

Achondroplasia - Pipeline Review, H2 2020

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Abstracts

Achondroplasia - Pipeline Review, H2 2020

SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Achondroplasia - Pipeline Review, H2 2020, provides an overview of the Achondroplasia (Musculoskeletal Disorders) pipeline landscape.

Achondroplasia is a bone growth disorder that causes disproportionate dwarfism. This is caused by mutations in the FGFR3 gene. Symptoms include decreased muscle tone, apnea, hydrocephalus, short arms and legs, disproportionately large head compared to the body and kyphosis. Treatment includes growth hormones.

REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Achondroplasia - Pipeline Review, H2 2020, provides comprehensive information on the therapeutics under development for Achondroplasia (Musculoskeletal Disorders), complete with analysis by stage of development, drug target, mechanism of action (MoA), route of administration (RoA) and molecule type. The guide covers the descriptive pharmacological action of the therapeutics, its complete research and development history and latest news and press releases.

The Achondroplasia (Musculoskeletal Disorders) pipeline guide also reviews of key players involved in therapeutic development for Achondroplasia and features dormant and discontinued projects. The guide covers therapeutics under Development by Companies/Universities/Institutes, the molecules developed by Companies in Pre-Registration, Phase II, Phase I, Preclinical and Discovery stages are 1, 2, 2, 1 and 1 respectively.

Achondroplasia (Musculoskeletal Disorders) pipeline guide helps in identifying and

tracking emerging players in the market and their portfolios, enhances decision making capabilities and helps to create effective counter strategies to gain competitive advantage. The guide is built using data and information sourced from Global Markets Direct's proprietary databases, company/university websites, clinical trial registries, conferences, SEC filings, investor presentations and featured press releases from company/university sites and industry-specific third party sources. Additionally, various dynamic tracking processes ensure that the most recent developments are captured on a real time basis.

Note:

Certain content/sections in the pipeline guide may be removed or altered based on the availability and relevance of data.

SCOPE

The pipeline guide provides a snapshot of the global therapeutic landscape of Achondroplasia (Musculoskeletal Disorders).

The pipeline guide reviews pipeline therapeutics for Achondroplasia (Musculoskeletal Disorders) by companies and universities/research institutes based on information derived from company and industry-specific sources.

The pipeline guide covers pipeline products based on several stages of development ranging from pre-registration till discovery and undisclosed stages.

The pipeline guide features descriptive drug profiles for the pipeline products which comprise, product description, descriptive licensing and collaboration details, R&D brief, MoA & other developmental activities.

The pipeline guide reviews key companies involved in Achondroplasia (Musculoskeletal Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Achondroplasia (Musculoskeletal Disorders) therapeutics based on mechanism of action (MoA), drug target, route of administration (RoA) and molecule type.

The pipeline guide encapsulates all the dormant and discontinued pipeline projects.

The pipeline guide reviews latest news related to pipeline therapeutics for Achondroplasia (Musculoskeletal Disorders)

REASONS TO BUY

Procure strategically important competitor information, analysis, and insights to formulate effective R&D strategies.

Recognize emerging players with potentially strong product portfolio and create effective counter-strategies to gain competitive advantage.

Find and recognize significant and varied types of therapeutics under development for Achondroplasia (Musculoskeletal Disorders).

Classify potential new clients or partners in the target demographic.

Develop tactical initiatives by understanding the focus areas of leading companies.

Plan mergers and acquisitions meritoriously by identifying key players and it's most promising pipeline therapeutics.

Formulate corrective measures for pipeline projects by understanding Achondroplasia (Musculoskeletal Disorders) pipeline depth and focus of Indication therapeutics.

Develop and design in-licensing and out-licensing strategies by identifying prospective partners with the most attractive projects to enhance and expand business potential and scope.

Adjust the therapeutic portfolio by recognizing discontinued projects and understand from the know-how what drove them from pipeline.

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Featured News & Press Releases

Sep 11, 2020: BioMarin announces presentation of Vosoritide phase 3 data in children with Achondroplasia at the American Society for Bone and Mineral Research 2020 Annual Meeting

Sep 08, 2020: BioMarin announces The Lancet publishes detailed vosoritide phase 3 data demonstrating statistically significant increase in annualized growth velocity (AGV) over 52 weeks in children with achondroplasia

Aug 20, 2020: BioMarin submits new drug application to U.S. Food and Drug Administration for Vosoritide to treat children with achondroplasia

Aug 13, 2020: European Medicines Agency validates BioMarin's Marketing Authorization Application for vosoritide to treat children with achondroplasia

Aug 12, 2020: Ascendis Pharma receives Orphan Designation for TransCon CNP for the treatment of achondroplasia in Europe

Jul 23, 2020: BioMarin submits marketing authorization application to European Medicines Agency for vosoritide to treat children with achondroplasia

Jul 15, 2020: BridgeBio Pharma's QED Therapeutics doses first child in Phase 2 clinical trial of the investigational medicine Infigratinib in Achondroplasia

Jul 15, 2020: RIBOMIC announces dosing of first subject in RBM-007 phase 1 clinical trial for Achondroplasia

May 11, 2020: BridgeBio Pharma's QED Therapeutics announces preclinical data demonstrating potential of low-dose infigratinib in achondroplasia

Apr 06, 2020: BioMarin plans regulatory submissions for marketing authorization of Vosoritide to treat children with achondroplasia in 3Q 2020 in both US and Europe

Jan 08, 2020: Wacker Biotech provides active ingredient to treat achondroplasia in children for Ascendis Pharma global phase-2 trial

Dec 16, 2019: BioMarin announces positive final results from placebo-controlled phase 3 data in children with achondroplasia treated with Vosoritide

Nov 14, 2019: BioMarin announces cumulative additional height gain of 9.0 cm over 54 months versus natural history in children with Achondroplasia treated with Vosoritide in phase 2 study

Nov 06, 2019: BioMarin to highlight innovative development pipeline including vosoritide at R&D Day on November 14th

Oct 17, 2019: QED and parent company BridgeBio announce preclinical data supporting tolerability and activity of low-dose infigratinib in treating Achondroplasia

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COMPANIES MENTIONED

Ascendis Pharma A/S

BioMarin Pharmaceutical Inc

Pfizer Inc

PhaseBio Pharmaceuticals Inc

QED Therapeutics Inc

Ribomic Inc

TagCyx Biotechnologies

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