

# Iduronate 2 Sulfatase (Alpha L Iduronate Sulfate Sulfatase or Idursulfase or IDS or EC 3.1.6.13) -Pipeline Review, H1 2018

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## **Abstracts**

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### SUMMARY

Iduronate 2 Sulfatase (Alpha L Iduronate Sulfate Sulfatase or Idursulfase or IDS or EC 3.1.6.13) pipeline Target constitutes close to 11 molecules. Out of which approximately 10 molecules are developed by companies and remaining by the universities/institutes. The latest report Iduronate 2 Sulfatase - Pipeline Review, H1 2018, 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.

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.

The molecules developed by companies in Phase III, Phase II, IND/CTA Filed, Preclinical and Discovery stages are 1, 4, 1, 3 and 1 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.

Furthermore, this report 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. 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 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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3.1.6.13) - Dormant Products

Iduronate 2 Sulfatase (Alpha L Iduronate Sulfate Sulfatase or Idursulfase or IDS or EC 3.1.6.13) - Product Development Milestones

Featured News & Press Releases

May 28, 2018: JCR Receives Approval in Brazil to Initiate Phase 2 Clinical Trial of JR-141 for Hunter Syndrome

May 02, 2018: REGENXBIO Receives FDA Fast Track Designation for RGX-121 Gene Therapy for the Treatment of Mucopolysaccharidosis Type II

Apr 04, 2018: Sangamo Announces Publication In Molecular Therapy Of Preclinical Study Data From MPS II In Vivo Genome Editing Program

Mar 27, 2018: JCR Announces Designation of JR-141 for Hunter Treatment under 'SAKIGAKE Designation System' by Japan's Ministry of Health, Labour and Welfare Mar 21, 2018: UNC Pediatrics Delivers Investigational Genome Editing Therapy in Clinical Trial for the Rare Hunter Syndrome

Feb 06, 2018: Sangamo Therapeutics Presents Initial Safety Data from CHAMPIONS Genome Editing Study for MPS II at WORLDSymposium

Jan 29, 2018: JCR to Present Data On JR-141 At The 14th Annual WORLD Symposium 2018

Dec 19, 2017: Shire Announces Top-Line Results for Phase II/III Clinical Trial in Children with Hunter Syndrome and Cognitive Impairment

Dec 19, 2017: REGENXBIO Announces IND Active for Phase I/II Trial of RGX-121 to Treat Mucopolysaccharidosis Type II

Dec 07, 2017: Sangamo Announces EMA Recommendation of Orphan Medicinal Product Designation for Investigational Genome Editing Treatment For MPS II

Nov 15, 2017: Sangamo Announces Treatment of First Patient in Landmark Phase 1/2 Clinical Trial Evaluating In Vivo Genome Editing for MPS II

Jul 13, 2017: Sangamo Receives Fast Track Designation From The FDA For SB-913 May 04, 2017: Sangamo Therapeutics Announces Rare Pediatric Disease Designation for SB-913 In Vivo Genome Editing Treatment for MPS II

Mar 31, 2017: JCR Announces First Patient Dosed in Phase 1/2 Clinical Trial of JR-141 in Hunter Syndrome

Mar 01, 2017: Sangamo Therapeutics Receives Orphan Drug Designation from the FDA for SB-913 Genome Editing Treatment for MPS II

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#### **COMPANIES MENTIONED**

AngioChem Inc ArmaGen Inc Denali Therapeutics Inc GC Pharma JCR Pharmaceuticals Co Ltd Laboratorios Del Dr Esteve SA RegenxBio Inc Sangamo Therapeutics Inc Shire Plc



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