

# Iduronate 2 Sulfatase - Pipeline Review, H1 2020

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### **Abstracts**

Iduronate 2 Sulfatase - Pipeline Review, H1 2020

#### SUMMARY

Iduronate 2 Sulfatase (Alpha L Iduronate Sulfate Sulfatase or Idursulfase or IDS or EC 3.1.6.13) - Iduronate 2-sulfatase (IDS) is a sulfatase enzyme associated with Hunter syndrome. Iduronate 2-sulfatase is required for the lysosomal degradation of heparan sulfate and dermatan sulfate. Mutations in this X-chromosome gene that result in enzymatic deficiency lead to Hunter syndrome.

Iduronate 2 Sulfatase (Alpha L Iduronate Sulfate Sulfatase or Idursulfase or IDS or EC 3.1.6.13) pipeline Target constitutes close to 9 molecules. Out of which approximately 8 molecules are developed by companies and remaining by the universities/institutes. The molecules developed by companies in Pre-Registration, Phase III, Phase II, IND/CTA Filed and Preclinical stages are 1, 2, 2, 1 and 2 respectively. Similarly, the universities portfolio in Preclinical stages comprises 1 molecules, respectively. Report covers products from therapy areas Genetic Disorders and Central Nervous System which include indications Mucopolysaccharidosis II (MPS II) (Hunter Syndrome) and Cognitive Impairment.

The latest report Iduronate 2 Sulfatase - Pipeline Review, H1 2020, outlays comprehensive information on the Iduronate 2 Sulfatase (Alpha L Iduronate Sulfate Sulfatase or Idursulfase or IDS or EC 3.1.6.13) targeted therapeutics, complete with analysis by indications, stage of development, mechanism of action (MoA), route of administration (RoA) and molecule type. It also reviews key players involved in Iduronate 2 Sulfatase (Alpha L Iduronate Sulfate Sulfatase or Idursulfase or IDS or EC 3.1.6.13) targeted therapeutics development with respective active and dormant or discontinued projects.



The report is built using 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 Iduronate 2 Sulfatase (Alpha L Iduronate Sulfate Sulfatase or Idursulfase or IDS or EC 3.1.6.13)

The report reviews Iduronate 2 Sulfatase (Alpha L Iduronate Sulfate Sulfatase or Idursulfase or IDS or EC 3.1.6.13) 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 Iduronate 2 Sulfatase (Alpha L Iduronate Sulfate Sulfatase or Idursulfase or IDS or EC 3.1.6.13) targeted therapeutics and enlists all their major and minor projects

The report assesses Iduronate 2 Sulfatase (Alpha L Iduronate Sulfate Sulfatase or Idursulfase or IDS or EC 3.1.6.13) 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 Iduronate 2 Sulfatase (Alpha L Iduronate Sulfate Sulfatase or Idursulfase or IDS or EC 3.1.6.13) 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 Iduronate 2 Sulfatase (Alpha L Iduronate Sulfate Sulfatase or Idursulfase or IDS or EC 3.1.6.13)

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 Iduronate 2 Sulfatase (Alpha L Iduronate Sulfate Sulfatase or Idursulfase or IDS or EC 3.1.6.13) 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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GC Pharma

JCR Pharmaceuticals Co Ltd

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Featured News & Press Releases

Jan 29, 2020: REGENXBIO announces presentations at the 16th Annual

WORLDSymposium 2020

Dec 18, 2019: REGENXBIO announces interim data from phase I/II trial of RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II)



Sep 09, 2019: CANbridge Pharmaceuticals' Hunterase Granted Priority Review by the Chinese National Medical Products Administration

Jul 29, 2019: CANbridge Pharmaceuticals submits New Drug Application for Hunterase for the treatment of Hunter Syndrome in China

Jun 11, 2019: Denali Therapeutics receives orphan drug and rare pediatric disease designation for DNL310, and expands its portfolio of brain penetrant enzyme replacement programs

Apr 02, 2019: Sangamo provided an update on its in vivo genome editing program: SB-913

Feb 28, 2019: JCR completes patient enrollment in phase 2 clinical trial of JR-141 for Hunter Syndrome in Brazil

Feb 28, 2019: JCR receives EMA Orphan Designation for JR-141 in Hunter Syndrome Feb 07, 2019: Sangamo announces interim results of phase 1/2 CHAMPIONS study showing preliminary evidence of in vivo genome editing in patients with MPS II treated with SB-913

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

Jan 04, 2019: Sangamo announces upcoming clinical data Presentation on SB-913 At WORLDSymposium 2019

Dec 28, 2018: JCR completed enrollment in phase 3 clinical trial of JR-141 in Japan for Hunter syndrome

Dec 21, 2018: Notice on the publication of the phase 1/2 clinical trial results for hunter syndrome in molecular therapy

Oct 19, 2018: JCR Pharmaceuticals: Notice of Orphan Drug Designation by the US Food Drug Administration for JR-141 for Hunter Syndrome

Sep 05, 2018: Sangamo Announces 16 influential fall week clinical results including reductions in glycosaminoglycans in phase 1/2 trial evaluating SB-913, a zinc finger nuclease genome editing treatment for MPS II (Hunter Syndrome)

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#### **COMPANIES MENTIONED**

Bioasis Technologies Inc
Denali Therapeutics Inc
Esteve Pharmaceuticals SA
GC Pharma
JCR Pharmaceuticals Co Ltd
RegenxBio Inc
Sangamo Therapeutics Inc



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