

# **Achondroplasia Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update**

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## **Abstracts**

Achondroplasia Drugs in Development by Stages, Target, MoA, RoA, Molecule Type and Key Players, 2022 Update

### **SUMMARY**

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Achondroplasia - Drugs In Development, 2022, 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 - Drugs In Development, 2022, 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, 3, 2, 3 and 3 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

Apr 19, 2022: RIBOMIC announces completion of IND submission for an observational study for continuous phase 2 trial of RBM-007 for treatment of Achondroplasia

Nov 19, 2021: BioMarin receives FDA approval for VOXZOGO (vosoritide) for injection, indicated to increase linear growth in children with achondroplasia aged 5 and up with open growth plates

Nov 19, 2021: FDA approves first drug to improve growth in children with most common form of dwarfism

Nov 15, 2021: RIBOMIC announces RBM-007 phase 1 clinical trial results for achondroplasia

Aug 30, 2021: BioMarin's Voxzogo receives EC approval for achondroplasia in children

Jul 22, 2021: Lixte Biotechnology comments on report that its PP2A inhibitor LB-100 combined with another investigational agent stimulates bone growth in models of dwarfism

Jun 25, 2021: BioMarin receives positive CHMP opinion in Europe for Vosoritide for the treatment of children with Achondroplasia from age 2 until growth plates close

May 12, 2021: RIBOMIC announces completion of its phase I trial of RBM-007 for the treatment of achondroplasia

May 06, 2021: RIBOMIC announces publication of research paper on aptamer drug discovery for achondroplasia

Apr 15, 2021: BioMarin announces new and updated data at 2021 American College of

Medical Genetics and Genomics (ACMG) Annual Clinical Genetics Meeting  
demonstrating commitment to understanding achondroplasia and potential treatment  
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Mar 20, 2021: BioMarin announces oral presentation at ENDO2021, the Endocrine  
Society's annual meeting, with data demonstrating 2 years of treatment benefit in  
children with achondroplasia treated with Vosoritide

Mar 03, 2021: BioMarin completes full enrollment in phase 2 study of vosoritide for  
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Jan 07, 2021: VISEN Pharmaceuticals receives IND approval to initiate phase 2 clinical  
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Dec 21, 2020: BioMarin announces benefit maintained for over two years in children  
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Dec 15, 2020: Pfizer doses first participants as part of global achondroplasia phase 2  
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