

Prader-Willi Syndrome - Pipeline Insight, 2021

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Abstracts

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DelveInsight's, "Prader-Willi Syndrome - Pipeline Insight, 2021," report provides comprehensive insights about 18+ companies and 18+ pipeline drugs in Prader-Willi 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

Prader-Willi Syndrome Understanding

Prader-Willi Syndrome: Overview

Prader-Willi syndrome (PWS) is a genetic multisystem disorder characterized during infancy by lethargy, diminished muscle tone (hypotonia), a weak suck and feeding difficulties with poor weight gain and growth and other hormone deficiency. In childhood, features of this disorder include short stature, small genitals and an excessive appetite. Affected individuals do not feel satisfied after completing a meal (satiety). Without intervention, overeating can lead to onset of life-threatening obesity. The food compulsion requires constant supervision. Individuals with severe obesity may have an increased risk of cardiac insufficiency, sleep apnea, diabetes, respiratory problems and other serious conditions that can cause life-threatening complications. All individuals with PWS have some cognitive impairment that ranges from low normal intelligence with

learning disabilities to mild to moderate intellectual disability. Behavioral problems are common and can include temper tantrums, obsessive/compulsive behavior, and skin picking.

'Prader-Willi Syndrome - Pipeline Insight, 2021' report by DelveInsight outlays comprehensive insights of present scenario and growth prospects across the indication. A detailed picture of the Prader-Willi Syndrome pipeline landscape is provided which includes the disease overview and Prader-Willi Syndrome treatment guidelines. The assessment part of the report embraces, in depth Prader-Willi 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, Prader-Willi 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 Prader-Willi Syndrome R&D. The therapies under development are focused on novel approaches to treat/improve Prader-Willi Syndrome.

Prader-Willi Syndrome Emerging Drugs Chapters

This segment of the Prader-Willi 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.

Prader-Willi Syndrome Emerging Drugs

Diazoxide choline controlled release (DCCR): Soleno Therapeutics

Diazoxide choline controlled-release tablet is a novel, proprietary extended-release, crystalline salt formulation of diazoxide, which is administered once-daily. The parent molecule, diazoxide, has been used for decades in thousands of patients in a few rare

diseases in neonates, infants, children and adults, but has not been approved for use in PWS. Soleno conceived of and established extensive patent protection on the therapeutic use of diazoxide and DCCR in patients with PWS. The DCCR development program is supported by data from five completed Phase I clinical studies in healthy volunteers and three completed Phase II clinical studies, one of which was in PWS patients. In the PWS Phase II study, DCCR showed promise in addressing hyperphagia, the hallmark symptom of PWS, as well as several other symptoms such as aggressive/destructive behaviors, fat mass and abnormal lipid profiles. It is currently in phase III stage of development.

Intranasal carbetocin (LV-101): Levo Therapeutics

Intranasal carbetocin is similar to the naturally-occurring hormone oxytocin, but with greater selectivity for the effect of interest – stimulation of oxytocin receptors. These receptors are involved in the regulation of both social-emotional and feeding behaviors. In July 2021, company announced that the U.S. Food and Drug Administration (FDA) had granted Priority Review for its New Drug Application (NDA) for LV-101 (intranasal carbetocin) as a treatment for hyperphagia and behavioral distress associated with PWS.

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

Prader-Willi Syndrome: Therapeutic Assessment

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

Major Players in Prader-Willi Syndrome

There are approx. 18+ key companies which are developing the therapies for Prader-Willi Syndrome. The companies which have their Prader-Willi Syndrome drug candidates in the most advanced stage, i.e. phase III include, Soleno Therapeutics.

Phases

DelveInsight's report covers around 18+ 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

Prader-Willi 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

Peptides

Polymer

Small molecule

Gene therapy

Product Type

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

Prader-Willi Syndrome: Pipeline Development Activities

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

Prader-Willi Syndrome Report Insights

Prader-Willi Syndrome Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Prader-Willi 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 Prader-Willi Syndrome drugs?

How many Prader-Willi Syndrome drugs are developed by each company?

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

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

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

Key Players

Soleno Therapeutics

Levo Therapeutics

Inversago Pharma

Saniona

LG Life Sciences

GLWL Research

OptiNose

Larimar Therapeutics

Helsinn

ConSynance Therapeutics

Neuren Pharmaceuticals

Radius Health

Rhythm

Tonix Pharmaceuticals

Key Products

Diazoxide choline controlled release (DCCR)

Intranasal carbetocin (LV-101)

INV 101

Tesomet

Eutropin

GLWL 01

OPN-300

ZGN 1258

HM 04

CSTI 500

NNZ 2591

RAD-011

RM 853

TNX-2900

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Assessment by Stage and Molecule Type

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Diazoxide choline controlled release (DCCR): Soleno Therapeutics

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report.....

Mid Stage Products (Phase II)

Comparative Analysis

Tesomet: Saniona

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report.....

Early Stage Products (Phase I)

Comparative Analysis

INV 101: Inversago Pharma

Product Description

Research and Development

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