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Pompe Disease – Pipeline Insight, 2020

https://marketpublishers.com/r/P3EB8A0EB51EN.html
Date: September 2020
Pages: 90
Price: US$ 2,500.00 (Single User License)
ID: P3EB8A0EB51EN

Abstracts

This report can be delivered to the clients within 72 - 96 Hours

DelveInsight's, "Pompe Disease – Pipeline Insight, 2020," report provides comprehensive insights about 30+ companies and 30+ pipeline drugs in Pompe Disease pipeline landscape. It covers the pipeline drug profiles, including clinical and nonclinical stage products. It also covers the therapeutics assessment by product type, stage, route of administration, and molecule type. It further highlights the inactive pipeline products in this space.

Geography Covered

Global coverage

Pompe Disease Understanding

Pompe Disease: Overview

Pompe disease is a rare disease continuum with variable rates of disease progression and different ages of onset. First symptoms can occur at any age from birth to late adulthood. Earlier onset compared to later onset is usually associated with faster progression and greater disease severity. At all ages, skeletal muscle weakness characterizes the disease causing mobility problems and affecting the respiratory system.The most severely affected infants usually present within the first 3 months after birth. They have characteristic heart (cardiac) problems (dysfunction due to heart enlargement) in addition to generalized skeletal muscle weakness and a life expectancy of less than 2 years, if untreated (classic infantile Pompe disease). Less severe forms of Pompe disease with onset during childhood, adolescence, or adulthood, rarely manifest

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cardiac problems, but gradually lead to walking disability and reduced respiratory function. It is a rare, multisystemic, hereditary disease, which is caused by ‘pathogenic variations’ (abnormalities / mutations) in the ‘GAA gene’.

Symptoms

Patients with the ‘classic infantile’ form of Pompe disease are the most severely affected. Although hardly any symptoms may be apparent at birth, the disease usually presents within the first three months of life with rapidly progressive muscle weakness (‘floppy infants’), diminished muscle tone (hypotonia), respiratory deficiency, and a type of heart disease known as hypertrophic cardiomyopathy, a condition characterized by abnormal thickening of the walls of the heart (mainly the left chamber and the wall between the left and right chamber) resulting in diminished cardiac function. These problems together culminate in cardio-respiratory failure within the first 2 years of life.

Many infants have a large, protruding, tongue and a moderate enlargement of the liver. The legs often rest in a frog position and feel firm on palpation (pseudo-hypertrophy).

Feeding and swallowing problems as well as respiratory difficulties, which are often combined with respiratory tract infections, are common. Major developmental milestones such as rolling over, sitting up, and standing are delayed or not achieved. Mental development is usually normal. Virtually all infants experience hearing loss. The ‘classic infantile’ form of Pompe disease is caused by a total absence of acid alpha-glucosidase (GAA) activity and by a rapid buildup of glycogen in skeletal muscle and heart.

Diagnosis

The diagnosis of Pompe disease is based on a thorough clinical evaluation, a detailed patient and family history, and a variety of biochemical tests with first of all the measuring of GAA activity. In individuals suspected of having Pompe disease, blood can be drawn and the function/activity of GAA (the ‘enzymatic activity’) can be measured in white blood cells (leukocytes), but only if the proper assay conditions are being used and acarbose is added to the reaction mixture to inhibit the activity of glucoamylase. Alternatively, the GAA activity/functional assay can also be performed on dried blood spots, but this method is not any quicker, less reliable, and also requires the use of acarbose to inhibit the glucoamylase activity. A variety of other tests can be performed to detect or assess symptoms potentially associated with Pompe disease such as sleep studies, tests that measure lung function, and tests that measure muscle

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function. Muscle MRI (imaging by magnetic resonance) is used to visualize the degree of muscle damage.

Treatment

Enzyme replacement therapy (ERT) is an approved treatment for all patients with Pompe disease. It involves the intravenous administration of recombinant human acid alpha-glucosidase (rhGAA). This treatment is called Lumizyme (marketed as Myozyme outside the United States), and was first approved by the U.S. Food and Drug Administration (FDA) in 2006. Currently in 2020, it is broadly agreed that ERT extends the life expectancy of patients with classic-infantile Pompe disease / IOPD with years (the longest treated patient is at present 24 years old). Additional treatment of Pompe disease is symptomatic and supportive. Respiratory support may be required, as most patients have some degree of respiratory compromise and/or respiratory failure. Physical therapy may be helpful to strengthen respiratory muscles. Some patients may need respiratory assistance through mechanical ventilation (i.e. Bipap or volume ventilators) during the night and/or periods of the day or during respiratory tract infections. Mechanical ventilation support can be through noninvasive or invasive techniques.

Pompe Disease Emerging Drugs Chapters

This segment of the Pompe Disease report encloses its detailed analysis of various drugs in different stages of clinical development, including phase II, I, preclinical and Discovery. It also helps to understand clinical trial details, expressive pharmacological action, agreements and collaborations, and the latest news and press releases.

Pompe Disease Emerging Drugs

Cipaglucosidase alfa: Amicus Therapeutics

Amicus Therapeutics are leveraging our investigational biologics and Chaperone-Advanced Replacement Therapy (CHART) to develop AT-GAA, an investigational therapy that consists of recombinant human acid alpha-glucosidase (rhGAA) enzyme with an optimized carbohydrate structure (designated ATB200), administered with a small molecule pharmacological chaperone (designated AT2221).

SPK-3006: Spark Therapeutics

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Spark Therapeutics is developing SPK-3006, an investigational gene therapy for the potential treatment of Pompe disease. Pompe disease is an oftentimes fatal lysosomal storage disorder and neuromuscular disease, with systemic, multi-organ manifestations resulting from loss of function mutations in the gene encoding acid alpha-glucosidase (GAA). The initial construct for SPK-3006 was in-licensed from Genethon in 2017, and Spark retains global commercialization rights. It is currently in phase I/II stage of development.

Recombinant Adeno-Associated Virus Acid Alpha-Glucosidase: Lacerta Therapeutics

Recombinant Adeno-Associated Virus Acid Alpha-Glucosidase is being developed by Lacerta Therapeutics, Inc. it is currently in phase I stage of development.

Further product details are provided in the report……..

Pompe Disease: Therapeutic Assessment

This segment of the report provides insights about the different Pompe Disease drugs segregated based on following parameters that define the scope of the report, such as:

Major Players in Pompe Disease

There are approx. 30+ key companies which are developing the therapies for Pompe Disease. The companies which have their Pompe Disease drug candidates in the most advanced stage, i.e. phase III include Amicus Therapeutics and others

Phases

DelveInsight’s report covers around 30+ products under different phases of clinical development like

Late-stage products (Phase II and Phase II/III)

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Mid-stage products (Phase II and Phase II/III)

Early-stage products (Phase I/II and Phase I) along with the details of

Pre-clinical and Discovery stage candidates

Discontinued & Inactive candidates

Route of Administration

Pompe Disease pipeline report provides the therapeutic assessment of the pipeline drugs by the Route of Administration. Products have been categorized under various ROAs such as

Infusion

Intradermal

Intramuscular

Intranasal

Intravenous

Oral

Parenteral

Subcutaneous

Topical

Molecule Type

Products have been categorized under various Molecule types such as

Gene therapies

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Small molecule

Vaccines

Polymers

Peptides

Monoclonal antibodies

Product Type

Drugs have been categorized under various product types like Mono, Combination and Mono/Combination.

Pompe Disease: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase II, I, preclinical and discovery stage. It also analyses Pompe Disease therapeutic drugs key players involved in developing key drugs.

Pipeline Development Activities

The report covers the detailed information of collaborations, acquisition and merger, licensing along with a thorough therapeutic assessment of emerging Pompe Disease drugs.

Report Highlights

The companies and academics are working to assess challenges and seek opportunities that could influence Pompe Disease R&D. The therapies under development are focused on novel approaches to treat/improve Pompe Disease.

In February 2019, the U.S. Food and Drug Administration (FDA) granted Amicus a Breakthrough Therapy Designation to ATB200/AT2221 for the treatment of late onset Pompe disease.

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In November 2019 ATB200/AT2221 was granted a PIM by the MHRA. Orphan designations have been granted by the European Commission for both ATB200 and AT2221 in the treatment of glycogen storage disease type II (Pompe disease).

In September 2017, ATB200/AT2221was granted US orphan drug status for the treatment of Pompe disease.

Pompe Disease Report Insights

Pompe Disease Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Pompe Disease Report Assessment

Pipeline Product Profiles

Therapeutic Assessment

Pipeline Assessment

Inactive drugs assessment

Unmet Needs

Key Questions

Current Treatment Scenario and Emerging Therapies:

How many companies are developing Pompe Disease drugs?

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How many Pompe Disease drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Pompe Disease?

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Pompe Disease therapeutics?

What are the recent trends, drug types and novel technologies developed to overcome the limitation of existing therapies?

What are the clinical studies going on for Pompe Disease and their status?

What are the key designations that have been granted to the emerging drugs?

Key Players

Immusoft

Asklepios Biopharmaceutical

Audentes Therapeutics

Genzyme

Lacerta Therapeutics

Selecta Biosciences

Greenovation Biotech

BioMarin Pharmaceutical

Oxyrane

Key Products

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Cipaglucosidase alfa

SPK-3006

Recombinant Adeno-Associated Virus Acid Alpha-Glucosidase

Reveglucosidase alfa

Research programme: lysosomal storage disease therapeutics

Research programme: alpha-glucosidase gene therapy

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