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Fragile X Syndrome – Pipeline Insight, 2020

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Abstracts

This report can be delivered to the clients within 96 Hours

DelveInsight's, "Fragile X Syndrome – Pipeline Insight, 2020," report provides comprehensive insights about 35+ companies and 35+ pipeline drugs in Fragile X 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

Fragile X Syndrome Understanding

Fragile X Syndrome: Overview

Fragile X syndrome (FXS) is a genetic disorder. FXS is caused by changes in a gene that scientists called the fragile X mental retardation 1 (FMR1) gene when it was first discovered. The FMR1 gene usually makes a protein called fragile X mental retardation protein (FMRP). FMRP is needed for normal brain development. People who have FXS do not make this protein. People who have other fragile X-associated disorders have changes in their FMR1 gene but usually make some of the protein.

FXS affects both males and females. However, females often have milder symptoms than males. The exact number of people who have FXS is unknown, but a review of research studies estimated that about 1 in 7,000 males about 1 in 11,000 females have

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been diagnosed with FXS.

Symptoms

Signs that a child might have FXS include:

Developmental delays (not sitting, walking, or talking at the same time as other children the same age);

Learning disabilities (trouble learning new skills); and

Social and behavior problems (such as not making eye contact, anxiety, trouble paying attention, hand flapping, acting and speaking without thinking, and being very active).

Diagnosis

FXS can be diagnosed by testing a person's DNA from a blood test. A doctor or genetic counselor can order the test. Testing also can be done to find changes in the FMR1 gene that can lead to fragile X-associated disorders.

A diagnosis of FXS can be helpful to the family because it can provide a reason for a child's intellectual disabilities and behavior problems. This allows the family and other caregivers to learn more about the disorder and manage care so that the child can reach his or her full potential. However, the results of DNA tests can affect other family members and raise many issues.

Treatment

There is no cure for FXS. However, treatment services can help people learn important skills. Services can include therapy to learn to talk, walk, and interact with others. In addition, medicine can be used to help control some issues, such as behavior problems.

Fragile X Syndrome Emerging Drugs Chapters

This segment of the Fragile X Syndrome report encloses its detailed analysis of various drugs in different stages of clinical development, including phase III, II, I, preclinical and Discovery. It also helps to understand clinical trial details, expressive pharmacological

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action, agreements and collaborations, and the latest news and press releases.

Fragile X Syndrome Emerging Drugs

Zygel: Zynerba Pharmaceuticals

Zygel is the first and only pharmaceutically-produced CBD, a non-euphoric cannabinoid, formulated as a patent-protected permeation-enhanced gel for transdermal delivery through the skin and into the circulatory system. Zygel is being developed for patients suffering from FXS, ASD in pediatric patients, 22q, and a heterogeneous group of rare and ultra-rare epilepsies known as developmental and epileptic encephalopathies (DEE). It is in phase II/III stage of treatment.

BPN14770: Tetra Therapeutics

BPN14770 is a novel therapeutic agent that selectively inhibits phosphodiesterase?4D (PDE4D) to enhance early and late stages of memory formation. This unique mechanism of action has the potential. Preclinical animal models show that BPN14770 has the potential to promote the maturation of connections between neurons, which is impaired in patients with Fragile X Syndrome. Tetra currently is conducting an investigational phase II study of BPN14770 in adults with Fragile X Syndrome.

AUT00206: Autifony Therapeutics

AUT00206 is being developed by Autifony Therapeutics for the treatment of Fragile X Syndrome. The drug recently completed a first-in-human clinical trial in healthy volunteers and was safe and well tolerated. In mice with a targeted knockout of the FMR1 gene, AUT00206 treatment for 21 days improved both cognitive and behavioral abnormalities that are similar to those that occur in children with Fragile X syndrome.

Pridopidine: Prilenia Therapeutics

Pridopidine is an orally bioavailable small molecule investigational drug exhibiting potential neuroprotective effect in multiple neurodegenerative diseases with a favorable safety profile. It is the most selective high affinity Sigma-1-receptor (S1R) agonist. The

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S1R regulates key cellular processes relevant to neurodegenerative diseases, such as calcium homeostasis, cytoskeleton dynamics, restoring mitochondrial health and neurotrophic factor release. S1R is implicated in cellular differentiation, neuroplasticity, neuroprotection, and cognitive functioning of the brain. It is in preclinical stage of development.

Further product details are provided in the report

Fragile X Syndrome: Therapeutic Assessment

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

Major Players in Fragile X Syndrome

There are approx. 35+ key companies which are developing the therapies for Fragile X Syndrome. The companies which have their Fragile X Syndrome drug candidates in the most advanced stage, i.e. phase II/III include Zynerba Pharmaceuticals and others

Phases

DelveInsight’s report covers around 35+ products under different phases of clinical development like

Late-stage products (Phase III)

Mid-stage products (Phase II)

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

Pre-clinical and Discovery stage candidates

Discontinued & Inactive candidates

Route of Administration

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Fragile X 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

- Infusion
- Intradermal
- Intramuscular
- Intranasal
- Intravenous
- Oral
- Parenteral
- Subcutaneous
- Topical.
- Molecule Type

Products have been categorized under various Molecule types such as

- Gene therapies
- Small molecule
- Vaccines
- Polymers
- Peptides
- Monoclonal antibodies

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Product Type

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

Fragile X Syndrome: Pipeline Development Activities

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

Report Highlights

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

Zynerba Pharmaceuticals announced that the US Food and Drug Association has granted orphan-drug designation to ZYN002 cannabidiol (CBD) gel, for the treatment of Fragile X syndrome (FXS).

In July 2017, Autifony Therapeutics Limited announced that the US Food and Drug Administration (FDA) has granted AUT00206 an Orphan Drug Designation for the treatment of Fragile X Syndrome.

Tetra Discovery Partners announced that the US Food and Drug Administration (FDA) has granted Orphan Drug Designation for BPN14770 for the treatment of Fragile X Syndrome.

Fragile X Syndrome Report Insights

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Key Questions

Current Treatment Scenario and Emerging Therapies:

How many companies are developing Fragile X Syndrome drugs?

How many Fragile X Syndrome drugs are developed by each company?

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

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

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

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What are the clinical studies going on for Fragile X Syndrome and their status?

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

Key Players

Zynerba Pharmaceuticals

Tetra Therapeutics

Autifony Therapeutics

Prilenia Therapeutics

Alcobra Ltd.

Seaside Therapeutics, Inc.

Marinus Pharmaceuticals

Novartis Pharmaceuticals

Neuren Pharmaceuticals

Hoffmann-La Roche

Key Products

Zygel

BPN14770

AUT00206

Pridopidine

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MG01CI

Arbaclofen

Ganaxolone

AFQ056

NNZ-2566

RO4917523

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