

# **Alpha L-Iduronidase (IDUA or EC 3.2.1.76) Development by Therapy Areas and Indications, Stages, MoA, RoA, Molecule Type and Key Players, 2022 Update**

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## **Abstracts**

Alpha L-Iduronidase (IDUA or EC 3.2.1.76) Development by Therapy Areas and Indications, Stages, MoA, RoA, Molecule Type and Key Players, 2022 Update

### **SUMMARY**

According to the recently published report 'Alpha L-Iduronidase - Drugs In Development, 2022'; Alpha L-Iduronidase (IDUA or EC 3.2.1.76) pipeline Target constitutes close to 14 molecules. Out of which approximately 13 molecules are developed by companies and remaining by the universities/institutes.

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 report 'Alpha L-Iduronidase - Drugs In Development, 2022' 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; that are being developed by Companies / Universities.

It 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. Currently, The molecules developed by companies in Phase II, IND/CTA Filed, Preclinical and Discovery stages are 4, 1, 6 and 2 respectively. Similarly, the universities portfolio in Discovery stages comprises 1 molecules, respectively. Report covers products from therapy areas Genetic Disorders, Central Nervous System, Musculoskeletal Disorders, Oncology and Undisclosed which include indications Mucopolysaccharidosis I (MPS I) (Hurler Syndrome ), Unspecified, Unspecified Cancer, Unspecified Musculoskeletal Disorders and Unspecified Neurologic Disorders.

**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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Orchard Therapeutics Plc

Ossianix Inc

RainBio Inc

RegenxBio Inc

Sigilon Therapeutics Inc

Tega Therapeutics Inc

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Featured News & Press Releases

Feb 09, 2022: REGENXBIO presents positive initial data from Phase I/II of RGX-111 for the treatment of severe MPS I at 18th annual WORLDSymposium 2022

Feb 07, 2022: Immusoft to present at WORLDSymposium 2022 Conference

Feb 04, 2022: JCR pharmaceuticals to present posters on JR-171 at the 18th annual WORLDSymposium 2022

Jan 31, 2022: Sigilon Therapeutics to present preclinical data at the 18th Annual WORLDSymposium on Lysosomal Diseases, MPS-1 and MPS-6

Nov 18, 2021: Orchard Therapeutics announces new New England Journal Medicine publication of interim proof-of-concept study results of OTL-203 for Hurler Syndrome

Oct 05, 2021: JCR Pharmaceuticals: US FDA grants Fast Track Designation for JR-171 for the treatment of Mucopolysaccharidosis Type I (MPSI)

Sep 09, 2021: Sigilon Therapeutics announces acceptance of clinical trial application in the UK for SIG-005 for the treatment of mucopolysaccharidosis type I

Jul 23, 2021: Sigilon Therapeutics presents preclinical data at the 16th International Symposium on MPS and Related Diseases

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

Feb 12, 2021: US FDA grants orphan drug designation to JR-171 for the treatment of Mucopolysaccharidosis 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 abstract on OTL-203 at 2021 WORLDSymposium

Dec 17, 2020: Sigilon Therapeutics receives Orphan Drug Designation for SIG-005 for the treatment of mucopolysaccharidosis type I

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

Oct 30, 2020: JCR announces first patient dosed in phase 1/2 global clinical trial of JR-171 for mucopolysaccharidosis type I (MPS I)

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