# An open label extension study to evaluate the safety of long term dosing of AMG 531 in trombocytopenic subjects with myelodysplastic syndromes (MDS).

Published: 22-08-2007
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To provide long-term safety data for the use of AMG 531 in thrombocytopenic subjects with IPSS low or intermediate-1 risk MDS.

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| **Ethical review** | Approved WMO |
| **Status** | Pending |
| **Health condition type** | Haematopoietic neoplasms (excl leukaemias and lymphomas) |
| **Study type** | Interventional |

## Summary

### ID

NL-OMON31052

**Source**
ToetsingOnline

**Brief title**
Extens. trial to evaluate safety of long term AMG531 dosing in MDS patients

### Condition

- Haematopoietic neoplasms (excl leukaemias and lymphomas)

**Synonym**
Myelodysplasia

**Research involving**
Human

### Sponsors and support

**Primary sponsor:** Amgen
**Source(s) of monetary or material Support:** Amgen Inc.

## Intervention

**Keyword:** AMG 531, Extension, MDS, safety

## Outcome measures

### Primary outcome

The incidence of all adverse events including clinically significant changes in

laboratory values and incidence of antibody formation

### Secondary outcome

• The incidence of bleeding events.

• The incidence of platelet transfusions.

• The duration of platelet response.

# Study description

### Background summary

Title of the study:
An Open Label Extension Study Evaluating the Safety of Long Term Dosing of AMG 531 in Thrombocytopenic Subjects with Myelodysplastic Syndromes (MDS)

Background of the study:
Myelodysplastic syndrome (MDS)is a heterogenous group of clonal disorders in the hematopoetic stem cell characterised by a varying degree of cytopenia in one or more cell lines. Prognosis can be expressed by the International Prognostic Scoring System (IPSS) score. Patients with low or intermediary-1 risk according to the IPSS are often treated with supportive care. About 30% of these patients have trombocytopenia at presentation, in the others it develops during the course of the disease. Thrombocytopenia can give rise to bleeding, often of the oral mucosa, and spontaneous hematomas. This negatively affects the quality of life of these patients. Thrombocyte transfusions are only effective for a few days and are given only in case of serious bleeding. Other than a thrombocyte transfusion, a thrombocytopenia cannot be treated at the moment. AMG 531 is a recombinantly expressed protein in E. Coli which offers a possible new treatment for thrombocytopenia in MDS patients.

### Study objective

To provide long-term safety data for the use of AMG 531 in thrombocytopenic subjects with IPSS low or intermediate-1 risk MDS.

### Study design

This is an open label extension study. Patients who have participated in the AMG 531 20050159 or 20060198 study and fulfil the protocol specified in- and exclusion criteria are allowed to participate in this open label extension study.
Patients will enter the screening when patients have signed and dated the Informed Consent Form. If patients meet the participation criteria, the treatment phase will start. In this period, the actual treatment with AMG 531 takes place. It will last at most until June 2009. Patients will visit the hospital weekly during this treatment phase. Patients will need to return one week and four weeks after their last administration of AMG 531 to close the study.

### Intervention

QW or Q2W Injections with AMG 531

### Study burden and risks

Patients will visit the hospital every week. At the first and last visit, a bone marrow aspirate and biopsy will be done. Physical examination and laboratory evaluations will be regularly done. At the first visit, a pregnancy test, if applicable, will be done.
AMG 531 has been studied in 2 phase 1 studies in healthy volunteers and 2 phase 2 studies in ITP patients. There is also an ongoing open label extension study in patients with ITP. To date no neutralizing antibodies to endogenous TPO have been detected. In healthy volunteers the most common adverse events were headache, fatigue and flu-like symptoms. In ITP patients these were headache, fatigue and epistaxis. In patients with ITP an increase of bone marrow reticulin was reported.

## Contacts

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**Scientific**

Amgen

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# Trial sites

## Listed location countries

Netherlands

# Eligibility criteria

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

## Inclusion criteria

Completion of an end of study visit in AMG 531 study 20050159 or 20060198 study for the treatment of thrombocytopenia in subjects with IPSS low to int-1 MDS.

## Exclusion criteria

Evidence of progression/transformation of disease
Prior history of leukemia or aplastic anemia
Prior histor of bone marrow or stem cell transplantation
Receipt of hypomethylating agents or immunomodulating agents, high-dose chemotherapy targeted at MDS, or histone deacetylase inhibitors

# Study design

## Design

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| Study phase: | 2 |
| Study type: | Interventional |

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

### Recruitment

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| Recruitment status: | Pending |
| Start date (anticipated): | 01-09-2007 |
| Enrollment: | 2 |
| Type: | Anticipated |

## Ethics review

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| Approved WMO Date: | 22-08-2007 |
| Application type: | First submission |
| Review commission: | CMO regio Arnhem-Nijmegen (Nijmegen) |
| Approved WMO Date: | 15-11-2007 |
| Application type: | Amendment |
| Review commission: | CMO regio Arnhem-Nijmegen (Nijmegen) |
| Approved WMO Date: | 22-04-2008 |
| Application type: | Amendment |
| Review commission: | CMO regio Arnhem-Nijmegen (Nijmegen) |
| Approved WMO Date: | 26-11-2008 |
| Application type: | Amendment |
| Review commission: | CMO regio Arnhem-Nijmegen (Nijmegen) |
| Approved WMO Date: | 12-03-2009 |
| Application type: | Amendment |
| Review commission: | CMO regio Arnhem-Nijmegen (Nijmegen) |
| Approved WMO Date: | 07-07-2009 |

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| Application type: | Amendment |
| Review commission: | CMO regio Arnhem-Nijmegen (Nijmegen) |
| Approved WMO Date: | 07-10-2009 |
| Application type: | Amendment |
| Review commission: | CMO regio Arnhem-Nijmegen (Nijmegen) |
| Approved WMO Date: | 27-01-2010 |
| Application type: | Amendment |
| Review commission: | CMO regio Arnhem-Nijmegen (Nijmegen) |
| Approved WMO Date: | 25-02-2010 |
| Application type: | Amendment |
| Review commission: | CMO regio Arnhem-Nijmegen (Nijmegen) |
| Approved WMO Date: | 14-04-2010 |
| Application type: | Amendment |
| Review commission: | CMO regio Arnhem-Nijmegen (Nijmegen) |
| Approved WMO Date: | 14-10-2010 |
| Application type: | Amendment |
| Review commission: | CMO regio Arnhem-Nijmegen (Nijmegen) |
| Approved WMO Date: | 22-10-2010 |
| Application type: | Amendment |
| Review commission: | CMO regio Arnhem-Nijmegen (Nijmegen) |
| Approved WMO Date: | 09-11-2010 |
| Application type: | Amendment |
| Review commission: | CMO regio Arnhem-Nijmegen (Nijmegen) |
| Approved WMO Date: | 14-03-2011 |
| Application type: | Amendment |
| Review commission: | CMO regio Arnhem-Nijmegen (Nijmegen) |
| Approved WMO Date: | 07-06-2011 |

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| Application type: | Amendment |
| Review commission: | CMO regio Arnhem-Nijmegen (Nijmegen) |

# 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 |
|---|---|
| EudraCT | EUCTR2007-001516-24-NL |
| CCMO | NL17901.091.07 |