# TREATMENT OF GAUCHER DISEASE

## GAUCHER DISEASE TYPES 1 AND 3 ARE TREATABLE

**Gaucher disease types 1 and 3 can be treated by[1,2]:**

- Enzyme replacement therapy (ERT)
- Bone marrow transplant (BMT)

The morbidity and mortality associated with BMT limit its utilisation in individuals with Gaucher disease types 1 and 3.[2]

Thus, **BMT has been largely superseded by ERT**.[2]

**Treatment for Gaucher disease type 2[2]**

- It is not clear if brain damage can be reversed once it has manifested.
- Pre-symptomatic diagnosis or in utero therapy may be necessary to mitigate the neuronal loss.

## IMIGLUCERASE IS CLINICALLY EFFECTIVE IN GAUCHER DISEASE TYPES 1 AND 3[3]

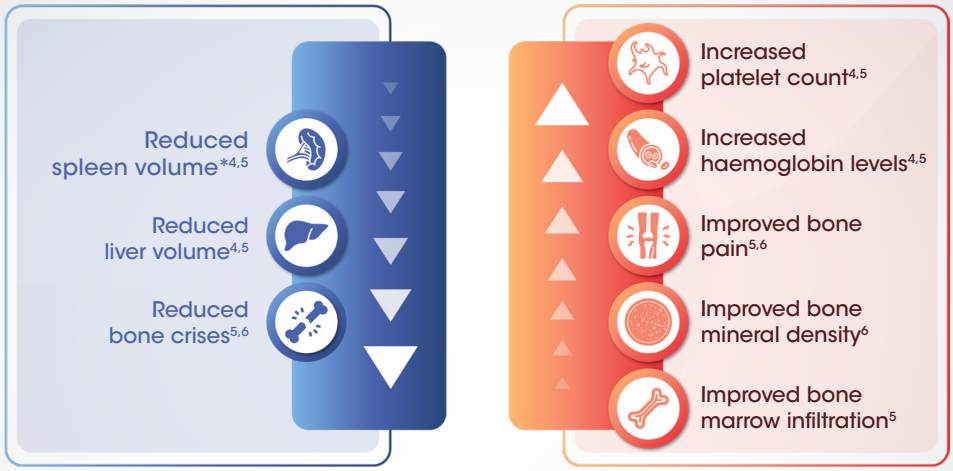
Imiglucerase is a recombinant DNA-produced analogue of human ß-glucocerebrosidase.[3]

**Imiglucerase is suitable for[3]:**

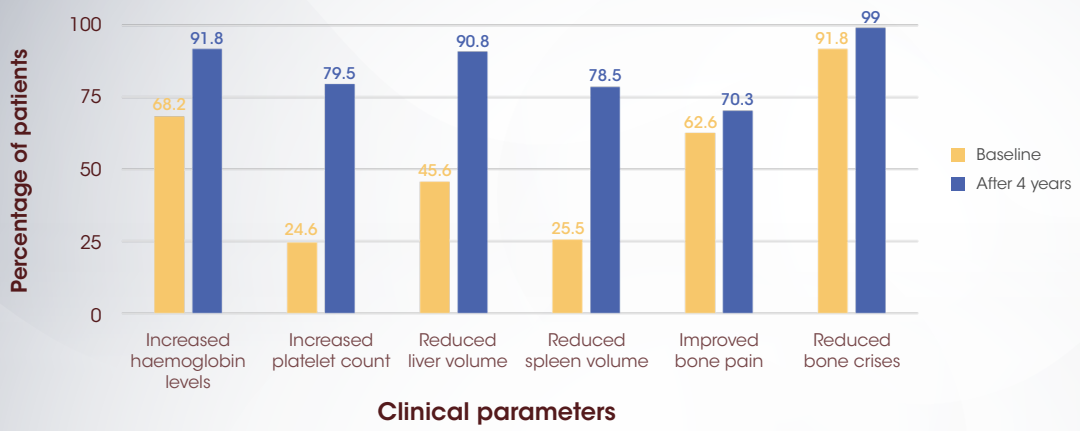
- Gaucher disease type 1
- Gaucher disease type 3 without neurological symptoms

Imiglucerase has been the **standard of care** for the treatment of Gaucher disease patients since its introduction in 1994.[4]

## GAUCHER DISEASE PATIENTS ACHIEVE MULTIPLE THERAPEUTIC GOALS WITH IMIGLUCERASE



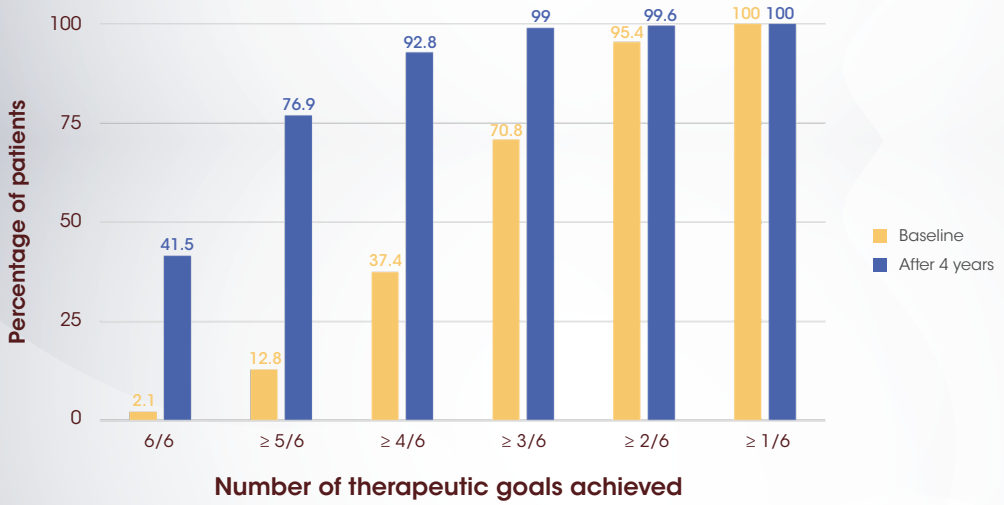
# MORE PATIENTS ACHIEVE THERAPEUTIC GOALS OVER 4 YEARS OF IMIGLUCERASE TREATMENT[4]




*Adapted from Weinreb NJ, et al. Am J Hematol. 2008.*

At least 70% of patients achieve each individual therapeutic goal after 4 years of treatment.[4]

# OVER 90% OF PATIENTS ACHIEVE AT LEAST 4 THERAPEUTIC GOALS AFTER 4 YEARS OF IMIGLUCERASE TREATMENT[4]




*Adapted from Weinreb NJ, et al. Am J Hematol. 2008.*

Over 40% of patients meet all 6 therapeutic goals after 4 years of treatment.[4]

# TEN YEARS OF IMIGLUCERASE TREATMENT REVEALS SUSTAINABLE IMPROVEMENTS IN ALL PARAMETERS

## Non-splenectomised patients[7]

| Improved parameter | Mean change after 10 years | p-value |
|---|---|---|
| Increased hemoglobin levels (g/dL) | + 2.4 | < 0.0001 |
| Increased platelet count (x $10^3$/$mm^3$) | + 71.3 | < 0.0001 |
| Reduced spleen volume (multiples of normal) | - 14.3 | < 0.0001 |
| Reduced liver volume (multiples of normal) | - 0.8 | < 0.0001 |

*Adapted from Weinreb NJ, et al. J Inherit Metab Dis. 2013.*

# TEN YEARS OF IMIGLUCERASE TREATMENT REVEALS SUSTAINABLE IMPROVEMENTS IN ALL PARAMETERS

## Splenectomised patients[7]

| Improved parameter | Mean change after 10 years | p-value |
|---|---|---|
| Increased hemoglobin levels (g/dL) | + 1.5 | < 0.0001 |
| Increased platelet count (x $10^3$/$mm^3$) | + 73.4 | < 0.0001 |
| Reduced liver volume (multiples of normal) | - 1.2 | < 0.0001 |

*Adapted from Weinreb NJ, et al. J Inherit Metab Dis. 2013.*

# PATIENTS REPORTED IMPROVED BONE PAIN WITH IMIGLUCERASE TREATMENT

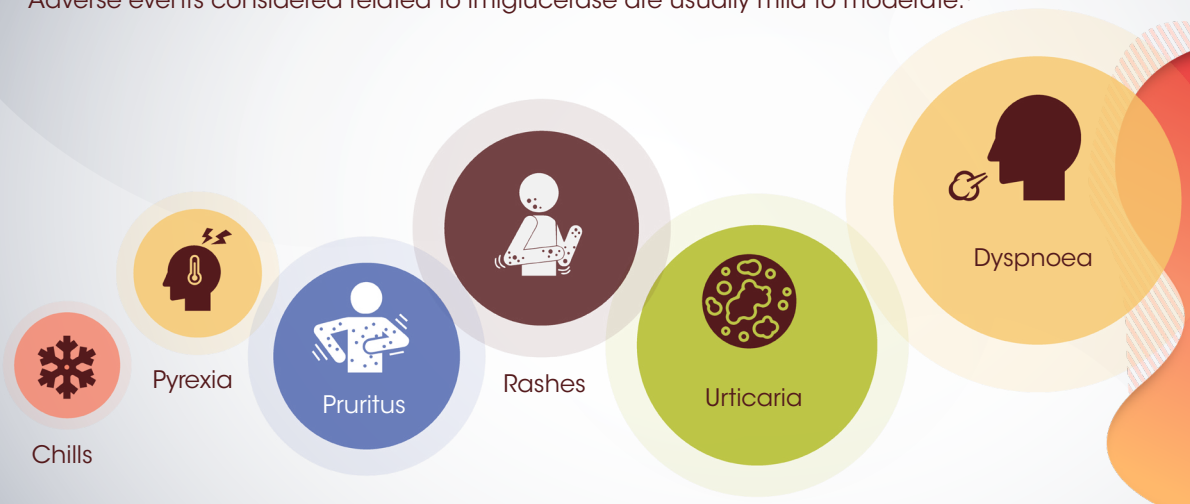

*Adapted from Sims KB, et al. Clin Genet. 2008.*

Improvement in bone pain was observed at 3 months ($p = 0.001$ vs. baseline), and continued progressively throughout the 48-month study.[6]

Substantial improvements were also observed in bone crises and bone mineral density.[6]

# GOOD TOLERANCE OF IMIGLUCERASE ALLOWS FOR IMPROVED PATIENT QUALITY OF LIFE[5]

Adverse events considered related to imiglucerase are usually mild to moderate.[5]




Between 1994 and 2004, **only 4 out of 4237** patients who received imiglucerase were stopped therapy due to infusion reactions.[8]

**No serious adverse events** were reported with imiglucerase.[5]

Each of these events occur in **<1%** of the total patient population.[8]

*Non-splenectomised patients.

**For healthcare professionals only**

## References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