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Angelman Syndrome – Pipeline Insight, 2020

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Abstracts

This report can be delivered to the clients within 72 Hours

DelveInsight's, "Angelman Syndrome – Pipeline Insight, 2020," report provides comprehensive insights about 5+ companies and 5+ pipeline drugs in Angelman 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

Angelman Syndrome Understanding

Angelman Syndrome: Overview

Angelman syndrome is a rare genetic and neurological disorder characterized by severe developmental delay and learning disabilities; absence or near absence of speech; inability to coordinate voluntary movements (ataxia); tremulousness with jerky movements of the arms and legs and a distinct behavioral pattern characterized by a happy disposition and unprovoked episodes of laughter and smiling. Although those with the syndrome may be unable to speak, many gradually learn to communicate through other means such as gesturing. In addition, children may have enough receptive language ability to understand simple forms of language communication.

Symptoms

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Children with Angelman syndrome experience delays in reaching developmental milestones (developmental delays) and have severe learning disabilities. Children with Angelman syndrome also have significant communication difficulties. Most children do not develop the ability to speak more than a few words. Children usually can understand simple commands. Older children and adults may be able to communicate through gesturing and or using communication boards.

Diagnosis

A diagnosis of Angelman syndrome may be made based upon a detailed patient history, a thorough clinical evaluation and identification of characteristic findings. About 80% of cases can be confirmed through a variety of specialized blood tests such as DNA methylation (detects, but does not discriminate between chromosome deletion, imprinting center defect and paternal uniparental disomy).

Fluorescent in situ hybridization (FISH) or, most commonly, microarray chromosome analysis can detect the characteristic deletion (seen in 70% of cases) of chromosome 15q11-q13 in cells of the body.

Treatment

At this time, therapies for Angelman syndrome are symptomatic and supportive. Anti-seizure medications (anticonvulsants) are helpful to those experiencing seizures. Ankle braces/supports and physical therapy can help in achievement of walking. Scoliosis can develop in about 10% and may require braces or surgical correction.

Angelman Syndrome Emerging Drugs Chapters

This segment of the Angelman 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.

Angelman Syndrome Emerging Drugs

GTX-102: GeneTx Biotherapeutics / Ultragenyx Pharmaceutical Inc.

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GTX-102 is an investigational antisense oligonucleotide designed to target and inhibit expression of UBE3A-AS. Nonclinical studies show that GTX-102 reduces the levels of UBE3A-AS and reactivates expression of the paternal UBE3A allele in neurons of the CNS. Reactivation of paternal UBE3A expression in animal models of Angelman syndrome has been associated with improvements in some of the neurological symptoms associated with the condition.

The Phase 1/2 KIK-AS (Knockdown of UBE3A-antisense in Kids with Angelman Syndrome) study is an ongoing open-label, multiple-dose, dose-escalating study will enroll 20 patients to evaluate the safety, tolerability, and potential efficacy of GTX-102 in pediatric patients with Angelman syndrome. Preliminary data from the first cohorts of the study are expected in the first half of 2021.

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

Angelman Syndrome: Therapeutic Assessment

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

Major Players in Angelman Syndrome

There are approx. 5+ key companies which are developing the therapies for Angelman Syndrome. The companies which have their Angelman Syndrome drug candidates in the mid to advanced stage, i.e. phase III and Phase I/II include, Ovid Therapeutics Inc., GeneTX Biotherapeutics, LLC etc.

Phases

DelveInsight’s report covers around 5+ products under different phases of clinical development like

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

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

Pre-clinical and Discovery stage candidates

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Discontinued & Inactive candidates

Route of Administration

Angelman 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

Intrathecal

Molecule Type

Products have been categorized under various Molecule types such as

Small molecules

Gene therapies

Product Type

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

Angelman Syndrome: Pipeline Development Activities

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

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Report Highlights

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

GTX-102 has been granted Orphan Drug Designation and Rare Pediatric Disease Designation from the U.S. Food and Drug Administration (FDA). In August 2019, GeneTx and Ultragenyx announced a partnership to develop GTX-102, with Ultragenyx receiving an exclusive option to acquire GeneTx.

Angelman Syndrome Report Insights

Angelman Syndrome Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Angelman Syndrome Report Assessment

Pipeline Product Profiles

Therapeutic Assessment

Pipeline Assessment

Inactive drugs assessment

Unmet Needs

Key Questions

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Current Treatment Scenario and Emerging Therapies:

How many companies are developing Angelman Syndrome drugs?

How many Angelman Syndrome drugs are developed by each company?

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

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

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

Key Players

GeneTX Biotherapeutics, LLC

Ultragenyx Pharmaceutical Inc

Hoffmann-La Roche

Ovid Therapeutics Inc.

Biogen

Key Products

GTX-102

RO7248824

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OV101

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