

Pompe Disease R&D Pipeline Analysis Report, Q4 2020

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Abstracts

Pompe Disease Pipeline Overview

The Q4 Pompe Disease pipeline report is part of the VPA Metabolic Disorder disease pipeline service. This quarterly review describes the pipeline of drugs under development for Pompe Disease, provides an update on the mechanism of action and targets, licensing and collaboration opportunities, company profiles including their business information, and market developments.

Pompe Disease Types, Symptoms, Causes, and Available Treatment Options The comprehensive report on the indication presents Pompe Disease disease overview, Pompe Disease types, Pompe Disease symptoms, causes, and FDA/EMA approved treatment options.

Pompe Disease Pipeline Trends and Insights

The year 2020 witnessed progress in innovations and the number of pipeline agents researched for Pompe Disease indication. The report presents near-term and long-term pipeline development trends and potential insights.

Pompe Disease Company Trends and Insights

The report analyzes the development progress, current status, investments, partnerships, and other developments of 18 companies. Business profiles and contact details of the companies actively perusing Pompe Disease pipeline are assessed.

Pompe Disease R&D Pipeline Development Phase Trends and Insights From the discovery stage of development to phase 3 development, all the potential pipeline drug candidates are analyzed in the report. Pompe Disease discovery stage,



research stage, Phase 1, phase 2, Phase 3, IND enabling studies are provided in the research.

Small Pompe Disease companies look for licensing and collaboration partners
High R&D costs and long duration pose challenges for small companies operating in the
industry. Further, the presence of large companies and stringent regulations act as
barriers for Pompe Disease pipeline development companies. Accordingly, a significant
portion of small companies presents potential licensing and collaboration opportunities.

Pompe Disease Pipeline Market News and Developments during 2020 The Pompe Disease industry news, pipeline research, market developments including advancement in phases, pre-clinical study results, clinical trial launches, acquisitions and mergers, regulatory approvals, and others are included in the report.

Pompe Disease Mechanism of Action and Targets

Leading Mechanism of Action and targets being researched by all the companies and their progress is included. New mechanisms of actions introduced during the year, route of administration, orphan drug designation, and others are provided in the study.

New molecular entity details

Drug candidates identified as new molecular entities by FDA, EMA, and other regulators are included in the research.

Sources and Research Methodology

Data is updated daily across diseases in the infectious pipeline service. We review our quarterly pipeline reports for the latest 5 years to present longitudinal insights.

Scope and Coverage

Pompe Disease pipeline development activities across the early phase, midstage, and late phase pipelines including therapeutic candidates

Drug profiles comprising of drug overview, mechanism of action, co-developers, originators, route of administration, molecule type, orphan drug status, new molecular entity, area, and other details.

Company-company partnerships, company-institute partnerships, and investment details of companies are included



18 companies are included including Abeona Therapeutics Inc, Actus
Therapeutics Inc, Amicus Therapeutics Inc, Audentes Therapeutics Inc, Avidity
Biosciences LLC, Avrobio Inc, EpiVax Inc, Etubics Corp, Genzyme Corp,
greenovation Biotech GmbH, JCR Pharmaceuticals Co Ltd, NanoMedSyn SAS,
Oxyrane Belgium NV, Pharming Group NV, RespireRx Pharmaceuticals Inc,
Sarepta Therapeutics Inc, Spark Therapeutics Inc, Valerion Therapeutics LLC,

Disease overview, Pipeline trends, market analysis, and other developments

Potential licensing/new business opportunities in Pompe Disease pipeline market

Highlights

Global coverage of companies and pipeline agents

2020 Trends, market analysis, and developments

Potential growth opportunities

Comprehensive details of drug candidates

Reasons to Buy

Drive pipeline research and commercial assessment

Assess most promising drug candidates and stay ahead of the competition

Strengthen your pipeline through identifying business expansion and acquisition opportunities

Develop new candidates based on most focused targets and mechanism of actions



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Amicus Therapeutics Inc

Audentes Therapeutics Inc

Avidity Biosciences LLC

Avrobio Inc

EpiVax Inc

Etubics Corp

Genzyme Corp

greenovation Biotech GmbH

JCR Pharmaceuticals Co Ltd

NanoMedSyn SAS

Oxyrane Belgium NV



Pharming Group NV
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