

Congenital Adrenal Hyperplasia - Pipeline Insight, 2021

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Abstracts

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DelveInsight's, "Congenital Adrenal Hyperplasia – Pipeline Insight, 2021," report provides comprehensive insights about 5+ companies and 5+ pipeline drugs in Congenital Adrenal Hyperplasia 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

Congenital Adrenal Hyperplasia Understanding

Congenital Adrenal Hyperplasia: Overview

Congenital adrenal hyperplasia (CAH) is a group of rare inherited autosomal recessive disorders characterized by a deficiency of one of the enzymes needed to make specific hormones. CAH effects the adrenal glands located at the top of each kidney. Normally, the adrenal glands are responsible for producing three different hormones: 1. corticosteroids, which gauge the body's response to illness or injury; 2. mineralocorticoids, which regulate salt and water levels; and 3. androgens, which are male sex hormones. An enzyme deficiency will make the body unable to produce one or more of these hormones, which in turn will result in the overproduction of another type



of hormone precursor in order to compensate for the loss. The most common cause of CAH is the absence of the enzyme 21-hydroxylase. Different mutations in the gene responsible for 21-hydroxylase result in different levels of the enzyme, producing a spectrum of effects. Genetic testing for the gene mutations associated with the various forms of CAH is available, but is most often performed when pre-pregnancy genetic counseling is indicated, after an endocrinologist confirms the diagnosis through blood hormone tests, or if results of hormone tests are not definitive. Treatment of CAH varies greatly depending on the type and severity. CAH cannot be cured, but it can be effectively treated. The primary goal of treating classical CAH is to reduce the excess androgen production and replace the deficient hormones.

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

Congenital Adrenal Hyperplasia Emerging Drugs Chapters

This segment of the Congenital Adrenal Hyperplasia 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.



Congenital Adrenal Hyperplasia Emerging Drugs

Chronocort: Diurnal Group plc

Chronocort is a preparation of hydrocortisone that has been specifically designed for patients with CAH, an orphan condition, caused by deficiency of adrenal enzymes, most commonly 21-hydroxylase. Diurnal holds the relevant Orphan Drug Designation for use of Chronocort in CAH in the US. It is under review by the European Medicines Agency (EMA) and the UK Medicines and Healthcare products Regulatory Agency (MHRA), with the submissions based on the Phase III clinical trial and safety extension study data. the US Food and Drug Administration (FDA) has agreed a Special Protocol Assessment (SPA) for Chronocort (modified-release hydrocortisone) for the design, endpoints and statistical analysis approach of a pivotal study of Chronocort for the treatment for Congenital Adrenal Hyperplasia (CAH) in the US. The SPA provides agreement that the Phase III trial design adequately addresses objectives that would support the regulatory submission for drug approval.

Further product details are provided in the report.

Congenital Adrenal Hyperplasia: Therapeutic Assessment

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

Major Players in Congenital Adrenal Hyperplasia

There are approx. 5+ key companies which are developing the therapies for Congenital Adrenal Hyperplasia. The companies which have their Congenital Adrenal Hyperplasia drug candidates in the most advanced stage, i.e. Registered include, Diurnal Group plc.

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

Congenital Adrenal Hyperplasia 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.

Congenital Adrenal Hyperplasia: Pipeline Development Activities

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

Congenital Adrenal Hyperplasia Report Insights

Congenital Adrenal Hyperplasia Pipeline Analysis

Therapeutic Assessment

Unmet Needs

Impact of Drugs

Congenital Adrenal Hyperplasia 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 Congenital Adrenal Hyperplasia drugs?

How many Congenital Adrenal Hyperplasia drugs are developed by each company?

How many emerging drugs are in mid-stage, and late-stage of development for the treatment of Congenital Adrenal Hyperplasia?

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

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



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Introduction **Executive Summary** Congenital Adrenal Hyperplasia: Overview Causes Mechanism of Action Signs and Symptoms Diagnosis **Disease Management** Pipeline Therapeutics **Comparative Analysis** Therapeutic Assessment Assessment by Product Type Assessment by Stage and Product Type Assessment by Route of Administration Assessment by Stage and Route of Administration Assessment by Molecule Type Assessment by Stage and Molecule Type Late Stage Products (Registered) **Comparative Analysis** Chronocort: Diurnal Group plc **Product Description** Research and Development **Product Development Activities** Drug profiles in the detailed report. Mid Stage Products (Phase II) Comparative Analysis SPR001: spruce Biosciences **Product Description** Research and Development **Product Development Activities** Drug profiles in the detailed report. Early stage products (Phase I/II) **Comparative Analysis** AAV BBP-631: Adrenas Therapeutics Inc **Product Description** Research and Development Product Development Activities



Drug profiles in the detailed report. Inactive Products Comparative Analysis Congenital Adrenal Hyperplasia Key Companies Congenital Adrenal Hyperplasia Key Products Congenital Adrenal Hyperplasia- Unmet Needs Congenital Adrenal Hyperplasia- Market Drivers and Barriers Congenital Adrenal Hyperplasia- Future Perspectives and Conclusion Congenital Adrenal Hyperplasia Analyst Views Congenital Adrenal Hyperplasia Key Companies Appendix



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