

# **Pompe Disease - Market Access, Reimbursement Outlook, Competitive Landscape, Epidemiology and Forecast Report - 2015 To 2030**

<https://marketpublishers.com/r/P6E7E33D7F6EN.html>

Date: March 2018

Pages: 203

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

ID: P6E7E33D7F6EN

## **Abstracts**

Thelansis Knowledge Partners 'Pompe Disease' - 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 scenarios, 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 Valerion Therapeutics, Llc, Amicus Therapeutics, Actus Therapeutics, Inc., Genzyme, A Sanofi Company, Amicus Therapeutics, Lacerta Therapeutics, Inc

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