

Alpha L-Iduronidase (IDUA or EC 3.2.1.76) - Drugs In Development, 2021

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Abstracts

Alpha L-Iduronidase (IDUA or EC 3.2.1.76) - Drugs In Development, 2021

SUMMARY

Alpha L-Iduronidase (IDUA or EC 3.2.1.76) pipeline Target constitutes close to 13 molecules. Out of which approximately 12 molecules are developed by companies and remaining by the universities/institutes. The latest report Alpha L-Iduronidase - Drugs In Development, 2021, outlays comprehensive information on the Alpha L-Iduronidase (IDUA or EC 3.2.1.76) targeted therapeutics, complete with analysis by indications, stage of development, mechanism of action (MoA), route of administration (RoA) and molecule type.

Alpha L-Iduronidase (IDUA or EC 3.2.1.76) - Alpha L-iduronidase is an enzyme encoded by IDUA gene. It hydrolyzes the terminal alpha-L-iduronic acid residues of two glycosaminoglycans, dermatan sulfate and heparan sulfate. Mutations in this gene that result in enzymatic deficiency lead to the autosomal recessive disease mucopolysaccharidosis type I (MPS I). The molecules developed by companies in Phase II, Preclinical and Discovery stages are 4, 6 and 2 respectively. Similarly, the universities portfolio in Discovery stages comprises 1 molecules, respectively. Report covers products from therapy areas Genetic Disorders which include indications Mucopolysaccharidosis I (MPS I) (Hurler Syndrome).

Furthermore, this report also reviews key players involved in Alpha L-Iduronidase (IDUA or EC 3.2.1.76) targeted therapeutics development with respective active and dormant or discontinued projects. Driven by data and information sourced from 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.

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

SCOPE

The report provides a snapshot of the global therapeutic landscape for Alpha L-Iduronidase (IDUA or EC 3.2.1.76)

The report reviews Alpha L-Iduronidase (IDUA or EC 3.2.1.76) targeted therapeutics under development by companies and universities/research institutes based on information derived from company and industry-specific sources

The report covers pipeline products based on various stages of development ranging from pre-registration till discovery and undisclosed stages

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

The report reviews key players involved in Alpha L-Iduronidase (IDUA or EC 3.2.1.76) targeted therapeutics and enlists all their major and minor projects

The report assesses Alpha L-Iduronidase (IDUA or EC 3.2.1.76) targeted therapeutics based on mechanism of action (MoA), route of administration (RoA) and molecule type

The report summarizes all the dormant and discontinued pipeline projects

The report reviews latest news and deals related to Alpha L-Iduronidase (IDUA or EC 3.2.1.76) targeted therapeutics

REASONS TO BUY

Gain strategically significant competitor information, analysis, and insights to



formulate effective R&D strategies

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

Identify and understand the targeted therapy areas and indications for Alpha L-Iduronidase (IDUA or EC 3.2.1.76)Identify the use of drugs for target identification and drug repurposing

Identify potential new clients or partners in the target demographic

Develop strategic initiatives by understanding the focus areas of leading companies

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

Devise corrective measures for pipeline projects by understanding Alpha L-Iduronidase (IDUA or EC 3.2.1.76) development landscape

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



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Development

ArmaGen Inc

BioStrategies LC

Denali Therapeutics Inc

EdiGene Inc

Gain Therapeutics Inc

Immusoft Corp

JCR Pharmaceuticals Co Ltd

Orchard Therapeutics Plc

Ossianix Inc

Rain Bio Inc

RegenxBio Inc

Tega Therapeutics Inc

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Product Description

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Product Description

Mechanism Of Action

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Featured News & Press Releases

Mar 30, 2021: EMA grants Orphan Drug Designation to JR-171 for the treatment of Mucopolysaccaridosis Type I (MPS I)

Feb 12, 2021: US FDA grants orphan drug designation to JR-171 for the treatment of Mucopolysaccaridosis Type I (MPS I)

Feb 09, 2021: Orchard Therapeutics announces interim data for OTL-203 showing positive clinical results in multiple disease manifestations of mucopolysaccharidosis type I Hurler syndrome (MPS-IH)

Jan 28, 2021: Orchard Therapeutics to present abstact on OTL-203 at 2021 WORLDSymposium

Dec 01, 2020: RegenxBio announces dosing of first patient in phase I/II trial of RGX-111 for the treatment of Mucopolysaccharidosis type I

Sep 28, 2020: Orchard Therapeutics receives EMA PRIME designation for OTL-203 for the treatment of MPS-I

Sep 01, 2020: Orchard Therapeutics announces additional interim results from proof-of-concept study of OTL-203 for MPS-I

Aug 24, 2020: Orchard Therapeutics announces clinical data presentations at the 46th Annual Meeting of the European Society for Blood and Marrow Transplantation (EBMT) Jul 20, 2020: Orchard Therapeutics announces orphan drug and rare pediatric disease designations for OTL-203 for the treatment of MPS-I

Jul 08, 2020: REGENXBIO provides update on progress of Clinical Programs including RGX-111 for Rare Genetic Neurodegenerative Diseases

May 15, 2020: Orchard Therapeutics presents new interim data from OTL-203 proof-of-concept study for MPS-I

Feb 10, 2020: Orchard Therapeutics announces presentation on OTL-203 at 16th Annual WORLD Symposium

Dec 08, 2019: Orchard Therapeutics presents clinical data on OTL-203, at the 61st American Society of Hematology Annual Meeting

Nov 06, 2019: Orchard Therapeutics to present on its Hurler Syndrome drug candidate



OTL-203 at the 61st American Society of Hematology Annual Meeting Sep 04, 2019: Orchard Therapeutics announces encouraging update from proof-ofconcept study of OTL-203 for the treatment of Mucopolysaccharidosis Type I (MPS-I)

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Pipeline by BioStrategies LC, 2021

Pipeline by Denali Therapeutics Inc, 2021

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