

# GrowthDifferentiation Factor 8 - Pipeline Review, H2 2019

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## Abstracts

GrowthDifferentiation Factor 8 - Pipeline Review, H2 2019

#### SUMMARY

According to the recently published report 'GrowthDifferentiation Factor 8 - Pipeline Review, H2 2019'; Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) pipeline Target constitutes close to 10 molecules.

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 report 'GrowthDifferentiation Factor 8 - Pipeline Review, H2 2019' 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; that are being developed by Companies / Universities.

It 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. Currently, The molecules developed by companies in Pre-Registration, Phase II, Phase I and Preclinical stages are 1, 4, 2 and 3 respectively. Report covers products from therapy areas Central Nervous System, Genetic Disorders,



Musculoskeletal Disorders, Hematological Disorders and Oncology which include indications Amyotrophic Lateral Sclerosis, Spinal Muscular Atrophy (SMA), Duchenne Muscular Dystrophy, Anemia, Charcot-Marie-Tooth Disease, Fibrodysplasia Ossificans Progressiva (Myositis Ossificans Progressiva), Limb-Girdle Muscular Dystrophy, Muscle Wasting Disorders, Myelodysplastic Syndrome, Myelofibrosis, Neuro Muscular Disorders, Post-Essential Thrombocythemia Myelofibrosis (Post-ET MF), Post-Polycythemia Vera Myelofibrosis (PPV-MF) and Thalassemia.

#### 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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R&D Progress



- domagrozumab Drug Profile
- Product Description
- Mechanism Of Action
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- Dec 09, 2019: Reblozyl (luspatercept-aamt) studies evaluating treatment of Anemia in rare blood diseases presented at American Society of Hematology (ASH) annual meeting
- Dec 06, 2019: Bristol-Myers Squibb and Acceleron Pharma provide update on FDA Advisory Committee for Reblozyl (luspatercept-aamt)
- Dec 03, 2019: Bristol-Myers Squibb and Acceleron Pharma announce FDA Advisory



Committee will review Reblozyl (luspatercept-aamt) for Use in patients with myelodysplastic syndromes

Nov 19, 2019: Scholar Rock reports preliminary pharmacokinetic and pharmacodynamic data from TOPAZ phase 2 trial of SRK-015 for the treatment of patients with spinal muscular atrophy

Nov 11, 2019: Celgene-Acceleron's Reblozyl gets US approval to treat anaemia Nov 08, 2019: FDA approves REBLOZYL (luspatercept-aamt) for the treatment of anemia in adults with beta thalassemia who require regular red blood cell transfusions Nov 06, 2019: Celgene to present abstracts on Luspatercept at American Society of Hematology (ASH) 2019 Annual Meeting

Nov 06, 2019: Acceleron announces Luspatercept presentations at the 61st American Society of Hematology Annual Meeting

Oct 02, 2019: Scholar Rock to Present Preclinical and Phase 1 Clinical Data on SRK-015 at the World Muscle Society Congress

Sep 16, 2019: Acceleron announces topline results from the phase 2 trial of ACE-083 in patients with Facioscapulohumeral Muscular Dystrophy

Sep 05, 2019: Scholar Rock announces appointment of George Nomikos, M.D., Ph.D., as Vice President, Head of Medical Research, Muscle Franchise

Jun 06, 2019: FDA agrees to review Celgene-Acceleron's luspatercept in anaemia Jun 03, 2019: Scholar Rock announces positive final results from Phase 1 clinical trial of SRK-015 in healthy volunteers

May 09, 2019: Scholar Rock starts patient dosing in Phase II study of SRK-015 in SMA May 08, 2019: Scholar Rock announces initiation of patient dosing in Phase 2 trial of SRK-015 in Spinal Muscular Atrophy

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#### **COMPANIES MENTIONED**

Acceleron Pharma Inc Biogen Inc Bioleaders Corp Chugai Pharmaceutical Co Ltd PeptiDream Inc Pfizer Inc Regeneron Pharmaceuticals Inc Scholar Rock Inc



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