

Basal Cell Nevus Syndrome (BCNS) – Pipeline Insight, 2020

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Abstracts

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DelveInsight's, "Basal Cell Nevus Syndrome (BCNS) – Pipeline Insight, 2020," report provides comprehensive insights about 5+ companies and 5+ pipeline drugs in Basal Cell Nevus Syndrome (BCNS) 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

Basal Cell Nevus Syndrome (BCNS) Understanding

Basal Cell Nevus Syndrome (BCNS): Overview

Basal Cell Nevus Syndrome (BCNS) is a multisystemic autosomal dominant disorder with a high penetrance and variable expressiveness. BCNS is a rare inherited condition characterised by multiple and early onset basal cell carcinomas and other tumours including melanoma, medulloblastoma, meningioma, breast carcinoma, non-Hodgkin lymphoma, and ovarian fibroma. The other names for BCNS include basal cell carcinoma nevus syndrome (BCCNS), Gorlin-Goltz syndrome, Gorlin syndrome, and nevoid basal cell carcinoma syndrome. These basal cell naevi are actually tiny basal cell carcinomas. It is mostly caused by mutations in the PTCH1 gene, sometimes



mutations in the SUFU and PTCH2 genes can cause a similar set of characteristics (phenotype).

Symptoms

The symptoms of Basal Cell Nevus Syndrome (BCNS) include:

Multiple basal cell carcinomas

Recurrent keratocystic odontogenic tumors of the jaws

Skeletal malformations (some affected individuals may have distinctive facial features)

Odontogenic cysts in the jaw

Broad forehead

Pits on palms and soles

Rib anomalies

Calcified falx cerebri (noted on skull X-ray)

Diagnosis

A diagnosis of Basal Cell Nevus Syndrome (BCNS) is based upon physical examination, detection of a pathogenic genetic variant, identification of characteristic clinical findings and imaging studies. The physical examination include oral exploration, cephalic diameter, craniofacial morphology, skin exploration, and evaluation of the palms and soles. Complementary tests includes skull X-rays, orthopantogram, chest X-ray, and spinal X-ray. Ultrasound examinations are used to diagnose cardiac and ovarian fibromas. The diagnosis of BCNS can be confirmed by the detection of a mutation of the PTCH gene.

Treatment

Treating the underlying cause of Basal Cell Nevus Syndrome (BCNS) a multidisciplinary



approach. The first-line of treatment for limited BCCs (nodular or aggressive) is usually surgical excision by standard or micro excisional/Mohs techniques. A wide variety of other treatment options for individuals with BCCs includes topical chemotherapy, drug therapy, cryotherapy, electrodessication and curettage, and photodynamic therapy. Future treatment options for BCNS patients include PTCH1- or SMO-specific targeted drugs which prevent the expression of tumor mediating genes within the hedgehog pathway.

Basal Cell Nevus Syndrome (BCNS) Emerging Drugs Chapters

This segment of the Basal Cell Nevus Syndrome (BCNS) 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 action, agreements and collaborations, and the latest news and press releases.

Basal Cell Nevus Syndrome (BCNS) Emerging Drugs

Patidegib - PellePharm

Patidegib is an investigational topical treatment designed to mitigate the tumor burden in patients. It is designed to block the Smo signal so that the cells stop dividing and thus inhibits the hedgehog pathway rapidly along with a potentially shrink of BCCs. PellePharm has received both Orphan Drug Designation and Breakthrough Therapy Designation for Patidegib Topical Gel in BCNS from the FDA, as well as Orphan Drug Designation in BCNS from EMA's Committee for Orphan Medicinal Products in the EU.

Vismodegib: Roche

Vismodegib is an experimental, oral smoothened antagonist and thus inhibits hedgehog pathway. It is under Phase II development for Basal cell nevus syndrome.

Further product details are provided in the report

Basal Cell Nevus Syndrome (BCNS): Therapeutic Assessment

This segment of the report provides insights about the different Basal Cell Nevus



Syndrome (BCNS) drugs segregated based on following parameters that define the scope of the report, such as:

Major Players in Basal Cell Nevus Syndrome (BCNS)

There are approx. 5+ key companies which are developing the therapies for Basal Cell Nevus Syndrome (BCNS). The companies which have their Basal Cell Nevus Syndrome (BCNS) drug candidates in the most advanced stage, i.e. phase III include, PellePharm.

Phases

DelveInsight's report covers around 5+ 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

Basal Cell Nevus Syndrome (BCNS) 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

intravitreal



Subretinal

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.
Basal Cell Nevus Syndrome (BCNS): Pipeline Development Activities
The report provides insights into different therapeutic candidates in phase II, I, preclinical and discovery stage. It also analyses Basal Cell Nevus Syndrome (BCNS) therapeutic drugs key players involved in developing key drugs.

The report covers the detailed information of collaborations, acquisition and merger, licensing along with a thorough therapeutic assessment of emerging Basal Cell Nevus

Report Highlights

Syndrome (BCNS) drugs.

Pipeline Development Activities



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

Basal Cell Nevus Syndrome (BCNS) Report Insight

Basal Cell Nevus Syndrome (BCNS) Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Basal Cell Nevus Syndrome (BCNS) 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 Basal Cell Nevus Syndrome (BCNS) drugs?

How many Basal Cell Nevus Syndrome (BCNS) drugs are developed by each company?



How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Basal Cell Nevus Syndrome (BCNS)?

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

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

Key Players

Palvella Therapeutics

Sun Pharmaceutical Industries

Roche

PellePharm

Asana BioSciences

Mayne Pharma

Key Products

Sirolimus topical

Sonidegib

Vismodegib



Patidegib

Gusacitinib

SUBA-Itraconazole



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Product Description

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Product Development Activities

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Vismodegib - Roche

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report



Early Stage Products (Phase I)

Comparative Analysis

Sirolimus topical: Palvella Therapeutics

Product Description

Research and Development

Product Development Activities

Drug profiles in the detailed report

Inactive Products

Comparative Analysis

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Basal Cell Nevus Syndrome (BCNS) Key Products

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