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Hunter Syndrome - Pipeline Insight, 2021

https://marketpublishers.com/r/H5640A72C39DEN.html
Date: July 2021
Pages: 60
Price: US$ 1,500.00 (Single User License)
ID: H5640A72C39DEN

Abstracts

This report can be delivered to the clients within 48 Hours

DelveInsight's, "Hunter Syndrome – Pipeline Insight, 2021," report provides comprehensive insights about 10+ companies and 10+ pipeline drugs in Hunter Syndrome 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

Hunter Syndrome Understanding

Hunter Syndrome: Overview

Hunter syndrome is a mucopolysaccharide disease caused by the enzymatic deficiency of iduronate-2-sulfatase (I2S). This is also called as mucopolysaccharoidosis Type II. Hunter syndrome is a hereditary disease in which the breakdown of a mucopolysaccharide (a chemical that is widely distributed in the body outside of cells) is defective. This chemical builds up and causes a characteristic facial appearance, abnormal function of multiple organs, and in severe cases, early death. In the neuronopathic form of this disorder, physical and mental development reaches a peak at 2-4 years of age with subsequent deterioration. Recurrent upper respiratory infections, a chronic runny nose, hearing impairment, liver and spleen enlargement, inguinal and abdominal hernias, joint stiffness and multiplex dysplasia, compression of

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tendons in the wrist (carpal tunnel syndrome), and joint stiffness which can result in reduction of hand function, growth failure and valvular disease commonly occur with this form of MPS II. Manifestations of MPS II may include not inflammatory joint stiffness, with associated restriction of movements; and coarsening of facial features, including thickening of the lips, tongue (macroglossia), and nostrils.

Patients with MPSII disease accumulate heparin and dermatan sulfates in the urines. Molecular genetic testing for mutations in the IDS gene is available to confirm the diagnosis. It is always important to rule out a multiple sulfatase deficiency by testing other sulfatase enzymes. Treatment for Hunter syndrome depends on the symptoms. An enzyme replacement therapy, idursulfase (Elaprase), was approved in 2006 by the U.S. Food and Drug Administration (FDA) as a treatment for MPS II.

'Hunter Syndrome - Pipeline Insight, 2021' report by DelveInsight outlays comprehensive insights of present scenario and growth prospects across the indication. A detailed picture of the Hunter Syndrome pipeline landscape is provided which includes the disease overview and Hunter Syndrome treatment guidelines. The assessment part of the report embraces, in depth Hunter Syndrome commercial assessment and clinical assessment of the pipeline products under development. In the report, detailed description of the drug is given which includes mechanism of action of the drug, clinical studies, NDA approvals (if any), and product development activities comprising the technology, Hunter Syndrome collaborations, licensing, mergers and acquisition, funding, designations and other product related details.

Report Highlights

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

Hunter Syndrome Emerging Drugs Chapters

This segment of the Hunter Syndrome 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.

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Hunter Syndrome Emerging Drugs

DNL310: Denali Therapeutics Inc

DNL310, or ETV:IDS, is a recombinant form of the iduronate 2-sulfatase (“IDS”) enzyme engineered to cross the blood-brain barrier using Denali’s proprietary ETV technology. DNL310 is intravenously administered and intended to improve overall clinical manifestations of Hunter Syndrome, including neurological symptoms, which are not adequately addressed by currently approved therapies. The FDA has granted orphan drug status and a rare pediatric disease designation to Denali Therapeutics pipeline candidate, DNL310. The drug is being evaluated in Phase I/II clinical trial to treat Hunter Syndrome.

RGX-121: Regenxbio Inc.

RGX-121 is a product candidate for the treatment of Mucopolysaccharidosis Type II (MPS II), also known as Hunter Syndrome. RGX-121 is designed to use the AAV9 vector to deliver the human iduronate-2-sulfatase gene (IDS) which encodes the iduronate-2-sulfatase (I2S) enzyme to the central nervous system (CNS). Delivery of the IDS gene within cells in the CNS could provide a permanent source of secreted I2S beyond the blood-brain barrier, allowing for long-term cross correction of cells throughout the CNS. RGX-121 has received orphan drug product, rare pediatric disease and Fast Track designations from the U.S. Food and Drug Administration.

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

Hunter Syndrome: Therapeutic Assessment

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

Major Players in Hunter Syndrome

There are approx. 10+ key companies which are developing the therapies for Hunter Syndrome. The companies which have their Hunter Syndrome drug candidates in the most advanced stage, i.e. Phase I/II include, Denali Therapeutics.

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Phases

DelveInsight's report covers around 10+ products under different phases of clinical development like

Late stage products (Phase III)

Mid-stage products (Phase II)

Early-stage product (Phase I) along with the details of

Pre-clinical and Discovery stage candidates

Discontinued & Inactive candidates

Route of Administration

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

Oral

Parenteral

Intravenous

Subcutaneous

Topical

Molecule Type

Products have been categorized under various Molecule types such as

Monoclonal Antibody

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- Peptides
- Polymer
- Small molecule
- Gene therapy
- Product Type

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

Hunter Syndrome: Pipeline Development Activities

The report provides insights into different therapeutic candidates in phase II, I, preclinical and discovery stage. It also analyses Hunter Syndrome 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 Hunter Syndrome drugs.

Hunter Syndrome Report Insights

- Hunter Syndrome Pipeline Analysis
- Therapeutic Assessment
- Unmet Needs
- Impact of Drugs

Hunter Syndrome Report Assessment

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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 Hunter Syndrome drugs?

How many Hunter Syndrome drugs are developed by each company?

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

What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Hunter Syndrome 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 Hunter Syndrome and their status?

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

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