Regulatory scenario

Reimbursement Scenario

Patent landscape

HCP's/ KOL's perspective

Payer's perspective

Analyst commentary Ultragenyx Pharmaceutical Inc, Shire

## Contents

### **1. DISEASE – KEY FINDINGS SUMMARY**

1. Clinical findings
  1. Disease overview
  2. Therapeutic practices
  3. Future outlook
2. Commercial findings
  1. Disease market scenario 2020
  2. Disease market scenario 2025
  3. Disease market scenario 2030

### **2. INDICATION - DISEASE OVERVIEW**

1. Disease introduction
2. Classification
3. Genetic Subtypes and subtypes
4. Signs and Symptoms
5. Risk Groups
6. Histology
7. Etiology
8. Pathogenesis
9. Diagnosis
  1. Biomarker testing
10. Treatment Algorithm
  1. Treatment in US (guidelines and real-world management)
  2. Treatment in EU-5 (guidelines and real-world management)
  3. Treatment in Japan (guidelines and real-world management)
  4. Treatment in China (guidelines and real-world management)
11. Current Treatment and Management Goals for Disease
12. Referral Patterns
  1. Referral Scenario in US
  2. Referral Scenario in EU-5
  3. Referral Scenario in Japan
  4. Referral Scenario in China
13. Disease Prognosis
14. Disease Progression Pattern
15. Disease Biomarkers – Current and Future Paradigm

16. Disease Observing Scores
17. Healthcare burden
  1. Healthcare burden in US
  2. Healthcare burden in EU-5
  3. Healthcare burden in Japan
  4. Healthcare burden in China
18. Unmet Needs

### **3. EPIDEMIOLOGY**

1. Epidemiology Overview
2. Epidemiology by Geography
  1. Disease Incidence/ Prevalence in US
  2. Disease Incidence/ Prevalence in EU-5
  3. Disease Incidence/ Prevalence in Japan
  4. Disease Incidence/ Prevalence in China
3. Epidemiology by Type and subtype
4. Epidemiology by stages
5. Epidemiology Trends

### **4. MARKET OUTLOOK**

1. US - Disease Market Forecast
  1. Market Evolution (Historic)
  2. Market Progression (Futuristic)
  3. Market Trends and Expectations
    1. Worst case scenario
    2. Base Case Scenario
    3. Best Case Scenario
    4. Drivers and Barriers
2. UK - Disease Market Forecast
  1. Market Evolution (Historic)
  2. Market Progression (Futuristic)
  3. Market Trends and Expectations
    1. Worst case scenario
    2. Base Case Scenario
    3. Best Case Scenario
    4. Drivers and Barriers
3. France - Disease Market Forecast

1. Market Evolution (Historic)
2. Market Progression (Futuristic)
3. Market Trends and Expectations
  1. Worst case scenario
  2. Base Case Scenario
  3. Best Case Scenario
  4. Drivers and Barriers
4. Germany - Disease Market Forecast
  1. Market Evolution (Historic)
  2. Market Progression (Futuristic)
  3. Market Trends and Expectations
    1. Worst case scenario
    2. Base Case Scenario
    3. Best Case Scenario
    4. Drivers and Barriers
5. Italy - Disease Market Forecast
  1. Market Evolution (Historic)
  2. Market Progression (Futuristic)
  3. Market Trends and Expectations
    1. Worst case scenario
    2. Base Case Scenario
    3. Best Case Scenario
    4. Drivers and Barriers
6. Spain - Disease Market Forecast
  1. Market Evolution (Historic)
  2. Market Progression (Futuristic)
  3. Market Trends and Expectations
    1. Worst case scenario
    2. Base Case Scenario
    3. Best Case Scenario
    4. Drivers and Barriers
7. Japan - Disease Market Forecast
  1. Market Evolution (Historic)
  2. Market Progression (Futuristic)
  3. Market Trends and Expectations
    1. Worst case scenario
    2. Base Case Scenario
    3. Best Case Scenario
    4. Drivers and Barriers

8. China - Disease Market Forecast
  1. Market Evolution (Historic)
  2. Market Progression (Futuristic)
  3. Market Trends and Expectations
    1. Worst case scenario
    2. Base Case Scenario
    3. Best Case Scenario
    4. Drivers and Barriers
9. Key Expected Milestones Impacting Market

## **5. COMPETITIVE LANDSCAPE**

1. Pipeline Therapies Overview
  1. Phase III Therapies
    1. Assets - Company
      1. Current Status
      2. Trials
      3. Approval Timeline
      4. Expected Product Positioning
      5. Market Forecast (Till 2030)
      6. Attribute analysis - Phase III Therapies
    2. Phase II Therapies
      1. Assets - Company
        1. Current Status
        2. Trials
        3. Approval Timeline
        4. Expected Product Positioning
        5. Market Forecast (Till 2030)
        6. Attribute analysis - Phase III Therapies)
    2. Assets - Company
      1. Current Status
      2. Trials
      3. Approval Timeline
      4. Expected Product Positioning
      5. Market Forecast (Till 2030)
      6. Attribute analysis - Phase III Therapies
    3. Phase I Therapies
      1. Assets - Company
        1. Current Status



2. Trials
3. Approval Timeline
4. Expected Product Positioning
5. Market Forecast (Till 2030)
6. Attribute analysis
4. Pre-clinical Therapies
  1. Asset - Company
    1. Current Status
    2. Pre-clinical Trials
    3. Estimated Timelines
  2. Asset - Company
    1. Current Status
    2. Pre-clinical Trials
    3. Estimated Timelines
  3. Asset - Company
    1. Current Status
    2. Pre-clinical Trials
    3. Estimated Timelines
5. Inactive Therapies
  1. Asset - Company
    1. Current Status
    2. Reasons for Inactivation
  2. Asset - Company
    1. Current Status
    2. Reasons for Inactivation
  3. Asset - Company
    1. Current Status
    2. Reasons for Inactivation

## **6. REGULATORY FRAMEWORK SCENARIO**

1. Regulatory Framework in US
  1. Approval Framework(s)
  2. Clinical Trial Guidance
  3. Macro Policies (if any)
  4. Expectations on Future Pricing and Reimbursement Outlook
2. Regulatory Framework in UK
  1. Approval Framework(s)
  2. Clinical Trial Guidance

3. Macro Policies (if any)
4. Expectations on Future Pricing and Reimbursement Outlook
3. Regulatory Framework in France
  1. Approval Framework(s)
  2. Clinical Trial Guidance
  3. Macro Policies (if any)
  4. Expectations on Future Pricing and Reimbursement Outlook
4. Regulatory Framework in Germany
  1. Approval Framework(s)
  2. Clinical Trial Guidance
  3. Macro Policies (if any)
  4. Expectations on Future Pricing and Reimbursement Outlook
5. Regulatory Framework in Italy
  1. Approval Framework(s)
  2. Clinical Trial Guidance
  3. Macro Policies (if any)
  4. Expectations on Future Pricing and Reimbursement Outlook
6. Regulatory Framework in Spain
  1. Approval Framework(s)
  2. Clinical Trial Guidance
  3. Macro Policies (if any)
  4. Expectations on Future Pricing and Reimbursement Outlook
7. Regulatory Framework in Japan
  1. Approval Framework(s)
  2. Clinical Trial Guidance
  3. Macro Policies (if any)
  4. Expectations on Future Pricing and Reimbursement Outlook
8. Regulatory Framework in China
  1. Approval Framework(s)
  2. Clinical Trial Guidance
  3. Macro Policies (if any)
  4. Expectations on Future Pricing and Reimbursement Outlook

## **7. CLINICAL TRIAL ASSESSMENT – CURRENT AND FUTURE PARADIGM**

1. Distribution of Primary Endpoints across trials
2. Distribution of Secondary Endpoints across trials
3. Evolution and acceptance of surrogate endpoints
4. Key Investigator initiated trials

## 5. Attrition analysis

1. Failed Trials, Reasons and Business Impact
  2. Terminated Trials, Reasons and Business Impact
  3. Withdrawn Trials, Reasons and Business Impact
- ## 6. Trial enrollment scenario and challenges
- ## 7. Clinical Trial Guidance (across geographies)

## 8. MARKET ACCESS

1. Market access-payers perspective,
2. Country Specific Regulatory scenarios,
3. Patent landscape,
4. reimbursement scenario; Public, Private,
5. Medicare, Medicaid,
6. Government, Private, Co-payment, Out of pocket

## 9. THELANSIS' COMMENTARY

1. Key Unmet needs in Indication-Disease
2. Possible Best-case Clinical Trial Strategies
3. Possible Best-case Market positioning strategies
4. Possible Best-case Market Access Strategies
5. Overall View on Indication-Disease Market in Dollar Value

## 10. REPORT METHODOLOGY

1. Secondary research
2. Primary research
3. Data collation
4. Insight Generation

## 11. ABOUT THELANSIS

1. Our Capabilities
2. Our Services
3. Our Contacts
4. Disclaimer

## I would like to order

Product name: Mucopolysaccharidosis Type Vii - Market Access, Reimbursement Outlook, Competitive Landscape, Epidemiology and Forecast Report - 2015 To 2030

Product link: <https://marketpublishers.com/r/ME8827AF554EN.html>

Price: US\$ 6,550.00 (Single User License / Electronic Delivery)

If you want to order Corporate License or Hard Copy, please, contact our Customer Service:

[info@marketpublishers.com](mailto:info@marketpublishers.com)

## Payment

To pay by Credit Card (Visa, MasterCard, American Express, PayPal), please, click button on product page <https://marketpublishers.com/r/ME8827AF554EN.html>

To pay by Wire Transfer, please, fill in your contact details in the form below:

First name:  
Last name:  
Email:  
Company:  
Address:  
City:  
Zip code:  
Country:  
Tel:  
Fax:  
Your message:

**\*\*All fields are required**

Customer signature \_\_\_\_\_

Please, note that by ordering from marketpublishers.com you are agreeing to our Terms & Conditions at <https://marketpublishers.com/docs/terms.html>

To place an order via fax simply print this form, fill in the information below and fax the completed form to +44 20 7900 3970

