

# A phase Ib/IIa clinical trial to investigate the safety and efficacy of recombinant human soluble Fc-gamma receptor IIb (SM101) for intravenous application in the treatment of patients with chronic adult idiopathic thrombocytopenic purpura (ITP)

<b>Submission date</b> 04/02/2010	<b>Recruitment status</b> No longer recruiting	<input type="checkbox"/> Prospectively registered
<b>Registration date</b> 18/05/2010	<b>Overall study status</b> Completed	<input type="checkbox"/> Protocol
<b>Last Edited</b> 19/04/2011	<b>Condition category</b> Haematological Disorders	<input type="checkbox"/> Statistical analysis plan
		<input type="checkbox"/> Results
		<input type="checkbox"/> Individual participant data
		<input type="checkbox"/> Record updated in last year

**Plain English summary of protocol**  
Not provided at time of registration

## Contact information

**Type(s)**  
Scientific

**Contact name**  
Mr Sascha Tillmanns

**Contact details**  
Am Klopferspitz 19  
Martinsried/ Munich  
Germany  
82152

## Additional identifiers

**Protocol serial number**  
SM101-201-itp-09

## Study information

## **Scientific Title**

A randomised, multicentre, double-blind, placebo-controlled, single/multiple dose escalation phase Ib/IIa clinical trial to investigate the safety and efficacy of recombinant human soluble Fc-gamma receptor IIb (SM101) for intravenous application in the treatment of patients with chronic adult idiopathic thrombocytopenic purpura (ITP)

## **Study objectives**

The primary objective of this trial is to evaluate the safety and tolerability of intravenously administered SM101 at various dose levels in a single/multiple dosing manner in subjects with chronic idiopathic thrombocytopenic purpura (ITP).

The secondary objective of this trial is to evaluate the efficacy, e.g. platelet count, pharmacokinetic and immunological parameters of intravenous (iv) administered SM101.

As of 19/04/2011 the anticipated end date for this trial has been extended from 31/01/2011 to 26/10/2012.

## **Ethics approval required**

Old ethics approval format

## **Ethics approval(s)**

1. Russia: Ethics Committee of the Federal Service on Surveillance in Health Care and Social Development approved on the 21st October 2009
2. Belgium: K.U. LEUVEN Hospitals Medical Ethics Committee approved on the 6th January 2010
3. Poland: Komisja Bioetyczna Slaskiego Uniwersytetu Medycznego w Katowicach approved on the 5th January 2010
4. Germany: Ethik-Kommission des Fachbereichs Medizin der Justus-Liebig Universitaet Giessen approved on the 14th January 2010
5. Ukraine: Ministry of Health of Ukraine, Central Ethics Committee approved on the 1st June 2010

## **Study design**

Randomised multicentre double blind placebo-controlled dose escalation and extension trial

## **Primary study design**

Interventional

## **Study type(s)**

Treatment

## **Health condition(s) or problem(s) studied**

Chronic adult idiopathic thrombocytopenic purpura

## **Interventions**

SM101 is a recombinant, soluble, non-glycosylated version of the human Fc- receptor Fc-RIIb.

This trial consists of a dose escalation and an extension part. In the dose escalation part, each dose group consists of 4 verum and 2 placebo patients who will receive verum or placebo as a single administration. The patients will be followed up for 4 weeks and will then continue with 4 weekly injections of verum or placebo. With an additional follow-up period of 12 weeks, the patients of one dose group complete their study participation.

Extension part:

The extension part will be conducted with an additional 15 newly recruited patients (10 verum, 5 placebo) in a parallel fashion and the subjects will receive a total of 4 IMP administrations. The subjects will then be followed for 3 months.

## **Intervention Type**

Drug

## **Phase**

Phase I/II

## **Drug/device/biological/vaccine name(s)**

Recombinant human soluble Fc-gamma receptor IIb (SM101)

## **Primary outcome(s)**

Incidence, severity, causality and seriousness of adverse events (AEs), laboratory results and AEs of special interest including bleeding events. AEs will be graded using the Common Terminology Criteria for Adverse Events (CTCAE), version 3.0.

Adverse Events will be recorded at an ongoing basis at every visit performed throughout the study (dose escalation part: screening, week 1, 2, 3, 4, 5, 6, 7, 8, 10, 12, 14, 16, 18, 20; extension part: screening, week 1, 2, 3, 4, 5, 8, 12, 16).

## **Key secondary outcome(s)**

1. Proportion of subjects with a substantial platelet response defined as:
  - 1.1. Platelet count greater than or equal to 30,000/ $\mu$ L from at least two blood samples, and
  - 1.2. At least two-fold increase of platelet count from baseline values from at least two blood samples
2. The following will be defined as efficacy end-points:
  - 2.1. Mean time to reach substantial platelet response
  - 2.2. Mean duration of platelet count greater than or equal to 30,000/ $\mu$ L
  - 2.3. Mean time to reach first platelet count of greater than or equal to 30,000/ $\mu$ L
  - 2.4. Proportion of subjects requiring rescue medication until end of study
  - 2.5. Proportion of subjects with World Health Organisation (WHO) bleeding events grade 2 (mild blood loss) or higher until end of study
  - 2.6. Immunological markers and IMP PK parameters

The platelet count will be determined at every visit performed throughout the study (dose escalation part: screening, week 1, 2, 3, 4, 5, 6, 7, 8, 10, 12, 14, 16, 18, 20; extension part: screening, week 1, 2, 3, 4, 5, 8, 12, 16). On treatment days (escalation part: days 1, 36, 43, 50 and 47; extension part: days 1, 8, 15 and 22) the platelet count will be measured 0.5 hours prior to dosing and 2, 4, 12, 24 and 48 hours after start of IMP administration.

## **Completion date**

26/10/2012

## **Eligibility**

### **Key inclusion criteria**

1. Written informed consent prior to any study related procedure
2. Male or female subjects aged 18 to 75 years, with or without splenectomy
3. Diagnosis of chronic idiopathic thrombocytopenic purpura (ITP) based on subject's history, physical examination, blood count and blood film examination according to the British Society for Haematology (BSH) and American Society of Hematology (ASH) guidelines for at least 6 months
4. Subject has previously received at least one ITP therapy
5. Platelet count less than 30,000/ $\mu$ L from at least two measurements
6. Subjects greater than 60 years of age must have had a documented history of chronic ITP with a bone marrow report to confirm the diagnosis

**Participant type(s)**

Patient

**Healthy volunteers allowed**

No

**Age group**

Adult

**Lower age limit**

18 years

**Sex**

All

**Key exclusion criteria**

1. Female subjects who are nursing or pregnant, who may be pregnant, or who contemplate pregnancy during the study period
2. Secondary thrombocytopenia
3. Subject received rituximab or any other B-cell depleting agent within 24 months preceding the first dose of investigational medical product (IMP)
4. All other previously completed ITP treatment must achieve at least 5 times their terminal half-life prior to first administration of IMP
5. Subject receives concomitant ITP medication other than corticosteroids, except rescue medication during the clinical trial
6. Splenectomy within 4 weeks prior to screening
7. History of or current alcohol or drug abuse
8. Any condition which in the judgment of the Investigator would place the subject at undue risk or interfere with the results of the study

**Date of first enrolment**

07/04/2010

**Date of final enrolment**

26/10/2012

**Locations**

## **Countries of recruitment**

Belgium

Germany

Poland

Russian Federation

Ukraine

## **Study participating centre**

**Am Klopferspitz 19**

Martinsried/ Munich

Germany

82152

## **Sponsor information**

### **Organisation**

SuppreMol GmbH (Germany)

### **ROR**

<https://ror.org/05jgtkc28>

## **Funder(s)**

### **Funder type**

Industry

### **Funder Name**

SuppreMol GmbH (Germany)

## **Results and Publications**

### **Individual participant data (IPD) sharing plan**

### **IPD sharing plan summary**

Not provided at time of registration