

# Alpha L-Iduronidase (IDUA or EC 3.2.1.76) - Pipeline Review, H2 2017

<https://marketpublishers.com/r/A85124FCC44EN.html>

Date: December 2017

Pages: 48

Price: US\$ 3,500.00 (Single User License)

ID: A85124FCC44EN

## Abstracts

Alpha L-Iduronidase (IDUA or EC 3.2.1.76) - Pipeline Review, H2 2017

### SUMMARY

According to the recently published report 'Alpha L-Iduronidase (IDUA or EC 3.2.1.76) - Pipeline Review, H2 2017'; Alpha L-Iduronidase (IDUA or EC 3.2.1.76) pipeline Target constitutes close to 10 molecules. Out of which approximately 8 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 (IDUA or EC 3.2.1.76) - Pipeline Review, H2 2017' 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 2, 1, 4 and 1 respectively. Similarly, the universities portfolio in Preclinical and Discovery stages comprises 1 and 1 molecules,

respectively. Report covers products from therapy areas Genetic Disorders which include indications Mucopolysaccharidosis I (MPS I) (Hurler Syndrome ).

**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

## Contents

Introduction

Global Markets Direct Report Coverage

Alpha L-Iduronidase (IDUA or EC 3.2.1.76) - Overview

Alpha L-Iduronidase (IDUA or EC 3.2.1.76) - Therapeutics Development

Products under Development by Stage of Development

Products under Development by Therapy Area

Products under Development by Indication

Products under Development by Companies

Products under Development by Universities/Institutes

Alpha L-Iduronidase (IDUA or EC 3.2.1.76) - Therapeutics Assessment

Assessment by Mechanism of Action

Assessment by Route of Administration

Assessment by Molecule Type

Alpha L-Iduronidase (IDUA or EC 3.2.1.76) - Companies Involved in Therapeutics Development

AngioChem Inc

ArmaGen Inc

Immusoft Corp

JCR Pharmaceuticals Co Ltd

RegenxBio Inc

Sangamo Therapeutics Inc

Tamid Bio Inc

Alpha L-Iduronidase (IDUA or EC 3.2.1.76) - Drug Profiles

AGT-181 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Cell Therapy for Mucopolysaccharidosis I - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Cell Therapy to Activate Alpha L-Iduronidase for Hurler Syndrome - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

JR-171 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Recombinant Alpha-L-Iduronidase Replacement for Mucopolysaccharidosis I - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Recombinant Alpha-L-Iduronidase Replacement for Mucopolysaccharidosis I - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

RGX-111 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

SB-318 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Tamid-001 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

X-372 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Alpha L-Iduronidase (IDUA or EC 3.2.1.76) - Dormant Products

Alpha L-Iduronidase (IDUA or EC 3.2.1.76) - Product Development Milestones

Featured News & Press Releases

Dec 08, 2017: Sangamo Announces EMA Recommendation of Orphan Medicinal Product Designation for Investigational Genome Editing Treatment for Mucopolysaccharidosis Type I

Dec 05, 2017: Fortress Biotech Forms Subsidiary Tamid Bio to Develop Novel AAV Gene Therapies in Orphan Diseases With Unmet Medical Needs

Nov 30, 2017: ArmaGen's AGT-181 Granted Fast Track Designation for the Treatment of Hurler Syndrome (MPS I)

Oct 26, 2017: JCR to Initiate Development of JR-171, a New Drug Candidate for Hurler

Syndrome Using J-Brain Cargo

Aug 08, 2017: REGENXBIO Announces IND Active for Phase I Trial of RGX-111 to Treat Mucopolysaccharidosis Type I

Jul 13, 2017: Sangamo Receives Fast Track Designation From The FDA For SB-318 In Vivo Genome Editing Product Candidate For The Treatment Of MPS I

Feb 27, 2017: Sangamo Therapeutics Receives Rare Pediatric Disease Designation From FDA For SB-318 In Vivo Genome Editing Therapeutic For MPS I

Feb 16, 2017: ArmaGen Reports Preliminary Evidence of Cognitive Improvement in Children with Hurler Syndrome (MPS I) Treated with AGT-181

Feb 08, 2017: Sangamo Therapeutics Announces Data on SB-318 at The 13th Annual WORLDSymposium Meeting

Feb 07, 2017: ArmaGen Announces Oral Presentation of Preliminary Results from its Phase 2 Clinical Trial of AGT-181 in Patients with MPS 1 to be Presented at WORLDSymposium 2017

Jan 11, 2017: Sangamo Therapeutics Receives Orphan Drug Designation from the FDA for SB-318 Genome Editing Treatment for MPS I

Sep 13, 2016: REGENXBIO Publishes Data from Ongoing Preclinical Studies of NAV Gene Therapy RGX-111

Jul 05, 2016: REGENXBIO Provides Update On Gene Therapy Development Program RGX-111

May 09, 2016: Sangamo BioSciences Presents Recent Developments From Research And ZFP Therapeutic Programs In Multiple Presentations At Annual Meeting of the American Society of Gene and Cell Therapy

Mar 31, 2016: ArmaGen Announces Initiation of Phase 2 Proof-of-Concept Clinical Trial in Brazil to Study AGT-181 for the Treatment of Hurler Syndrome

Appendix

Methodology

Coverage

Secondary Research

Primary Research

Expert Panel Validation

Contact Us

Disclaimer

## List Of Tables

### LIST OF TABLES

Number of Products under Development by Stage of Development, H2 2017  
Number of Products under Development by Therapy Areas, H2 2017  
Number of Products under Development by Indication, H2 2017  
Number of Products under Development by Companies, H2 2017  
Products under Development by Companies, H2 2017  
Number of Products under Investigation by Universities/Institutes, H2 2017  
Products under Investigation by Universities/Institutes, H2 2017  
Number of Products by Stage and Mechanism of Actions, H2 2017  
Number of Products by Stage and Route of Administration, H2 2017  
Number of Products by Stage and Molecule Type, H2 2017  
Pipeline by AngioChem Inc, H2 2017  
Pipeline by ArmaGen Inc, H2 2017  
Pipeline by Immusoft Corp, H2 2017  
Pipeline by JCR Pharmaceuticals Co Ltd, H2 2017  
Pipeline by RegenxBio Inc, H2 2017  
Pipeline by Sangamo Therapeutics Inc, H2 2017  
Pipeline by Tamid Bio Inc, H2 2017  
Dormant Projects, H2 2017

## List Of Figures

### LIST OF FIGURES

Number of Products under Development by Stage of Development, H2 2017

Number of Products by Mechanism of Actions, H2 2017

Number of Products by Stage and Mechanism of Actions, H2 2017

Number of Products by Routes of Administration, H2 2017

Number of Products by Stage and Routes of Administration, H2 2017

Number of Products by Molecule Types, H2 2017

Number of Products by Stage and Molecule Types, H2 2017

### COMPANIES MENTIONED

AngioChem Inc

ArmaGen Inc

Immusoft Corp

JCR Pharmaceuticals Co Ltd

RegenxBio Inc

Sangamo Therapeutics Inc

Tamid Bio Inc



## I would like to order

Product name: Alpha L-Iduronidase (IDUA or EC 3.2.1.76) - Pipeline Review, H2 2017

Product link: <https://marketpublishers.com/r/A85124FCC44EN.html>

Price: US\$ 3,500.00 (Single User License / Electronic Delivery)

If you want to order Corporate License or Hard Copy, please, contact our Customer Service:

[info@marketpublishers.com](mailto:info@marketpublishers.com)

## Payment

To pay by Credit Card (Visa, MasterCard, American Express, PayPal), please, click button on product page <https://marketpublishers.com/r/A85124FCC44EN.html>

To pay by Wire Transfer, please, fill in your contact details in the form below:

First name:  
Last name:  
Email:  
Company:  
Address:  
City:  
Zip code:  
Country:  
Tel:  
Fax:  
Your message:

**\*\*All fields are required**

Customer signature \_\_\_\_\_

Please, note that by ordering from marketpublishers.com you are agreeing to our Terms & Conditions at <https://marketpublishers.com/docs/terms.html>

To place an order via fax simply print this form, fill in the information below and fax the completed form to +44 20 7900 3970