

Adrenoleukodystrophy (Adrenomyeloneuropathy/ Schilder-Addison Complex) - Pipeline Review, H1 2020

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Abstracts

Adrenoleukodystrophy (Adrenomyeloneuropathy/Schilder-Addison Complex) - Pipeline Review, H1 2020

SUMMARY

Global Markets Direct's latest Pharmaceutical and Healthcare disease pipeline guide Adrenoleukodystrophy – Pipeline Review, H1 2020, provides an overview of the Adrenoleukodystrophy (Genetic Disorders) pipeline landscape.

'Adrenoleukodystrophy is a disorder that occurs most often in males. It mainly affects the nervous system and the adrenal glands, which are small glands located on top of each kidney. Abetalipoproteinemia is also a kind of Adrenoleukodystrophy (Adrenomyeloneuropathy/Schilder-Addison Complex) Bassen-Kornzweig syndrome is a rare disease passed down through families in which a person is unable to fully absorb dietary fats through the intestines. The disease is caused by a defect in gene that tells the body to create lipoproteins. It is symptomized by curvature of spine, muscle weakness, decrease of vision over time. Treatment involves large doses of vitamin supplements containing fat-soluble vitamins.'

REPORT HIGHLIGHTS

Global Markets Direct's Pharmaceutical and Healthcare latest pipeline guide Adrenoleukodystrophy – Pipeline Review, H1 2020, provides comprehensive information on the therapeutics under development for Adrenoleukodystrophy (Genetic 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 Adrenoleukodystrophy (Genetic Disorders) pipeline guide also reviews of key players involved in therapeutic development for Adrenoleukodystrophy (Adrenomyeloneuropathy/Schilder-Addison Complex) and features dormant and discontinued projects. The guide covers therapeutics under Development by Companies/Universities/Institutes, the molecules developed by Companies in Phase III, Phase II, Phase I, Preclinical and Discovery stages are 3, 2, 2, 4 and 1 respectively. Similarly, the Universities portfolio in Phase II, Phase I and Preclinical stages comprises 2, 1 and 2 molecules, respectively.

Adrenoleukodystrophy (Genetic 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 Adrenoleukodystrophy (Genetic Disorders).

The pipeline guide reviews pipeline therapeutics for Adrenoleukodystrophy (Genetic 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 Adrenoleukodystrophy (Genetic Disorders) therapeutics and enlists all their major and minor projects.

The pipeline guide evaluates Adrenoleukodystrophy (Genetic 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 Adrenoleukodystrophy (Genetic 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 Adrenoleukodystrophy (Genetic 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 Adrenoleukodystrophy (Genetic 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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MedDay SA

Minoryx Therapeutics sl

NeuroVia Inc

Orpheris Inc

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SwanBio Therapeutics Ltd

Viking Therapeutics Inc

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Featured News & Press Releases

Feb 20, 2020: Magenta Therapeutics announces updated phase 2 data on MGTA-456 cell therapy, demonstrating continued durability in inherited metabolic disorders

Sep 20, 2019: bluebird bio reports new data for Lenti-D gene therapy

Sep 04, 2019: Magenta Therapeutics announces FDA regenerative medicine advanced therapy (RMAT) designation granted to MGTA-456 for the treatment of Inherited Metabolic Disorders

Sep 03, 2019: bluebird bio to present data from clinical development program of Lenti-DTM Gene Therapy for Cerebral Adrenoleukodystrophy (CALD) at the 13th European

Pediatric Neurology Society (EPNS) Congress

May 09, 2019: Magenta Therapeutics presents updated phase 2 clinical data on MGTA-456 cell therapy at American Academy of Neurology Annual Meeting

Dec 02, 2018: Magenta Therapeutics presents new data from phase 2 study of MGTA-456 cell therapy in patients with inherited metabolic disorders

Nov 02, 2018: Magenta presents preclinical data on MGTA-456 at ASH annual meeting 2018

Sep 05, 2018: bluebird bio presents updated data from phase 2/3 starbeam study of investigational Lenti-D gene therapy for CALD and initial data from observational study ALD-103 of allogeneic hematopoietic stem cell transplant in CALD at 2018 SSIEM

Aug 03, 2018: bluebird bio to Present Updated Data for Lenti-D Gene Therapy for Cerebral Adrenoleukodystrophy at SSIEM 2018 Symposium

May 23, 2018: FDA Grants Breakthrough Therapy Designation to Lenti-D for the Treatment of Cerebral Adrenoleukodystrophy

Apr 05, 2018: Magenta Therapeutics Announces First Patient Transplanted with MGTA-456 in Phase 2 Study in Inherited Metabolic Disorders

Dec 04, 2017: Orpheris Appoints Neil Warma to Board of Directors

Nov 29, 2017: Orpheris Obtains FDA Agreement On Single, Seamless Phase 1/2/3 Trial to Registration for Its Lead Drug Candidate, OP-101, as a Treatment for Childhood Cerebral Adrenoleukodystrophy (ccALD)

Nov 27, 2017: Orpheris Receives Orphan Drug Designation from the U.S. FDA for its Lead Drug Candidate, OP-101, for the Treatment of ccALD

Oct 23, 2017: Viking Therapeutics Presents Results from Proof-of-Concept Study of VK0214 in In Vivo Model of X-Linked Adrenoleukodystrophy at the 87th Annual Meeting of the American Thyroid Association

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Applied Genetic Technologies Corp
bluebird bio Inc
Magenta Therapeutics Inc
MedDay SA
Minoryx Therapeutics sl
NeuroVia Inc
Orpheris Inc
Poxel SA
SOM Biotech SL
SwanBio Therapeutics Ltd
Viking Therapeutics Inc

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