

Mucopolysaccharidosis

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Mucopolysaccharidosis is a group of autosomal recessive metabolic disorders caused by the absence or malfunctioning of lysosomal enzymes needed to break down molecules called glycosaminoglycans - long chains of sugar carbohydrates in each of our cells that help build bone, cartilage, tendons, corneas, skin and connective tissue. Glycosaminoglycans are also found in the fluid that lubricates our joints. People with a mucopolysaccharidosis either do not produce enough of one of the 11 enzymes required to break down these sugar chains into proteins and simpler molecules, or they produce enzymes that do not work properly. Over time, these glycosaminoglycans collect in the cells, blood and connective tissues. The result is permanent, progressive cellular damage which affects appearance, physical abilities, organ and system functioning, and, in most cases, mental development. The mucopolysaccharidoses are classified as lysosomal storage diseases. These are conditions in which large numbers of molecules that are normally broken down or degraded into smaller pieces by intracellular units called lysosomes accumulate in harmful amounts in the body's cells and tissues, particularly in the lysosomes. ([From the Wikipedia article Mucopolysaccharidosis](#).)

Recent Publications on Mucopolysaccharidosis:



- [Reprogramming erythroid cells for lysosomal enzyme production leads to visceral and CNS cross-correction in mice with Hurler syndrome.](#)
Restricting transgene expression to maturing erythroid cells can reduce... 12th November, 2009
Molecular and Gene Therapy Program, Division of Experimental Hematology- Proc Natl Acad Sci U S A. 2009 Nov 10. ([DOI Direct Link](#))
- [Neonatal intravenous injection of a gammaretroviral vector has a low incidence of tumor induction in mice.](#)
Neonatal intravenous injection of gammaretroviral vectors (gamma-RVs) with... 11th November, 2009
Department of Internal Medicine, Washington University School of Medicine,- Hum Gene Ther. 2008 Nov;19(11):1317-23.
- [Repeated intrathecal injections of recombinant human 4-sulphatase remove dural storage in mature mucopolysaccharidosis VI cats primed with a short-course tolerisation regimen.](#)
All MPS-VI cats treated thus far with weekly intravenous enzyme... 10th November, 2009
Lysosomal Diseases Research Unit, SA Pathology (at Women's and Children's- Mol Genet Metab. 2009 Oct 13. ([DOI Direct Link](#))
- [\[Difficult tracheal intubation using airway scope in a pediatric patient with Hunter syndrome\]](#)
Hunter syndrome, manifested by mucopolysaccharidosis II (MPS II), is a... 29th October, 2009
Department of Anesthesiology and Critical Care, Hiroshima University- Masui. 2009 Oct;58(10):1278-81.
- [\[Mucopolysaccharidosis: 20-year enzymatic diagnosis in Cuba\]](#)
INTRODUCTION: Mucopolysaccharidoses (MPS), which belong to the family of... 28th October, 2009

Instituto de Neurologia y Neurocirugia. Calle 29 y D. Vedado. CP 10400. La- Rev Neurol. 2009 Nov 1-15;49(9):458-62.

- [Joint contractures in the absence of inflammation may indicate mucopolysaccharidosis.](#)
*ABSTRACT: BACKGROUND: Undiagnosed patients with the attenuated form of...*27th October, 2009
Ospedale Meyer-Reumatologia,- *Pediatr Rheumatol Online J.* 2009 Oct 23;7:18. ([DOI Direct Link](#))
- [A systematic review of new advances in the management of mucopolysaccharidosis VI \(Maroteaux-Lamy syndrome\): focus on galsulfase.](#)
*INTRODUCTION: Mucopolysaccharidosis type VI (MPS VI, Maroteaux-Lamy...*24th October, 2009
Department of Surgery, McMaster University, McMaster Institute of Urology,- *Biologics.* 2009;3:459-68.
Epub 2009 Oct 12.
- [Introduction to Brazilian Guidelines to Diagnosis, Treatment, and Monitoring for Gaucher Disease, Fabry Disease, Mucopolysaccharidosis I, and Pompe Disease.](#)
*INTRODUCTION: Dyggve-Melchior-Clausen (DMC) syndrome is a rare autosomal...*23rd October, 2009
Universidade Federal de Sao Paulo, Sao Paulo, Brazil- *J Pediatr.* 2009 Oct;155(4 Suppl):S9.
- [A Self-inactivating gamma-Retroviral Vector Reduces Manifestations of Mucopolysaccharidosis I in Mice.](#)
*Mucopolysaccharidosis I (MPS I) is a lysosomal storage disease due to...*22nd October, 2009
Department of Medicine, Washington University School of Medicine, St- *Mol Ther.* 2009 Oct 20. ([DOI Direct Link](#))
- [Mutational Analysis of the alpha-L-Iduronidase Gene in Three Egyptian Families: Identification of Three Novel Mutations and Five Novel Polymorphisms.](#)
*Mucopolysaccharidosis type I (MPS I) is an autosomal recessive disorder...*21st October, 2009
1 Department of Medical Molecular Genetics, National Research Centre ,- *Genet Test Mol Biomarkers.* 2009 Oct 19. ([DOI Direct Link](#))
- [Glycosaminoglycan-mediated loss of cathepsin K collagenolytic activity in MPS I contributes to osteoclast and growth plate abnormalities.](#)
*Mucopolysaccharidoses are a group of lysosomal storage diseases...*17th October, 2009
University of British Columbia, Vancouver, Canada.- *Am J Pathol.* 2009 Nov;175(5):2053-62. Epub 2009 Oct 15. ([DOI Direct Link](#))
- [Genetic engineering of IgG-glucuronidase fusion proteins.](#)
*beta-Glucuronidase (GUSB) is a lysosomal enzyme that could be developed as...*16th October, 2009
ArmaGen Technologies, Inc., Santa Monica, CA, USA.- *J Drug Target.* 2009 Oct 14. ([DOI Direct Link](#))
- [Protein misfolding as an underlying molecular defect in mucopolysaccharidosis III type C.](#)
*Mucopolysaccharidosis type IIIC or Sanfilippo syndrome type C (MPS IIIC,...*14th October, 2009
Department of Medical Genetics, CHU Sainte-Justine University of Montreal,- *PLoS One.* 2009 Oct 13;4(10):e7434. ([DOI Direct Link](#))
- [Clinical and biochemical studies in mucopolysaccharidosis type II carriers.](#)
*The aim of the study was to characterize clinically and biochemically...*13th October, 2009
Department of Genetics,- *J Inherit Metab Dis.* 2009 Oct 10. ([DOI Direct Link](#))
- [Dyggve-Melchior-Clausen syndrome: clinical, genetic, and radiological study of 15 Egyptian patients from nine unrelated families.](#)
*INTRODUCTION: Dyggve-Melchior-Clausen (DMC) syndrome is a rare autosomal...*10th October, 2009
Human Genetics and Genome Research Division, Clinical Genetics Department,- *J Child Orthop.* 2009 Oct 9. ([DOI Direct Link](#))

BioNews Results for Mucopolysaccharidosis

- [Stainless steel, foreign particles found in variety of drugs](#)
New York Daily News: Nov 14 2009 1:27AM Matching: mucopolysaccharidosis
- [U.S. FDA: Some Genzyme drugs contaminated](#)
Reuters Health: Nov 14 2009 1:20AM Matching: mucopolysaccharidosis
- [FDA Warns of Contamination in Drugs for Rare Illnesses](#)
MedPage Today: Nov 14 2009 12:29AM Matching: mucopolysaccharidosis
- [REFILE-U.S. FDA: Some Genzyme drugs contaminated](#)
Forbes.com: Nov 13 2009 8:54PM Matching: mucopolysaccharidosis
- [REFILE-U.S. FDA: Some Genzyme drugs contaminated](#)
CNN Money: Nov 13 2009 8:19PM Matching: mucopolysaccharidosis
- [Stainless steel, foreign particles found in popular drugs like Cerezyme, others](#)
New York Daily News: Nov 13 2009 10:13PM Matching: mucopolysaccharidosis
- [REFILE-UPDATE 3-U.S. FDA: Some Genzyme drugs contaminated](#)
Forbes.com: Nov 13 2009 8:37PM Matching: mucopolysaccharidosis
- [REFILE-UPDATE 3-U.S. FDA: Some Genzyme drugs contaminated](#)
FinanzNachrichten.de: Nov 13 2009 8:34PM Matching: mucopolysaccharidosis
- [REFILE-UPDATE 3-U.S. FDA: Some Genzyme drugs contaminated](#)
Interactive Investor International: Nov 13 2009 8:24PM Matching: mucopolysaccharidosis
- [REFILE-UPDATE 3-U.S. FDA: Some Genzyme drugs contaminated](#)
Reuters: Nov 13 2009 8:15PM Matching: mucopolysaccharidosis
- [REFILE-UPDATE 3-U.S. FDA: Some Genzyme drugs contaminated](#)
Reuters UK: Nov 13 2009 8:15PM Matching: mucopolysaccharidosis

Mucopolysaccharidosis Clinical Trials:



- [Intrathecal Enzyme Replacement Therapy for Spinal Cord Compression in Mucopolysaccharidosis \(MPS\) I](#)

Mucopolysaccharidosis Patents:



- 7365172- [Loop peptide and TGF.alpha. for stimulating stem cell proliferation and migration](#)
- 7411051- [Antibodies to HDPPA04 polypeptide](#)
- 7419991- [3-\[5-\(2-fluoro-phenyl\)-\[1,2,4\]oxadiazol-3-yl\]-benzoic acid, compositions, and methods for the use thereof](#)
- 7429489- [Methods of controlling proliferation and differentiation of stem and progenitor cells](#)
- 7429646- [Antibodies to human tumor necrosis factor receptor-like 2](#)
- 7439043- [Galactosyl nucleotide sugars](#)

- [7439066- Methods for producing and using in vivo pseudotyped retroviruses using envelope glycoproteins from lymphocytic choriomeningitis virus \(LCMV\)](#)
- [7439332- Polypeptide having an activity to support proliferation or survival of hematopoietic stem or progenitor cells](#)
- [7442372- Delivery of therapeutic compounds to the brain and other tissues](#)
- [7446098- Combination therapy for treating protein deficiencies](#)
- [7449570- Use of nucleoside compounds for nonsense suppression and the treatment of genetic diseases](#)
- [7410954- Adenovirus serotype 30 \(Ad30\)](#)
- [7405233- Ureido substituted benzoic acid compounds and their use for nonsense suppression and the treatment of disease](#)
- [7402308- Method of delivering genes to the central nervous system of a mammal](#)
- [7368527- HADDE71 polypeptides](#)
- [7368531- Human secreted proteins](#)
- [7371366- GlcNAc phosphotransferase of the lysosomal targeting pathway](#)
- [7371388- Treatment of Sjogren's syndrome by administration of TR18 polypeptides](#)
- [7378231- Diagnosis of lysosomal storage disorders using saposins and other markers](#)
- [7388079- Delivery of pharmaceutical agents via the human insulin receptor](#)
- [7393526- Systemic gene delivery vehicles for the treatment of tumors](#)
- [7396811- Subcellular targeting of therapeutic proteins](#)
- [7399613- Sialic acid nucleotide sugars](#)
- [7399832- Metalloprotease-disintegrin ADAM23 \(SVPH3-17\)](#)
- [7465442- Reduction of dermal scarring](#)
- [7465580- Non-cytotoxic oriP replicon](#)
- [7569544- Methods of increasing delivery of active agents to brain comprising administering receptor associated protein \(RAP\) fragments conjugated to active agents](#)
- [7576101- Composition for and treatment of demyelinating diseases and paralysis by administration of remyelinating agents](#)
- [7592321- Methods of treating lysosomal storage related diseases by gene therapy](#)
- [7595318- Polyethylene glycol conjugates of heterocycloalkyl carboxamido propanoic acids](#)
- [7601816- Antibodies to cyclooxygenase variants](#)
- [7604958- Synthetic mammalian .alpha.-N-acetylglucosaminidase and genetic sequences encoding same](#)
- [7605236- Antibodies that immunospecifically bind to B lymphocyte stimulator protein](#)
- [7605249- Treatment of neurodegenerative disease through intracranial delivery of siRNA](#)
- [7608422- Simian immunodeficiency virus \(SIV\) molecular clone encoding mutant gag gene lacking inhibitory/instability regions](#)
- [7612107- Treatment and inhibition of disease conditions using flexible heteroarotinoids](#)
- [7566533- Clinically intelligent diagnostic devices and methods](#)
- [7563591- Method for assaying the activity of lysosomal enzymes](#)
- [7560431- Methods of increasing delivery of active agents to brain comprising administering receptor associated protein \(RAP\) fragments conjugated to active agents](#)
- [7470777- Compositions and methods related to modified retroviral vectors for restricted, site specific integration](#)
- [7479540- Adipomodulin and related molecules and methods](#)
- [7485314- Induction of antigen specific immunologic tolerance](#)
- [7498027- Methods of treatment using human tumor necrosis factor receptor TR17 antibodies](#)
- [7504105- Treatment using antibodies to cytokine receptor common gamma chain like](#)
- [7511052- Pyrimidinyl amide compounds which inhibit leukocyte adhesion mediated by VLA-4](#)
- [7514081- Methods of inhibiting an immune response with anti-human endokine alpha antibodies](#)
- [7544518- Rapid quantitative analysis of proteins or protein function in complex mixtures](#)
- [7547286- Method to reduce inflammation and tactile finger sensation deficit due to carpal tunnel syndrome or arthritis](#)

- 7560424- [Targeted therapeutic proteins](#)
- 7615224- [Multiplex-bead complex for determination of lysosomal storage disorders](#)

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