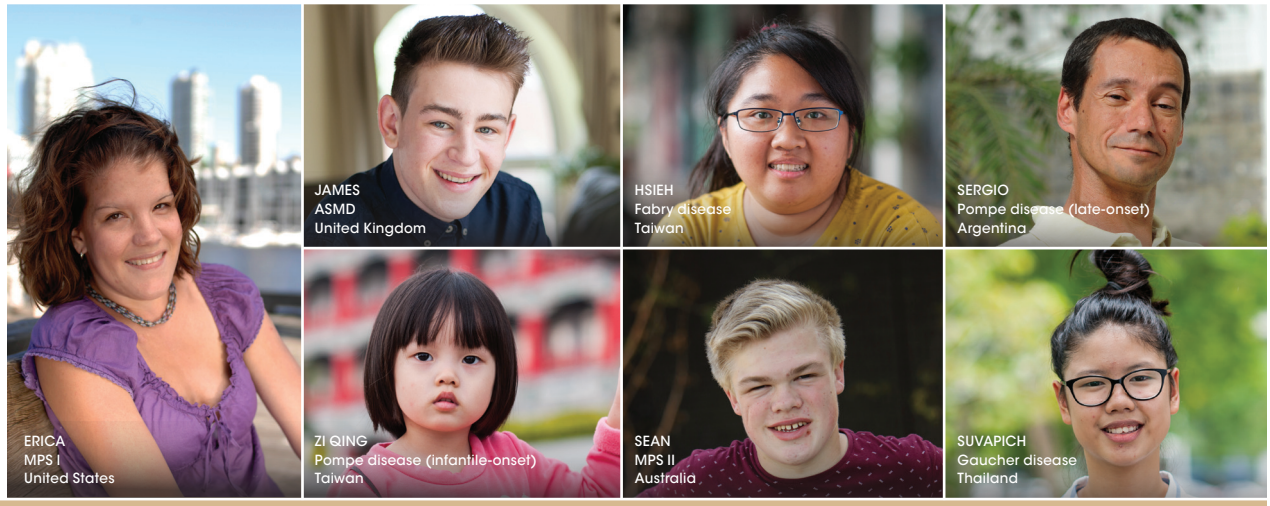
LYSOSOMAL STORAGE DISORDERS



AN OVERVIEW OF DISEASE AND DIAGNOSIS

SANOFI GENZYME

MAT-GLB-2003519 11/2020

ALFIE | ASMD | United Kingdom

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LSD BACKGROUND

2006 WORLDWIDE INCIDENCE[1]

1 in 1,500 to 7,000

LSDs ENCOMPASS **>50** DIFFERENT DISEASES

EARLY DIAGNOSIS is essential to both maximize potential improvement and prevent irreversible organ damage

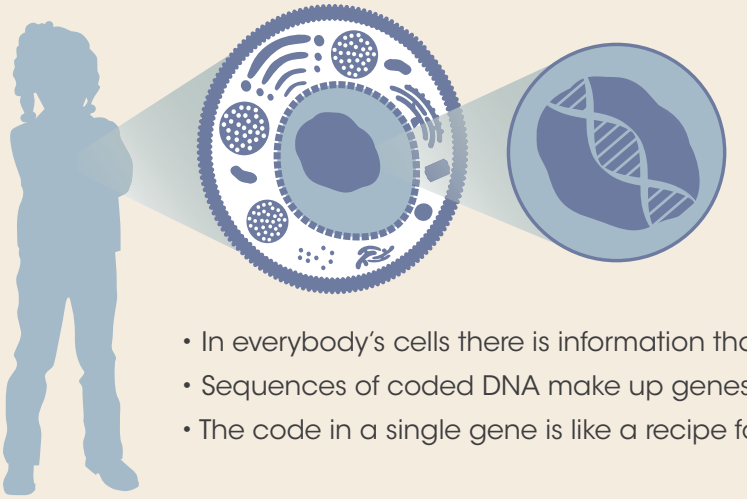
INHERITED

The majority of LSDs are inherited through an autosomal recessive manner. The three exceptions are X-linked: Fabry disease, Hunter syndrome, and Danon disease.[1]

SELECT EXAMPLES OF LSDS

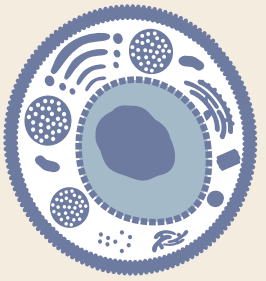
DISEASE	MISSING ENZYME	ACCUMULATING SUBSTANCE
ASMD	acid sphingomyelinase	sphingomyelin
Fabry disease	α-galactosidase A	globotriaosylceramide (GL-3)
Gaucher disease type 1	glucocerebrosidase	glucocerebroside
MPS I	α-L-iduronidase (IDUA)	glycosaminoglycans (GAGs)
MPS II	iduronate-2-sulfatase (IDS)	glycosaminoglycans (GAGs)
Pompe disease	acid α-glucosidase (GAA)	glycogen

HOW DOES IT WORK?



- In everybody's cells there is information that is coded into DNA
- Sequences of coded DNA make up genes
- The code in a single gene is like a recipe for a protein[2]

- Sometimes, there are mutations in that code
- This makes the recipe incomplete and can change the meaning of the code entirely
- People with some mutations may not be able to produce that protein. People with other mutations might not be able to produce enough of a working protein for their bodies to function normally[3]



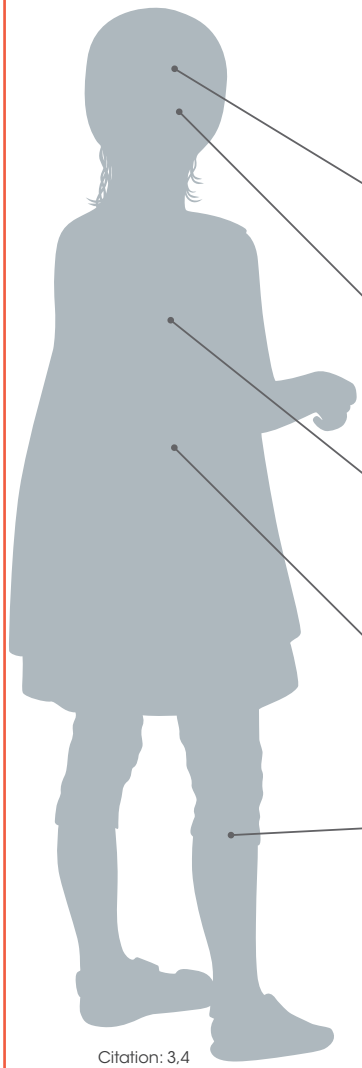
- In lysosomal storage disorders, there is a mutation in the gene encoding, in most cases, an enzyme (a type of protein that breaks down certain lipids or sugars)
- This leads to accumulation of fats and sugars in cell lysosomes, which disrupts normal function[4]

UNDERSTANDING ASMD

JAMES | United Kingdom

2015 WORLDWIDE INCIDENCE[2]

1 in 200,000

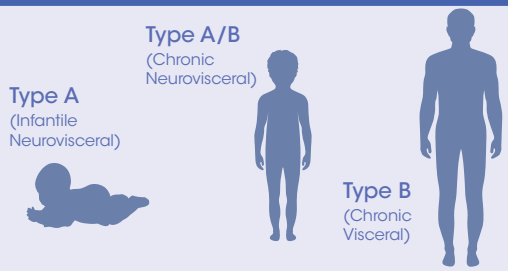


- HYPOTONIA*
- DEVELOPMENTAL DELAY**
- CHERRY RED MACULAE**
- PROGRESSIVE PULMONARY DYSFUNCTION
- HEPATOSPLENOMEGALY
- PATHOLOGIC FRACTURES***
- LOW PLATELETS, ABNORMAL CHOLESTEROL AND TRIGLYCERIDES, AND LOW HEMOGLOBIN

Citation: 3,4

* infantile neurovisceral ASMD
** infantile and chronic neurovisceral ASMD
*** chronic visceral and neurovisceral ASMD

TYPES OF ASMD[3]



Type A (Infantile Neurovisceral)

- Onset: Early Infancy
- Phenotype: Rapid and progressive visceral and neurological degeneration
- Life Expectancy: Infancy to early childhood

Acid Sphingomyelinase Deficiency (ASMD) is also known as Niemann-Pick Disease Type A (NPD A) and Type B (NPD B). ASMD is a serious and potentially life-threatening genetic disorder that causes accumulation of the unmetabolized lipid sphingomyelin in cells, resulting in damage to major organ systems. ASMD is represented by a broad clinical spectrum of disease, including both an infantile neurovisceral form (NPD A) and a chronic visceral form (NPD B).[1]

GENETIC PROFILE

ASMD is an autosomal recessive disorder caused by mutations in the *SMPD1* gene that encodes for the enzyme acid sphingomyelinase (ASM), which metabolizes sphingomyelin

Mutations in *SMPD1* result in various degrees of acid sphingomyelinase deficiency, leading to accumulation of sphingomyelin

Males and females have an equal chance of being affected[4]

IMPORTANCE OF EARLY DIAGNOSIS

The rarity of ASMD, heterogeneity of its manifestations, and challenging differential diagnosis can result in delayed diagnosis and management of patients.

Early diagnosis is important for ASMD as it allows for improved disease management in the absence of approved therapy.

Identifying known disease-causing alleles of ASMD allows for

1. Individual genetic counseling
2. Carrier screening for at-risk individuals
3. Family planning

HOW TO DIAGNOSE

A simple enzyme activity test can be performed on a DBS (dried blood spot) to indicate reduced ASM activity.

A positive DBS enzyme assay is highly indicative of ASMD, but an analysis from a whole blood sample and/or genetic testing is required to confirm the diagnosis.[5]

Type A/B (Chronic Neurovisceral)

- Onset: Infancy to childhood
- Phenotype: Slowly progressive variable visceral and neurological degeneration
- Life Expectancy: Childhood to mid-adulthood

Type B (Chronic Visceral)

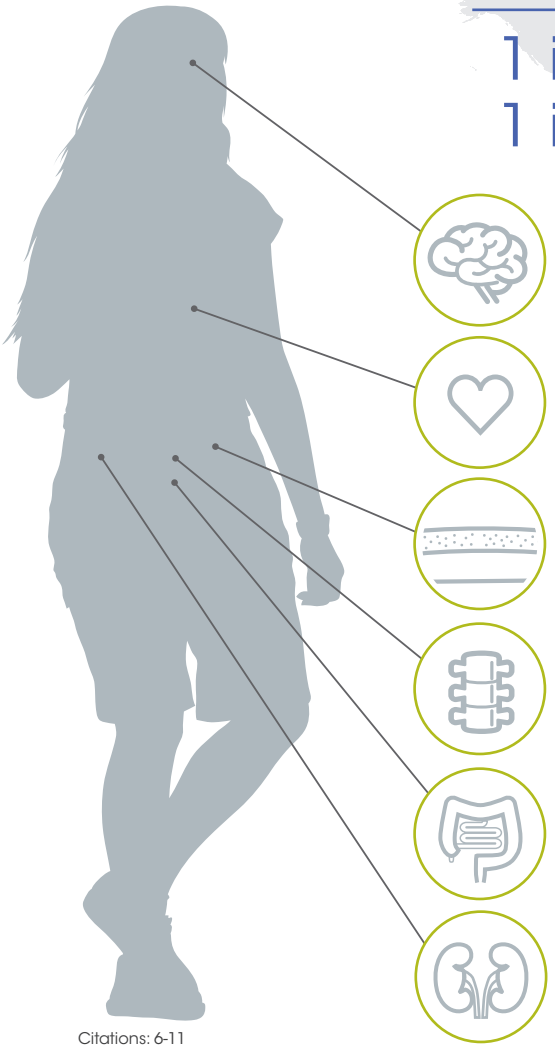
- Onset: Infancy to childhood
- Phenotype: Slowly progressive visceral disease with little to no neurological involvement
- Life Expectancy: Childhood to late adulthood

UNDERSTANDING FABRY DISEASE

HSIEH | Taiwan

2008 WORLDWIDE INCIDENCE[5,16]

1 in 40,000 males
1 in 30,000 females



DEPRESSION

LEFT VENTRICULAR HYPERTROPHY
(due to myocardial fibrosis)

ANGIOKERATOMA

NEUROPATHIC PAIN
(Chronic, pain attacks/crisis, evoked pain)

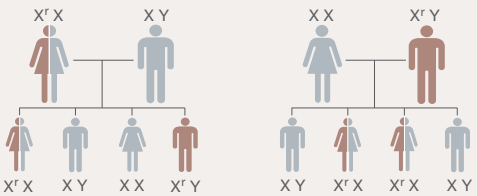
GASTROINTESTINAL DYSMOBILITY

RENAL INSUFFICIENCY

Citations: 6-11

INHERITANCE PATTERN

X-LINKED INHERITENCE



- *GLA* mutation
- No *GLA* mutation

Affected mothers have a 50% risk of passing along the defective gene (X^r) to their children, regardless of gender

Fabry Disease is a rare lysosomal storage disorder in which a deficiency of α-galactosidase A(α -Gal A) causes globotriaosylceramide (GL-3) and other substrates to accumulate in multiple cell types.[1,2] Accumulation of GL-3 can lead to progressive cell damage, organ failure, and eventually, premature death if left untreated.[3]

In Fabry disease, accumulation of GL-3 leads to fibrosis and end stage organ failure.[4]

GENETIC PROFILE

Fabry is inherited in an X-linked dominant manner. This gives females a greater chance of being affected.[5] There are over 770 mutations in the *GLA* gene that have been reported and they can vary in age of onset, rate of progression, organ involvement, and disease severity.[1,15]

IMPORTANCE OF EARLY DIAGNOSIS

The average time from symptom onset to diagnosis is 15 years for males and 18 years for females.[14] The large delay in diagnosis is due in part to the nonspecific nature of early symptoms, heterogeneous phenotypes, and lack of disease awareness.[1]

Fabry Disease is progressive and can lead to irreversible damage to major body organs. Early diagnosis is very important as it provides an opportunity to intervene early and avoid irreversible organ damage.[1]

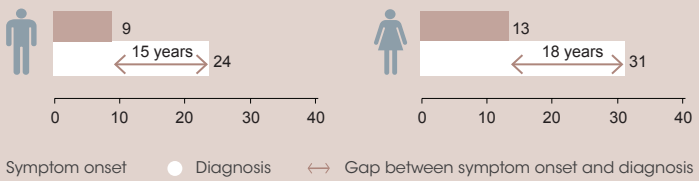
An initial diagnosis for Fabry Disease can be made through an α-GAL enzyme assay. This can by enzyme assay using plasma, leukocytes or skin cultured cells. Another common and easy way to test for α-GAL activity is a DBS (dried blood spot) assay.[12]

Enzyme assays that show a reduced or absent level of α-GAL should be followed up through a DNA analysis to confirm diagnosis and identify the specific *GLA* gene mutation.

Genetic testing and counseling should be offered to all relevant family members.[13]

TIME TO DIAGNOSIS

Male and female patients in the Fabry Registry were diagnosed 15 and 18 years, respectively, after onset of first symptoms[1]



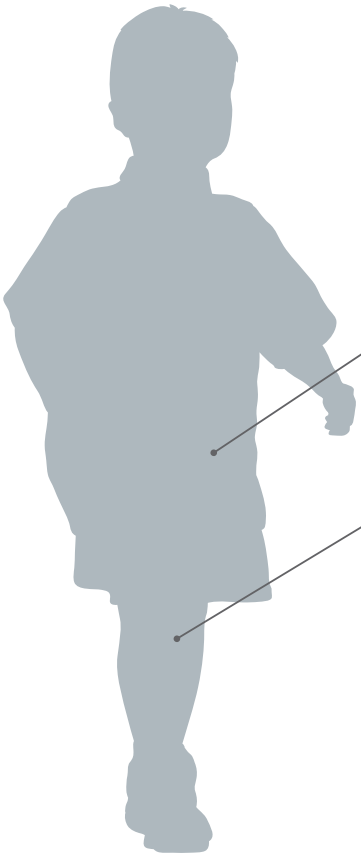
UNDERSTANDING GAUCHER DISEASE TYPE 1

SUVAPICH | Thailand

2006 DIAGNOSED PATIENT POPULATION[4]

1 in 40,000 in general population

1 in 500 to 1,000 people of Ashkenazi Jewish heritage



HEMATOLOGIC

Low platelets

Decreased hemoglobin levels

Bruising easily (due to low blood platelet count)

ENLARGEMENT OF LIVER AND SPLEEN

SKELETAL DISORDERS

Bone pain/bone crisis

Growth retardation

Pathologic fractures

Avascular necrosis

Low bone mineral density

PHENOTYPES OF GAUCHER DISEASE

Gaucher disease type 1 has a broad spectrum of severity. Symptoms can present in early childhood in a severe form or in adulthood in a mild form.[4]

Gaucher disease (pronounced go-shay) is the most common lysosomal storage disorder. It is caused by a deficiency in the enzyme glucocerebrosidase. Insufficient enzyme activity in Gaucher patients results in progressive accumulation of glucocerebroside in the macrophages. Clinical symptoms arise due to the displacement of normal cells by lipid engorged Gaucher cells. Accumulation occurs in organs throughout the body, typically the bone marrow, liver, and spleen.[1,2,3]

GENETIC PROFILE

Gaucher disease is inherited in an autosomal recessive manner. If both parents are carriers of the disease-causing allele, their child has a 25% chance of being affected with Gaucher disease. Males and females have an equal chance of being affected.[3,5]

Gaucher disease has a much higher prevalence in the Ashkenazi Jewish population occurring at a rate around 100 times higher than the general population.

Gaucher disease varies widely in clinical expression, and patients present with a broad spectrum of phenotypes. Patients may have severe disease in childhood, or may remain essentially symptom-free into late adulthood.

IMPORTANCE OF EARLY DIAGNOSIS

Delays in diagnosis of Gaucher disease are common. A patient with Gaucher disease may experience a delay for up to 10 years.[6]

Gaucher disease is progressive, yet almost 25% of patients do not get timely access to appropriate disease management because of delays in diagnosis.

77% of the symptomatic Gaucher disease population has never received a diagnosis.[2]

HOW TO DIAGNOSE

A simple enzyme activity test can be performed on a DBS (dried blood spot).

A positive DBS enzyme assay is highly indicative of Gaucher disease, but an analysis from a whole blood sample is required to confirm the diagnosis.[6]

	TYPE 1	TYPE 2	TYPE 3
NEUROLOGICAL EFFECTS	None	Severe	Moderate to severe
SYMPTOM ONSET	Any age	First year of life	Childhood
COURSE	Progressive	Rapidly progressive	Progressive

UNDERSTANDING MPS I

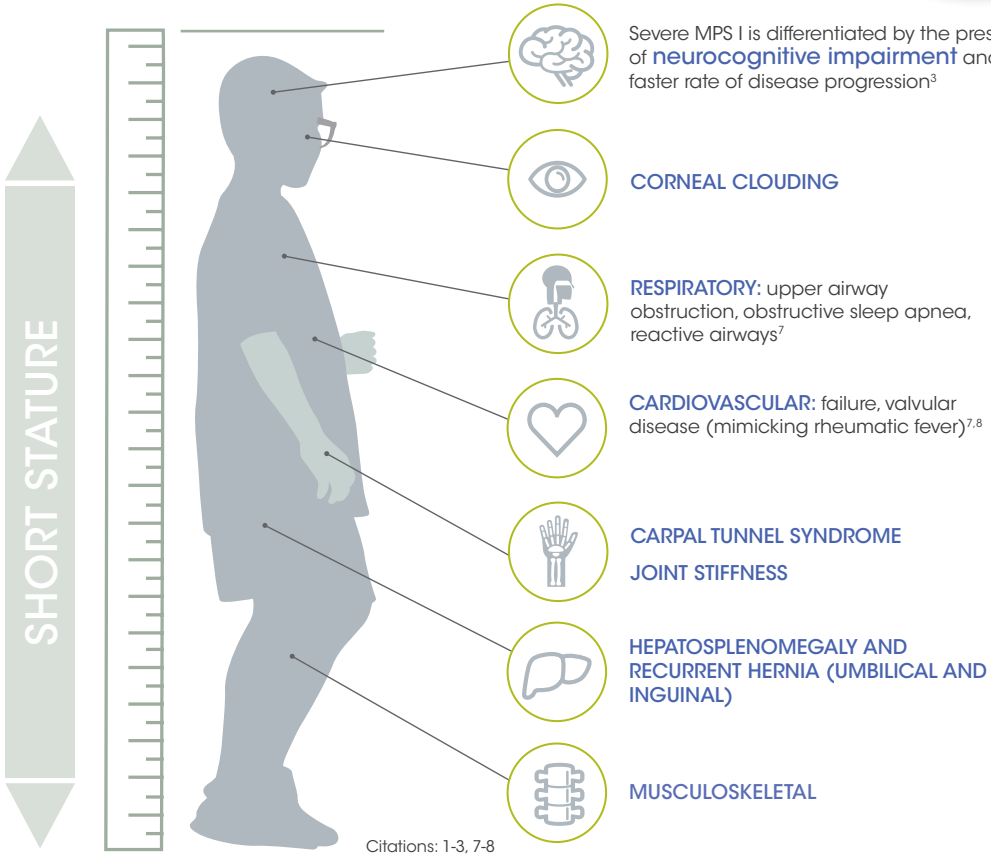
ERICA | United States

1999 WORLDWIDE INCIDENCE[2]

1 in 100,000

SEVERE ⇢ ATTENUATED

MPS I presents as a **disease spectrum**, ranging from the severe phenotype to the attenuated phenotype[2]



Severe MPS I is differentiated by the presence of **neurocognitive impairment** and a faster rate of disease progression[3]

CORNEAL CLOUDING

RESPIRATORY: upper airway obstruction, obstructive sleep apnea, reactive airways[7]

CARDIOVASCULAR: failure, valvular disease (mimicking rheumatic fever)[7,8]

CARPAL TUNNEL SYNDROME

JOINT STIFFNESS

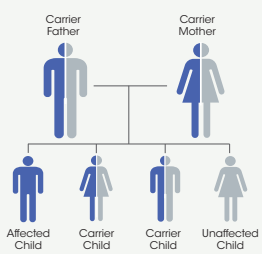
HEPATOSPLENOMEGALY AND RECURRENT HERNIA (UMBILICAL AND INGUINAL)

MUSCULOSKELETAL

Citations: 1-3, 7-8

INHERITANCE PATTERN

AUTOSOMAL RECESSIVE INHERITENCE



The mucopolysaccharidoses (MPS) are a group of rare, genetic disorders caused by the absence or malfunctioning of lysosomal enzymes needed to break down storage molecules called glycosaminoglycans (GAGs).[1]

MPS I, one of the seven types of MPS, results from deficiency of α-L-iduronidase (IDUA) due to pathogenic variants in the *IDUA* gene.[1]

GENETIC PROFILE

MPS I is an autosomal recessive disorder; over 250 mutations have been described in the literature.[1,4]

Genotype - phenotype correlations have been established for some mutations, but others are novel.[5]

There is a higher prevalence of severe MPS I in the Irish Traveller Community, due to homozygous W402X mutations.[6]

IMPORTANCE OF EARLY DIAGNOSIS

Diagnosis of MPS I is often delayed, due to the non-specific nature of symptoms.[9] Early diagnosis and intervention is essential to prevent or minimize irreversible organ damage an improve long-term clinical outcomes.[10]

MPS I newborn screening has gained increasing importance as the evidence base for early intervention and improved outcomes has been clearly demonstrated.[8]

A simple urine test to look for abnormally high levels of GAGs (heparan sulfate and dermatan sulfate) can be ordered.[1]

Diagnosis is confirmed via enzyme assay and genetic testing.[2,9]

FOR MORE INFORMATION:

- test4mps.com
- mps1disease.com/en/healthcare
- mpssociety.org
- mpsreference.eu

UNDERSTANDING MPS II

SEAN | Australia

SEVERE ⇢ ATTENUATED

MPS II presents as a **disease spectrum**, ranging from the severe (neuronopathic) phenotype to the attenuated (non-neuronopathic) phenotype.[3]

1999 WORLDWIDE INCIDENCE[2]

1 in 162,000

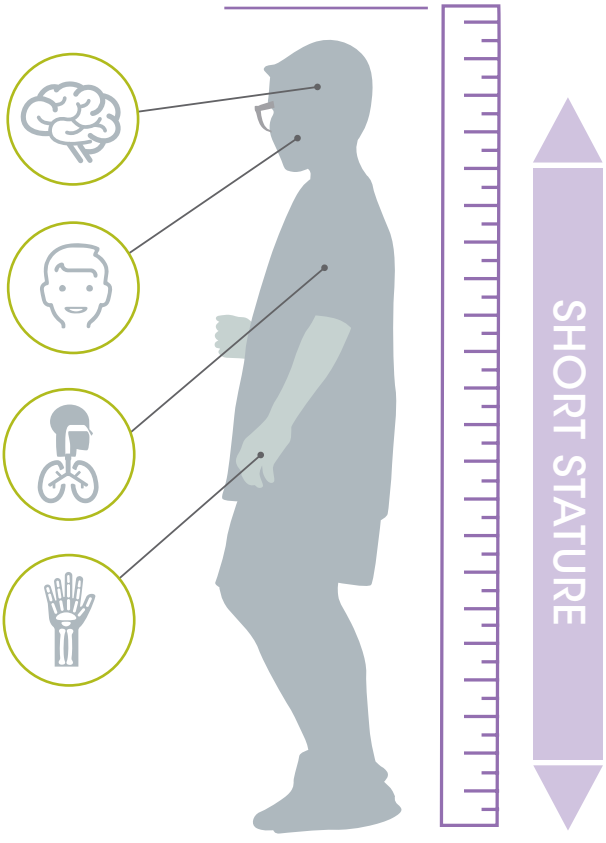
Severe MPS II is differentiated by the presence of **neurological signs and symptoms** including hyperactivity, aggressive behavior, and cognitive impairment

COARSE FACIAL FEATURES

RECURRENT EAR AND UPPER RESPIRATORY TRACT INFECTIONS

CARPAL TUNNEL SYNDROME

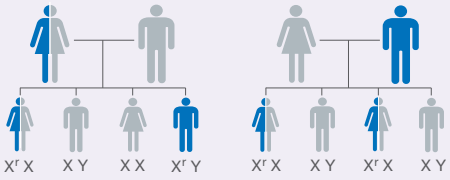
JOINT STIFFNESS



Citations: 1, 4, 5

INHERITANCE PATTERN

X-LINKED INHERITENCE



- *IDS* mutation
- No *IDS* mutation

Affected mothers have a 50% risk of passing along the defective gene (X^r) to their children, regardless of gender

The mucopolysaccharidoses (MPS) are a group of rare, genetic disorders caused by the absence or malfunctioning of lysosomal enzymes needed to break down storage molecules called glycosaminoglycans (GAGs).[1]

MPS II, also known as Hunter Syndrome, one of the seven types of MPS, results from deficiency of iduronate-2-sulfatase (IDS) due to the pathogenic variants in the *IDS* gene.[1]

GENETIC PROFILE

MPS II is an X-linked recessive disorder that almost exclusively affects males.[1]

Heterozygous MPS II females (carriers) are generally asymptomatic or not heavily affected.[1]

More than 300 mutations in the IDS gene have been reported, although genotype – phenotype correlations have not been established.[6]

Mutations that result in complete absence of IDS activity lead to a severe phenotype.[1,6]

IMPORTANCE OF EARLY DIAGNOSIS

Diagnosis of MPS II is often delayed, due to the non-specific nature of symptoms.[7] Early diagnosis and intervention is essential to prevent or minimize irreversible organ damage and improve long-term clinical outcomes.[7]

MPS II newborn screening has gained increasing importance as the evidence base for early intervention and improved outcomes has been clearly demonstrated.[8]

HOW TO DIAGNOSE

A simple urine test to look for abnormally high levels of GAGs (heparan sulfate and dermatan sulfate) can be ordered.[1]

Diagnosis is confirmed via enzyme assay and genetic testing.[9]

FOR MORE INFORMATION:

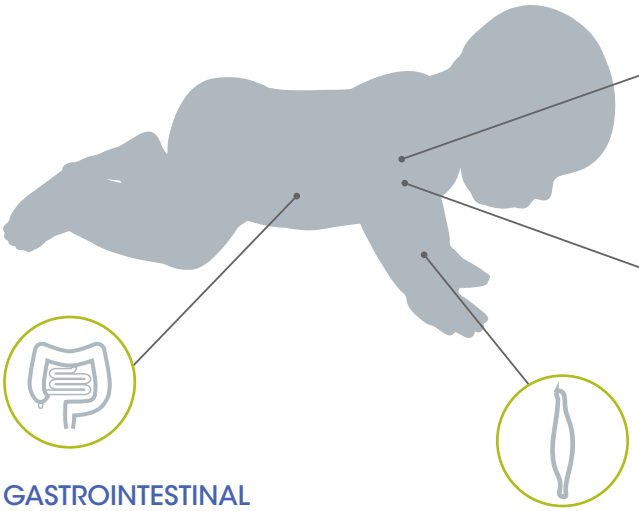
- test4mps.com
- huntersyndrome.info
- mpssociety.org
- mpsreference.eu

UNDERSTANDING POMPE DISEASE
(infantile-onset)

Zi Qing | Taiwan

1999 WORLDWIDE INCIDENCE[8]

1 in 40,000
Combined IOPD and LOPD



CARDIAC
Cardiomegaly

Progressive cardiomyopathy

RESPIRATORY
Respiratory distress and frequent infections

Sleep-disordered breathing

GASTROINTESTINAL
Macroglossia

Feeding difficulties

Failure to thrive/poor weight gain

Hepatomegaly

MUSCULOSKELETAL
Progressive muscle weakness and delayed motor milestones

Profound hypotonia

Citations: 2-5

GENOTYPE - PHENOTYPE CORRELATION

2 **severe** gene variants → **IOPD**

1 **severe** + 1 **mild** gene variant → **LOPD**

2 **mild** gene variants → **LOPD**

Pompe Disease is a progressive, multi-systemic, debilitating, and often life-threatening neuromuscular disorder. Pompe disease is linked to an inherited deficiency of acid alpha-glucosidase (GAA), which is responsible for the breakdown of glycogen inside the cells. Without sufficient GAA enzyme, glycogen accumulates primarily in muscle cells, which leads to progressive loss of muscle function. Infantile-onset Pompe disease (IOPD) is rapidly progressing and usually presents within the first month of life. If left untreated, IOPD is often fatal before age 1.[1]

GENETIC PROFILE

IOPD is inherited in an autosomal recessive manner. The *GAA* gene can have a spectrum of variants that lead to differing amounts of GAA enzyme activity.[1] Variants that leave little to no GAA enzyme activity manifest as the severe infantile onset form of Pompe Disease.[2]

IMPORTANCE OF EARLY DIAGNOSIS

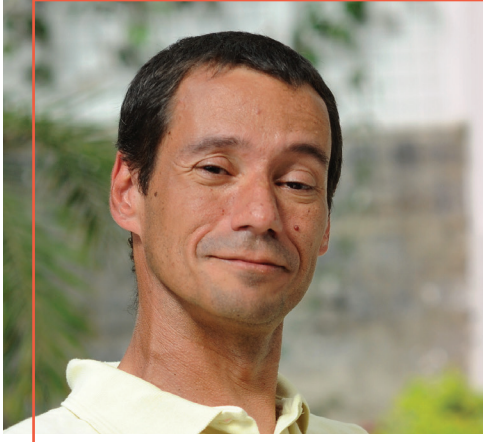
Infantile-Onset Pompe disease is rapidly progressing and early diagnosis can be crucial in providing supporting therapy. Delays in diagnosis of IOPD correlate with respiratory failure, profound motor impairment, and death.[6]

Newborn screening for Pompe disease can readily identify patients with IOPD. This allows immediate diagnosis and can lead to improving survival and outcomes.[7]

A relatively quick, simple, and minimally invasive way to screen for Pompe disease is the DBS (dried blood spot) enzyme assay. A DBS is able to detect low or absent levels of the GAA enzyme.[3]

Reduced GAA enzyme activity should always be followed with a second, non-blood sample and/or *GAA* gene sequencing to confirm diagnosis of Pompe Disease.[3]

	IOPD	LOPD
AGE OF ONSET	Infancy	Infancy - adulthood
PROGRESSION	Rapid	Varying
PREDOMINANT SYMPTOMS	Respiratory distress, hypotonia, muscle weakness	Progressive proximal muscle weakness, respiratory deficit



UNDERSTANDING POMPE DISEASE

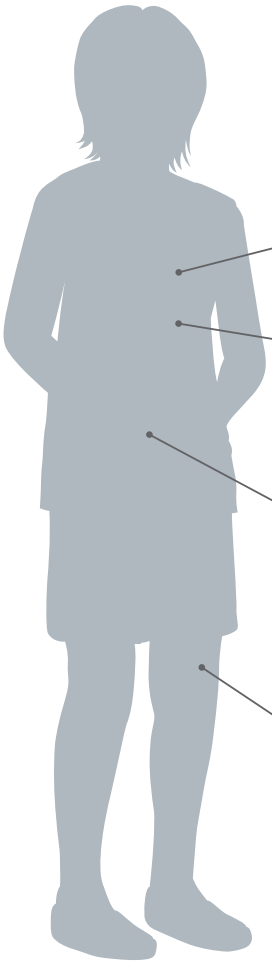
(late-onset)

SERGIO | Argentina

1999 WORLDWIDE INCIDENCE[10]

1 in 40,000

Combined IOPD and LOPD



CARDIAC

Less common among adults

RESPIRATORY

Respiratory failure/insufficiency

Diaphragm weakness, sleep-disordered breathing

Orthopnea, dyspnea, aspiration

GASTROINTESTINAL

Difficulty chewing/jaw muscle fatigue

Poor weight gain/maintenance

Swallowing difficulties/weak tongue

Gastroesophageal reflux, fecal incontinence

MUSCULOSKELETAL

Proximal muscle weakness, muscle pain

Frequent falls, gait abnormalities, difficulty walking/climbing stairs/getting up

EMG abnormalities, elevated CK, MRI changes

Citations: 2-7

GENOTYPE - PHENOTYPE CORRELATION

- 2 **severe** gene variants → **IOPD**
- 1 **severe** + 1 **mild** gene variant → **LOPD**
- 2 **mild** gene variants → **LOPD**

Pompe Disease is a progressive, multi-systemic, debilitating, and often life-threatening neuromuscular disorder. Pompe disease is linked to an inherited deficiency of acid alpha-glucosidase (GAA), which is responsible for the breakdown of glycogen inside the cells. Without sufficient GAA enzyme, glycogen accumulates primarily in muscle cells, which leads to progressive loss of muscle function.[1] Late-onset Pompe disease (LOPD) has a less rapid and more variable disease course, where symptoms may begin anywhere from infancy to adulthood.[1]

GENETIC PROFILE

LOPD is inherited in an autosomal recessive manner. The *GAA* gene can have a spectrum of variants that lead to differing amounts of GAA enzyme activity.[1] The mild variants that leave residual enzyme activity manifest as the less rapid form of Pompe Disease, LOPD.[8]

IMPORTANCE OF EARLY DIAGNOSIS

If treated early enough, muscle damage from GAA accumulation can be reversible. However, if left untreated, it can lead to irreversible deterioration of skeletal and respiratory muscle, disability, and premature death. The initiation of an intervention is crucial in preventing further deterioration of muscle and permanent disability.[4]

A person with LOPD may go several years before receiving a diagnosis. Newborn screening for Pompe can help diagnose patients before irreversible muscle damage occurs.[7]

HOW TO DIAGNOSE

A relatively quick, simple, and minimally invasive way to screen for Pompe disease is the DBS (dried blood spot) enzyme assay. A DBS is able to detect low or absent levels of the GAA enzyme.[9]

Reduced GAA enzyme activity should always be followed with a second, non-blood sample and/or *GAA* gene sequencing to confirm diagnosis of Pompe Disease.[9]

	IOPD	LOPD
AGE OF ONSET	Infancy	Infancy - adulthood
PROGRESSION	Rapid	Varying
PREDOMINANT SYMPTOMS	Respiratory distress, hypotonia, muscle weakness	Progressive proximal muscle weakness, respiratory deficit

PATIENT FOCUS

SANOFI GENZYME GLOBAL HUMANITARIAN PROGRAM

The primary goal of the Sanofi Genzyme Humanitarian program is to deliver therapies, to the best of our ability, to patients with lysosomal storage disorders who have a demonstrated need in certain circumstances where treatment access is limited.

QUICK FACTS:

More than **750 patients** in **65 countries** are in our program today

More than **3,000 patients** in over **90 countries** have received access to therapy

More than **300 patients** have been on treatment for more than **10 years**

25+ YEARS

Our program began in 1991, in our first year of having a commercial product. Since then, it has evolved and expanded to support 5 different lysosomal storage disorder communities across 6 continents.

CUSTOMIZED

Our program is customized based on the needs of the patients we serve. It is a resource to support patients where there is limited or no current access to therapy.

BRIDGE TO ACCESS

Our program serves as a bridge while countries work to establish a long term patient care support system. It has played a key role in helping build sustainable healthcare systems that are able to provide support and holistic care for patients globally.

MISSION

Our program represents one of the greatest achievements of our organization and truly embodies the spirit on which our company was established. It is a vital element of our mission to support development of sustainable healthcare systems and improve patients' lives.

In addition to operating its Humanitarian Program, Sanofi Genzyme offers support for patient diagnosis, treatment monitoring, patient advocacy and physician education.

For healthcare providers, many times our Humanitarian program serves as a physician's first experience is treating an LSD patient. Sanofi Genzyme helps provide training and education for patient identification, understanding of treatment expectations and monitoring.

For governments, Sanofi Genzyme collaborates with local officials to establish sustainable healthcare systems to care for the needs of patients with LSDs.

For the patient community, Sanofi Genzyme works closely with patient organizations to help raise awareness of the unique challenges that having an LSD can bring and to help address the unmet needs of this population.

SANOFI GENZYME IS DEVOTED TO PATIENTS AND FAMILIES OF THE RARE COMMUNITY

RARE DISEASE PUBLIC AFFAIRS AND PATIENT ADVOCACY

ONE PURPOSE. To learn from and revolutionize the Rare experience.

ONE APPROACH. Game-changing advocacy for and with Rare Community to enable science, solutions, and services that transform people's lives.

ONE GOAL. Making Rare Dreams A Reality!

SHARED OBJECTIVES TO TRANSFORM THE RARE COMMUNITY

We diligently focus our Patient Advoacy on matters that mean real progress for the LSD community.

CREATIVE PARTNERSHIPS AND PLATFORMS TO OPTIMIZE RARE IMPACT

Our pioneering spirit and passion spur creative opportunties and discoveries that enhance the standard of care.

ONE GLOBAL RARE PATIENT ADVOCACY PHILOSOPHY

INVESTING IN RARE

Critical resources are devoted to key collaborations aimed at improving diagnosis, management and care for those living with LSDs.

SOPHISTICATED RARE COMMUNITY ADVOCATES

Broaden and fortify Patient Advocacy to amplify the voice and authority of the Rare Community.

Each Rare life matters. Each Rare story matters. Each reveals the reality of being Rare. Through various fora we showcase the valiant heart and momentous impact of the Rare Community—one life at a time.

RARELIVES
THE MEANING OF LIVING WITH A RARE DISEASE

RARELIVES is a photographic journey featuring both the challenges and the often joyful daily achievements of those living a Rare life. RARELIVES is developed by Aldo Soligno and UNIAMO F.I.M.R. ONLUS, with the support of Sanofi Genzyme.

PAL AWARDS
Patient Advocacy Leadership Awards

Eight years ago, we launched the **Patient Advocacy Leadership (PAL) Awards** to support non-profits dedicated to LSD patients. We have since awarded greater than $800,000 for diverse projects in 28 countries.

Sanofi Genzyme TORCH AWARDS

Our **TORCH** recognizes individuals of all ages in the U.S. who have made a significant contribution to the LSD community. Sanofi Genzyme makes a contribution to an LSD-focused, non-profit at the choosing of each TORCH recipient.

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SANOFI GENZYME

THE HILL FAMILY
Fabry disease
United States

Sanofi Genzyme is the specialty care global business unit of Sanofi, focused on rare diseases, multiple sclerosis, oncology and immunology. We help people with debilitating and complex conditions that are often difficult to diagnose and treat. We are dedicated to discovering and advancing new therapies, providing hope to patients and their families around the world.

Our ambition is to be the industry leader in specialty care. We currently provide more than 20 treatments to patients globally, with many potential new therapies being studied in clinical trials and in Sanofi laboratories.

SANOFI GENZYME

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