# GLANZMANN THROMBASTHENIA NATURAL HISTORY STUDY+

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The main objective is to determine genetic phenotype and the prevalence of anti-HLA (human leucocyte antigen) and anti-HPA (human platelet antigen) antibodies in patients with Glanzmann thrombasthenia.

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| **Ethical review** | Approved WMO |
| **Status** | Recruiting |
| **Health condition type** | Platelet disorders |
| **Study type** | Observational invasive |

## Summary

### ID

NL-OMON56766

**Source**
ToetsingOnline

**Brief title**
Glanzmann-NHS+

### Condition

- Platelet disorders

**Synonym**
Glanzmann thrombasthenia, Glanzmann's disease

**Research involving**
Human

### Sponsors and support

**Primary sponsor:** Universitair Medisch Centrum Utrecht
**Source(s) of monetary or material Support:** Farmaceutisch bedrijf,Hemab ApS

## Intervention

**Keyword:** Antibody screening, Genetic mutations, Glanzmann thrombasthenia, Platelet disorder

## Outcome measures

### Primary outcome

To determine the genetic phenotype in patients with Glanzmann thrombasthenia.

### Secondary outcome

To estimate the prevalence of Human Leukocyte antigens (HLA) and Human Platelet Antigens (HPA) antibodies in patients with Glanzmann thrombasthenia. These antibodies may develop as a consequence of treatment with the current golden standard: platelet transfusion or transfusion with other blood products including red blood cells and plasma.

Together with the data from the Glanzmann-NHS registry, results from the Glanzmann-NHS+ study will allow to investigate whether there are correlations between genotype and clinical phenotype (bleeding score).

# Study description

### Background summary

Glanzmann thrombasthenia is a rare autosomal recessive platelet disorder characterized by a lack of functional integrins aplhaIIb or β3 (glycoproteins IIb/IIIa). The clinical phenotype is dominated by an increased mucocutaneous bleeding tendency. In absence of a primary bleeding prophylaxis, the current treatment of Glanzmann thrombasthenia is mainly focused on prevention or management of bleeding. However, as potential new therapies emerge, clinicians require long-term safety and efficacy data for both current treatment and new therapies.

The Glanzmann-NHS+ study was designed to investigate genetic phenotype and the prevalence of antibodies against human leucocyte antigen (HLA) and human platelet antigen (HPA), the latter two being a potential consequence of the current golden standard treatment: platelet transfusion. The results of this study will be merged with a longitudinal registry with retrospective and prospective data collection of clinical phenotype, haemorrhagic burden and bleeding management. Analysis of the data from the Glanzmann-NHS+ study and the registry will help us to get a better understanding of the clinical variation among participants with Glanzmann thrombasthenia. The ultimate goal is to accelerate improvement in the care of patients with Glanzmann thrombasthenia.

### Study objective

The main objective is to determine genetic phenotype and the prevalence of anti-HLA (human leucocyte antigen) and anti-HPA (human platelet antigen) antibodies in patients with Glanzmann thrombasthenia.

### Study design

The Glanzmann-NHS+ study is an international, cross-sectional, multicentre study to determine genetic mutation analysis and the prevalence of anti-HLA and anti-HPA antibodies in patients with Glanzmann thrombasthenia.

### Study burden and risks

Results of this study, combined with our longitudinal registry with retrospective and prospective data collection of clinical phenotype, haemorrhagic burden and bleeding management, will contribute to a better understanding of the Glanzmann thrombasthenia and to get more insight into the disease mechanisms. Together, this will help us to improve future therapy options and to improve care for this patient group. The study consists of a single venepuncture that will be combined with venepuncture during route outpatient follow-up. Risk imposed by participation are considered negligible.

## Contacts

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# Trial sites

## Listed location countries

Netherlands

# Eligibility criteria

**Age**
Adolescents (16-17 years)
Adults (18-64 years)
Elderly (65 years and older)

## Inclusion criteria

o Adult patients (>=16 years);
o Biochemically or genetically diagnosed Glanzmann thrombasthenia.
o Willing and able to give written informed consent

## Exclusion criteria

- Patients with acquired thrombasthenic states caused by auto-immune disorders or drugs.

# Study design

## Design

**Study type:** Observational invasive

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| Masking: | Open (masking not used) |
| Control: | Uncontrolled |
| Primary purpose: | Basic science |

### Recruitment

NL

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|---|---|
| Recruitment status: | Recruiting |
| Start date (anticipated): | 11-07-2024 |
| Enrollment: | 30 |
| Type: | Actual |

## Ethics review

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| Approved WMO Date: | 30-05-2024 |
| Application type: | First submission |
| Review commission: | METC NedMec |
| Approved WMO Date: | 03-09-2024 |
| Application type: | Amendment |
| Review commission: | METC NedMec |

## Study registrations

### Followed up by the following (possibly more current) registration

No registrations found.

### Other (possibly less up-to-date) registrations in this register

No registrations found.

### In other registers

| Register | ID |
|---|---|
| ClinicalTrials.gov | NCT06204042 |
| CCMO | NL85068.041.24 |