

# **Alpha N-Acetylglucosaminidase (N Acetyl Alpha Glucosaminidase or NAGLU or EC 3.2.1.50) - Pipeline Review, H2 2017**

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## **Abstracts**

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### **Summary**

Alpha N-Acetylglucosaminidase (N Acetyl Alpha Glucosaminidase or NAGLU or EC 3.2.1.50) pipeline Target constitutes close to 5 molecules. Out of which approximately 5 molecules are developed by Companies. The latest report Alpha N-Acetylglucosaminidase - Pipeline Review, H2 2017, outlays comprehensive information on the Alpha N-Acetylglucosaminidase (N Acetyl Alpha Glucosaminidase or NAGLU or EC 3.2.1.50) targeted therapeutics, complete with analysis by indications, stage of development, mechanism of action (MoA), route of administration (RoA) and molecule type.

Alpha N-Acetylglucosaminidase (N Acetyl Alpha Glucosaminidase or NAGLU or EC 3.2.1.50) - Alpha-N-acetylglucosaminidase is a protein associated with Sanfilippo syndrome. This enzyme is located in lysosomes compartments within cells that digest and recycle different types of molecules. Alpha-N-acetylglucosaminidase is involved in the step wise breakdown of large molecules called glycosaminoglycans (GAGs). Alpha-N-acetylglucosaminidase removes a sugar called N-acetylglucosamine when it is at the end of the GAG chain. The molecules developed by companies in Phase II, IND/CTA Filed, Preclinical and Discovery stages are 2, 1, 1 and 1 respectively. Report covers products from therapy areas Genetic Disorders which include indications Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome ).

Furthermore, this report also reviews key players involved in Alpha N-Acetylglucosaminidase (N Acetyl Alpha Glucosaminidase or NAGLU or EC 3.2.1.50) 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 N-Acetylglucosaminidase (N Acetyl Alpha Glucosaminidase or NAGLU or EC 3.2.1.50)

The report reviews Alpha N-Acetylglucosaminidase (N Acetyl Alpha Glucosaminidase or NAGLU or EC 3.2.1.50) 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 N-Acetylglucosaminidase (N Acetyl Alpha Glucosaminidase or NAGLU or EC 3.2.1.50) targeted therapeutics and enlists all their major and minor projects

The report assesses Alpha N-Acetylglucosaminidase (N Acetyl Alpha Glucosaminidase or NAGLU or EC 3.2.1.50) 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 N-Acetylglucosaminidase (N Acetyl Alpha Glucosaminidase or NAGLU or EC 3.2.1.50) 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 N-Acetylglucosaminidase (N Acetyl Alpha Glucosaminidase or NAGLU or EC 3.2.1.50)

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 N-Acetylglucosaminidase (N Acetyl Alpha Glucosaminidase or NAGLU or EC 3.2.1.50) 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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Featured News & Press Releases

Jan 19, 2017: Abeona Therapeutics Receives Orphan Drug Designation in The European Union for ABO-101 Gene Therapy in Sanfilippo Syndrome Type B

Jul 14, 2016: Alexion Presents New SBC-103 (rhNAGLU enzyme) Phase 1/2 Data on Brain MRI and Neurocognitive Assessments in Patients with Mucopolysaccharidosis IIIB (MPS IIIB)

May 24, 2016: Abeona Therapeutics Announces FDA Allowance of Investigational New Drug for Phase 1/2 Clinical Study With ABO-101 Gene Therapy for Patients With Sanfilippo Syndrome Type B (MPS IIIB)

Apr 21, 2016: BioMarin Enrolls First Patient in Phase 1/2 Trial of NAGLU Fusion Protein BMN 250 for Treatment of MPS IIIB (Sanfilippo B Syndrome)

Mar 01, 2016: SBC-103 (rhNAGLU enzyme) Shows a 26.2 Percent Mean Reduction in Heparan Sulfate in Cerebrospinal Fluid at the Highest Dose Studied in Patients with Mucopolysaccharidosis IIIB in Phase 1/2 Study at Six Months

Feb 25, 2016: ESTEVE Provides Update On EGT-201 For Sanfilippo B Syndrome

Jan 11, 2016: Abeona Therapeutics Announces Initial European Regulatory Approval for Phase 1/2 Gene Therapy Clinical Study for Patients With Sanfilippo Syndrome

Jun 04, 2015: Synageva BioPharma Completes Targeted Enrollment in Phase 1/2 Trial with SBC-103 for Mucopolysaccharidosis IIIB

May 20, 2015: PlasmaTech Biopharmaceuticals Announces Orphan Drug and Rare Pediatric Disease Designations For ABX-101 From FDA

Jan 26, 2015: Synageva BioPharma Announces Dosing Of Patients Commenced With SBC-103 In Phase 1/2 Study For Mucopolysaccharidosis IIIB And FDA Fast Track Designation Granted

Dec 15, 2014: Synageva BioPharma Announces Active Investigational New Drug Application For SBC-103 For The Treatment Of Mucopolysaccharidosis IIIB

Dec 10, 2014: FDA Grants BioMarin Orphan Drug Designation for NAGLU Fusion Protein, BMN 250, for the Treatment of MPS IIIB (Sanfilippo Syndrome Type B)

Feb 12, 2014: Synageva BioPharma Highlights SBC-103 Data at the Lysosomal

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Feb 11, 2014: BioMarin Announces Selection of NAGLU Fusion Protein Drug  
Development Candidate BMN 250 for the Treatment of Sanfilippo B

Jan 29, 2014: Synageva BioPharma Announces Data Presentations On SBC-103 at the  
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