

# Alpha L-Iduronidase - Pipeline Review, H1 2020

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## Abstracts

Alpha L-Iduronidase - Pipeline Review, H1 2020

### 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 - Pipeline Review, H1 2020, 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, Phase I, Preclinical and Discovery stages are 3, 1, 7 and 1 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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bluebird bio Inc

GT Gain Therapeutics SA

Immusoft Corp

JCR Pharmaceuticals Co Ltd

Orchard Therapeutics Plc

Ossianix Inc

RegenxBio Inc

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Featured News & Press Releases

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-of-concept study of OTL-203 for the treatment of Mucopolysaccharidosis Type I (MPS-I)

Aug 27, 2019: Orchard Therapeutics announces presentation on OTL-203 at Upcoming Society for the Study of Inborn Errors of Metabolism (SSIEM) 2019 Annual Symposium

Jul 17, 2019: Immusoft announces the appointment of Robert Hayes, Ph.D. as Chief Scientific Officer

Apr 02, 2019: Sangamo provided an update on its in vivo genome editing program: SB-318

Feb 12, 2019: JCR announces presentation on JR-171 for hurler syndrome at the 15th Annual ORLDSymposium 2019

Feb 07, 2019: Sangamo Announces Interim Results Of Phase 1/2 EMPOWERS Study Evaluating SB-318 Zinc Finger Nuclease (ZFN) In Vivo Genome Editing Demonstrating Increased Leukocyte IDUA Activity In Patients With MPS I

Feb 01, 2019: Sangamo Therapeutics to host conference call to review interim results from phase 1/2 EMPOWERS study for Mucopolysaccharidosis type I

Jan 04, 2019: Sangamo announces upcoming clinical data Presentation on SB-318 At WORLDSymposium 2019

Oct 17, 2018: Immusoft receives rare pediatric disease designation for treatment of MPS I

Jul 23, 2018: Sangamo treats first patient in EMPOWERS Study of SB-318

Jul 10, 2018: ArmaGen's AGT-181 52-Week Phase 1/2 Proof-of-Concept Study Results Published in Orphanet Journal of Rare Diseases

Jun 12, 2018: REGENXBIO Receives FDA Fast Track Designation for RGX-111 Gene

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### COMPANIES MENTIONED

ArmaGen Inc

BioStrategies LC

bluebird bio Inc

GT Gain Therapeutics SA

Immusoft Corp

JCR Pharmaceuticals Co Ltd

Orchard Therapeutics Plc

Ossianix Inc

RegenxBio Inc

Sangamo Therapeutics Inc

Tamid Bio Inc

Tega Therapeutics Inc

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