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Dravet Syndrome - Pipeline Insight, 2022

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Abstracts

This report can be delivered to the clients within 48-72 hours

DelveInsight's, "Dravet Syndrome - Pipeline Insight, 2022," report provides comprehensive insights about 15+ companies and 15+ pipeline drugs in Dravet 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

Dravet Syndrome Understanding

Dravet Syndrome: Overview

Dravet Syndrome is a severe and progressive genetic epilepsy characterized by frequent, prolonged and refractory seizures that usually begin within the first year of life. Dravet Syndrome is classified as a developmental and epileptic encephalopathy due to the developmental delays and cognitive impairment, in addition to seizure activity, that stem from the genetic mutation that causes the disease. Approximately 85% of those diagnosed with Dravet Syndrome have a mutation of the SCN1A gene.1 Dravet Syndrome is not usually caused by an inherited mutation. In 90% of these patients, the mutation is not found in the patient's parents Dravet Syndrome can be diagnosed, if a patient exhibits specific symptoms, genetic testing may be done via an epilepsy panel, to look for SCN1A and other genes commonly associated with epilepsy. Dravet

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syndrome is characterized by frequent, prolonged and refractory seizures that usually begin within the first year of life. However, the effects of Dravet syndrome are not limited to seizures, developmental delays, movement and balance issues and also language and speech disturbances. Dravet syndrome has a high rate of premature death due to the severity of this type of epilepsy. Up to 20% of children and adolescents living with Dravet die before adulthood.

' Dravet Syndrome - Pipeline Insight, 2022' report by DelveInsight outlays comprehensive insights of present scenario and growth prospects across the indication. A detailed picture of the Dravet Syndrome pipeline landscape is provided which includes the disease overview and Dravet Syndrome treatment guidelines. The assessment part of the report embraces, in depth Dravet 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, Dravet 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 Dravet Syndrome R&D. The therapies under development are focused on novel approaches to treat/improve Dravet Syndrome.

Dravet Syndrome Emerging Drugs Chapters

This segment of the Dravet 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.

Dravet Syndrome Emerging Drugs

Soticlestat : Takeda

Soticlestat is a potent, highly selective, oral, first-in-class inhibitor of the enzyme

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cholesterol 24-hydroxylase (CH24H)), with the potential to reduce seizure susceptibility and improve seizure control. CH24H is predominantly expressed in the brain, where it converts cholesterol into 24S-hydroxycholesterol (24HC) to adjust the homeostatic balance of brain cholesterol. 24HC is a positive allosteric modulator of the NMDA receptor and modulates glutamatergic signaling associated with epilepsy. Glutamate is one of the main neurotransmitters in the brain and has been shown to play a role in the initiation and spread of seizure activity. Recent literature indicates that CH24H is involved in over-activation of the glutamatergic pathway through modulation of the NMDA channel and that increased expression of CH24H can disrupt the reuptake of glutamate by astrocytes, resulting in epileptogenesis and neurotoxicity. Inhibition of CH24H by soticlestat reduces the neuronal levels of 24HC and may improve excitatory/inhibitory balance of NMDA channel activity.. It is being investigated by Ovid and Takeda for the treatment of rare developmental and epileptic encephalopathies (DEEs), a group of highly refractory epilepsy syndromes including Dravet Syndrome and LGS. Soticlestat is in Phase 3 stage of development for the treatment of Dravet Syndrome.

Lorcaserin : Eisai Inc.

Lorcaserin is a selective serotonin 5-HT2c receptor agonist. Stimulation of 5-HT2c receptors with lorcaserin may increase GABA-mediated inhibition and result in reduced seizure activity in Dravet syndrome patients. It is in Phase 3 stage of development for treatment of Dravet Syndrome.

EPX100 : Epygenix Therapeutics

EPX-100 is a first-generation antihistamine safely used from the 1950-1970s. EPX-100 was found to be a powerful suppressor of spontaneous convulsive behavior and electrographic seizures in zebrafish disease models for Dravet Syndrome. EPX-100’s antiepileptic action is not through a histaminergic mechanism of action but acts via modulation of serotonin (5HT) signaling pathways. It is in Phase 2 stage of development for the treatment of Dravet Syndrome.

STK-001 : Stoke Therapeutics

STK-001 is an investigational new medicine for the treatment of Dravet syndrome

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currently being evaluated in ongoing clinical trials. Stoke believes that STK-001, a proprietary antisense oligonucleotide (ASO), has the potential to be the first disease-modifying therapy to address the genetic cause of Dravet syndrome. STK-001 is designed to upregulate NaV1.1 protein expression by leveraging the non-mutant (wild-type) copy of the SCN1A gene to restore physiological NaV1.1 levels, thereby reducing both occurrence of seizures and significant non-seizure comorbidities. STK-001 has been granted orphan drug designation by the FDA as a potential new treatment for Dravet syndrome. It is in phase 2 stage of development for the treatment of Dravet Syndrome.

VRP324 : Virpax Pharmaceuticals

VRP324 Intranasal pharmaceutical-grade Schedule V cannabidiol (CBD). Highly purified pharmaceutical-grade cannabidiol (CBD), approved in the United States, has demonstrated efficacy with an acceptable safety profile in patients with Lennox-Gastaut or Dravet syndrome. CBD acts on cannabinoid (CB) receptors of the endocannabinoid system, which are found in numerous body areas, including the peripheral system and the central nervous systems, including the brain. The endocannabinoid system regulates many physiological responses of the body and neuronal excitability responses relevant to the pathophysiology of many disease types, including epilepsy. It is in Pre-Clinical stage of development for the treatment of Dravet Syndrome.

AntagoNAT : OPKO Health

AntagoNATs are oligonucleotide compounds that target non-coding natural antisense transcripts leading to an upregulation of a desired functional protein. CAMP4 has prioritized OPKO’s lead AntagoNAT compound to progress into clinical trials for the treatment of Dravet syndrome. It is in Pre-Clinical stage of development for the treatment of Dravet Syndrome.

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

Dravet Syndrome: Therapeutic Assessment

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

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Major Players in Dravet Syndrome

There are approx. 15+ key companies which are developing the therapies for Dravet Syndrome. The companies which have their Dravet Syndrome drug candidates in the most advanced stage, i.e. phase III include, Takeda.

Phases

DelveInsight's report covers around 15+ 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

Dravet 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

Intra-articular

Intraocular

Intrathecal

Intravenous

Ophthalmic

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Oral

Parenteral

Subcutaneous

Topical

Transdermal

Molecule Type

Products have been categorized under various Molecule types such as

Oligonucleotide

Peptide

Small molecule

Product Type

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

Dravet Syndrome : Pipeline Development Activities

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

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Dravet Syndrome Report Insights

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

Dravet Syndrome 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 Dravet Syndrome drugs?
- How many Dravet Syndrome drugs are developed by each company?
- How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Dravet Syndrome?
- What are the key collaborations (Industry–Industry, Industry–Academia), Mergers and acquisitions, licensing activities related to the Dravet Syndrome therapeutics?

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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 Dravet Syndrome and their status?

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

Key Players

Stoke Therapeutics

Epygenix Therapeutics

Takeda

Eisai Inc.

Virpax Pharmaceuticals

OPKO Health

GW Pharmaceuticals

Xenon Pharmaceuticals

Biocodex Pharmaceuticals

Insys Therapeutics, INC

Ovid Therapeutics

Novartis

Thermo Fisher Life Technologies

Biscayne Pharmaceuticals, Inc.

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Mylan

Sage Therapeutics, Inc.

AbbVie Inc

Johnson & Johnson

Key Products

STK-001

EPX-100

Soticlestat

Lorcaserin

Clemizole

VRP324

AntagoNAT

TANGO ASO

Diazepam

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