

# Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) Drugs in Development by Therapy Areas and Indications, Stages, MoA, RoA, Molecule Type and Key Players, 2022 Update

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# **Abstracts**

Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) Drugs in Development by Therapy Areas and Indications, Stages, MoA, RoA, Molecule Type and Key Players, 2022 Update

## **SUMMARY**

Growth/Differentiation Factor 8 (Myostatin or GDF8 or MSTN) pipeline Target constitutes close to 10 molecules, which are developed by Companies. The latest report Growth Differentiation Factor 8 - Drugs In Development, 2022, 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 Pre-Registration, Phase III, Phase I and Preclinical stages are 1, 3, 2, 2 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), Duchenne Muscular



Dystrophy, Alpha Thalassaemia, Amyotrophic Lateral Sclerosis, Anemia, Becker Muscular Dystrophy, Beta Thalassaemia, 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) and Sarcopenia.

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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Milestones

Featured News & Press Releases

Jul 08, 2022: Biohaven enrols first subject in Phase III spinal muscle atrophy trial

Jul 01, 2022: Scholar Rock to present phase 3 SAPPHIRE trial design at the 17th

International Congress on Neuromuscular Diseases (ICNMD 2022)

Jun 17, 2022: Positive phase 2 Topaz trial extension data demonstrate sizable and



sustained motor function improvement at 24 months with apitegromab for non-ambulatory patients with types 2 and 3 spinal muscular atrophy (SMA)

Jun 09, 2022: Scholar Rock to Present New Apitegromab Data Including 24-Month Efficacy and Safety Data from TOPAZ Phase 2 Trial at the 2022 Annual Cure SMA Conference

Jun 03, 2022: Bristol Myers Squibb withdraws supplemental biologics license application (sBLA) for Reblozyl (luspatercept-aamt) for non-transfusion dependent (NTD) beta thalassemia

Apr 21, 2022: Scholar Rock presents data analysis of multiple efficacy endpoints from the Apitegromab TOPAZ Phase 2 Trial at the 2022 European Paediatric Neurology Society Congress

Mar 25, 2022: Bristol Myers Squibb announces new Prescription Drug User Fee Act goal date for Reblozyl (luspatercept-aamt) supplemental biologics license application Mar 22, 2022: Scholar Rock presents data analysis of multiple efficacy endpoints from the apitegromab TOPAZ phase 2 trial at the American Academy of Neurology 2022 Annual Meeting

Mar 13, 2022: Scholar Rock to present data from TOPAZ Ambulatory Cohort Analysis at the 2022 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference Dec 03, 2021: U.S. Food and Drug Administration accepts for priority review supplemental biologics license application for Reblozyl (luspatercept-aamt) in adults with non-transfusion dependent (NTD) beta thalassemia

Nov 30, 2021: Scholar Rock announces design of phase 3 SAPPHIRE clinical trial evaluating apitegromab in non-ambulatory patients with Type 2 and Type 3 spinal muscular atrophy (SMA)

Oct 03, 2021: Scholar Rock to present Apitegromab TOPAZ phase 2 pharmacologic data at the 2021 World Congress of Neurology

Sep 30, 2021: Scholar Rock presents exploratory responder analysis on efficacy data from the apitegromab TOPAZ phase 2 trial at the Child Neurology Society Annual Meeting

Sep 23, 2021: Scholar Rock presents additional data analyses from the Apitegromab TOPAZ phase 2 trial at the World Muscle Society 2021 Virtual Congress

Sep 07, 2021: Therapeutic Goods Administration provides update on Reblozyl

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