

# Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) - Pipeline Review, H1 2018

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# **Abstracts**

Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) - Pipeline Review, H1 2018

#### SUMMARY

Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) pipeline Target constitutes close to 14 molecules. Out of which approximately 14 molecules are developed by Companies.

The latest report GrowthDifferentiation Factor 8 (Myostatin or GDF8 or MSTN) - Pipeline Review, H1 2018, outlays comprehensive information on the Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) targeted therapeutics, complete with analysis by indications, stage of development, mechanism of action (MoA), route of administration (RoA) and molecule type.

Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) - Myostatin also known as growth differentiation factor 8 or GDF-8 is a myokine a protein produced and released by myocytes. This protein is part of the transforming growth factor beta (TGF?) superfamily. Myostatin is found almost in muscles used for movement, where it is active both before and after birth. This protein normally restrains muscle growth, ensuring that muscles do not grow too large.

The molecules developed by companies in Phase III, Phase II, Phase I and Preclinical stages are 2, 4, 1 and 7 respectively. Report covers products from therapy areas Genetic Disorders, Musculoskeletal Disorders, Central Nervous System, Gastrointestinal, Hematological Disorders, Metabolic Disorders and Oncology which



include indications Duchenne Muscular Dystrophy, Amyotrophic Lateral Sclerosis, Muscular Dystrophy, Sarcopenia, Anemia, Becker Muscular Dystrophy, Charcot-Marie-Tooth Disease, Charcot-Marie-Tooth Disease Type I, Crohn's Disease (Regional Enteritis), Fibrodysplasia Ossificans Progressiva (Myositis Ossificans Progressiva), Fibrosis, Insulin Resistance, Myelodysplastic Syndrome, Myelofibrosis, Sickle Cell Disease, Spinal Muscular Atrophy (SMA) and Thalassemia.

Furthermore, this report also reviews key players involved in Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) 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 Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN)

The report reviews Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) 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 Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) targeted therapeutics and enlists all their major and minor projects

The report assesses Growth/Differentiation Factor 8 (Myostatin or GDF8 or



MSTN) 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 Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) 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 Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN)

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 Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) 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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Acceleron Pharma Inc

Amgen Inc

**Bioleaders Corp** 

F. Hoffmann-La Roche Ltd

PeptiDream Inc

Pfizer Inc

Regeneron Pharmaceuticals Inc

Sarepta Therapeutics Inc

Scholar Rock Inc

Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) - Drug Profiles

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Mechanism Of Action

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Mechanism Of Action

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Featured News & Press Releases

Feb 28, 2018: Acceleron Announces Publication of ACE-083 Phase 1 Trial Results in Muscle & Nerve

Feb 08, 2018: Acceleron Announces Appointment of Chief Commercial Officer

Feb 02, 2018: Scholar Rock Announces Publication of Comprehensive Pharmacological Study of SRK-015, a Novel Inhibitor of Myostatin Activation, Supporting the Treatment of Muscle Atrophy Disorders

Jan 23, 2018: Scholar Rock and Collaborators at Harvard Medical School and Northeastern University Elucidate Molecular Basis of Myostatin Activation - a Key Physiological Process in Muscle Health

Jan 08, 2018: Acceleron Announces Preliminary Results from Part 1 of the ACE-083 Phase 2 Trial in Patients with Facioscapulohumeral Dystrophy

Dec 10, 2017: Acceleron Announces Updated Results from Ongoing Phase 2 Trials of Luspatercept in Myelodysplastic Syndromes at the 59th Annual Meeting of the American Society of Hematology

Nov 01, 2017: Acceleron to Present Clinical Presentations on Investigational Drug Candidate Luspatercept at 59th American Society of Hematology Annual Meeting and Exposition

Sep 19, 2017: Acceleron Provides Clinical Update on Luspatercept at 2017 R&D Day

Sep 19, 2017: Acceleron Provides Clinical Update on Muscular Dystrophy Drug

Candidate ACE-083 at 2017 R&D Day

Sep 19, 2017: Acceleron Provides Update on Clinical Development Plans for ACE-2494 at 2017 R&D Day

Sep 14, 2017: Scholar Rock Announces Issuance of Broad Patent Covering Therapeutic Approach for Neuromuscular Diseases

Sep 05, 2017: Acceleron Announces Publication of Luspatercept Phase 2

Myelodysplastic Syndromes Study Results in The Lancet Oncology

Aug 01, 2017: Acceleron Announces First Patient Treated in Phase 2 Clinical Trial of ACE-083 in Charcot-Marie-Tooth Disease



Jul 10, 2017: Scholar Rock and the Miami Project to Cure Paralysis Present Preclinical Data Demonstrating Therapeutic Potential of SRK-015 in Spinal Cord Injury at 35th Annual National Neurotrauma Symposium

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#### **COMPANIES MENTIONED**

Acceleron Pharma Inc

Amgen Inc

**Bioleaders Corp** 

F. Hoffmann-La Roche Ltd

PeptiDream Inc

Pfizer Inc

Regeneron Pharmaceuticals Inc

Sarepta Therapeutics Inc

Scholar Rock Inc.



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