

# Growth/Differentiation Factor 8 - Pipeline Review, H2 2020

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## Abstracts

Growth/Differentiation Factor 8 - Pipeline Review, H2 2020

### SUMMARY

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.

Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) pipeline Target constitutes close to 9 molecules. The molecules developed by companies in Phase III, Phase II, Phase I and Preclinical stages are 1, 3, 3 and 2 respectively. Report covers products from therapy areas Central Nervous System, Musculoskeletal Disorders, Genetic Disorders, Hematological Disorders and Oncology which include indications Spinal Muscular Atrophy (SMA), Amyotrophic Lateral Sclerosis, Duchenne Muscular Dystrophy, Anemia, Fibrodysplasia Ossificans Progressiva (Myositis Ossificans Progressiva), Limb-Girdle Muscular Dystrophy, Muscle Wasting Disorders, Myelodysplastic Syndrome, Myelofibrosis, Neuromuscular Disorders, Post-Essential Thrombocythemia Myelofibrosis (Post-ET MF), Post-Polycythemia Vera Myelofibrosis (PPV-MF), Sarcopenia and Thalassemia.

The latest report Growth/Differentiation Factor 8 - Pipeline Review, H2 2020, 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. 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.

The report is built using 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

## Contents

List of Tables

List of Figures

Introduction

Global Markets Direct Report Coverage

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

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

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

Assessment

Assessment by Mechanism of Action

Assessment by Route of Administration

Assessment by Molecule Type

Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) - Companies Involved in  
Therapeutics Development

Accelaron Pharma Inc

Biogen Inc

Bioleaders Corp

Chugai Pharmaceutical Co Ltd

PeptiDream Inc

Pfizer Inc

Regeneron Pharmaceuticals Inc

Scholar Rock Inc

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

ALG-802 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

apitegromab - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

BIIB-110 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

BLSM-22 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

domagrozumab - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Iuspatercept - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

RG-70240 - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Synthetic Peptide to Inhibit Myostatin for Duchenne Muscular Dystrophy - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

trevogrumab - Drug Profile

Product Description

Mechanism Of Action

R&D Progress

Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) - Dormant Products

Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) - Discontinued Products

Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) - Product Development  
Milestones

Featured News & Press Releases

Sep 30, 2020: Scholar Rock presents data for SRK-015 at the World Muscle Society  
2020 Virtual Congress

Sep 29, 2020: Health Canada approves REBLOZYL (Iuspatercept), new class of  
treatment for adult patients living with beta thalassemia

Aug 27, 2020: Scholar Rock announces issuance of U.S. patent providing composition  
of matter protection for SRK-015

Aug 13, 2020: Scholar Rock announces that SRK-015 has received rare pediatric  
disease designation from U.S. FDA for the treatment of spinal muscular atrophy

Jun 26, 2020: European Commission approves Reblozyl (luspatercept) for the treatment of transfusion-dependent anemia in adult patients with myelodysplastic syndromes or beta thalassemia

Jun 12, 2020: Scholar Rock presents clinical and preclinical data for SRK-015 at the 2020 Virtual Cure SMA Research and Clinical Care Meeting

May 20, 2020: Acceleron announces presentations on REBLOZYL (luspatercept-aamt) at the 2020 American Society of Clinical Oncology and European Hematology Association Virtual Annual Meetings

May 18, 2020: Scholar Rock presents baseline demographics data from the SRK-015 TOPAZ trial at the American Academy of Neurology Conference

Apr 30, 2020: Novel drug shows promise for inherited blood disorder

Apr 30, 2020: Reblozyl (luspatercept) receives positive CHMP opinion for the treatment of adults with Anemia in Beta Thalassemia and Myelodysplastic Syndromes

Apr 03, 2020: U.S. Food and Drug Administration (FDA) approves Reblozyl (luspatercept-aamt), the first and only erythroid maturation agent, to treat anemia in adults with lower-risk myelodysplastic syndromes (MDS)

Mar 26, 2020: New England Journal of Medicine Publishes results from Pivotal Phase 3 BELIEVE Trial of Reblozyl (luspatercept-aamt) in Adult Patients With Beta Thalassemia

Mar 24, 2020: Scholar Rock presents data for SRK-015 at the Muscular Dystrophy Association Clinical and Scientific Conference

Feb 06, 2020: Scholar Rock presents data for SRK-015 at the SMA Europe 2nd International Scientific Congress

Jan 09, 2020: New England journal of medicine publishes results of pivotal phase 3 Reblozyl (luspatercept-aamt) MEDALIST trial

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 2020  
Number of Products under Development by Therapy Areas, H2 2020  
Number of Products under Development by Indication, H2 2020  
Number of Products under Development by Companies, H2 2020  
Products under Development by Companies, H2 2020  
Products under Development by Companies, H2 2020 (Contd..1), H2 2020  
Number of Products by Stage and Mechanism of Actions, H2 2020  
Number of Products by Stage and Route of Administration, H2 2020  
Number of Products by Stage and Molecule Type, H2 2020  
Pipeline by Acceleron Pharma Inc, H2 2020  
Pipeline by Biogen Inc, H2 2020  
Pipeline by Bioleaders Corp, H2 2020  
Pipeline by Chugai Pharmaceutical Co Ltd, H2 2020  
Pipeline by PeptiDream Inc, H2 2020  
Pipeline by Pfizer Inc, H2 2020  
Pipeline by Regeneron Pharmaceuticals Inc, H2 2020  
Pipeline by Scholar Rock Inc, H2 2020  
Dormant Products, H2 2020  
Dormant Products, H2 2020 (Contd..1), H2 2020  
Discontinued Products, H2 2020

## List Of Figures

### LIST OF FIGURES

Number of Products under Development by Stage of Development, H2 2020

Number of Products under Development by Therapy Areas, H2 2020

Number of Products under Development by Top 10 Indications, H2 2020

Number of Products by Stage and Mechanism of Actions, H2 2020

Number of Products by Routes of Administration, H2 2020

Number of Products by Stage and Routes of Administration, H2 2020

Number of Products by Molecule Types, H2 2020

Number of Products by Stage and Molecule Types, H2 2020



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