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Clinical study to evaluate the safety and tolerability of immunoglobulin intravenous (human) 10% (NewGam) administered at high infusion rates to patients with primary immunodeficiency diseases

Submission date
08/03/2011

Recruitment status
No longer recruiting

Registration date
16/03/2011

Overall study status
Completed

Last Edited
15/03/2019

Condition category
Other

[_] Prospectively registered
[_] Protocol
[_] Statistical analysis plan
[X] Results
[_] Individual participant data

Plain English Summary

Background and study aims
This study aims to find a treatment for Primary Immunodeficiency Diseases (PID). According to the leading experts in immunology, when part of the immune system (body mechanism that protects the body against infection caused by bacteria, viruses, fungi or parasites) is either absent or not functioning properly, it can result in an immune deficiency disease. When the cause of this deficiency is hereditary or genetic, it is called a primary immunodeficiency disease (PID). One of the most common signs of PID is an increased susceptibility to infections. You may have infections that are more frequent, longer lasting or harder to treat than are the infections of someone with a normal immune system. You may also get infections that a person with a healthy immune system likely wouldn't get (called opportunistic infections).
One of the common treatments for PID is Immunoglobulin therapy. Immunoglobulin consists of antibody proteins needed for the immune system to fight infections. It can be injected into a vein through an intravenous (IV) line. Treatment with IV immunoglobulin is needed every few weeks to maintain sufficient levels of antibodies in your blood.

Who can participate?
You may only participate in the NGAM-05 study if you completed the NGAM-01 study and did not notice any side effects during your last few IV immunoglobulin infusions of that study.

What does the study Involve?
This study, named NGAM-05, is an extension of the NGAM-01 study that was testing a new formulation of IV Immunoglobulin called NewGam. In the NGAM-05 study we are testing the same NewGam and you will receive the same dose as in NGAM-01. The difference is that the NewGam is given to you at a faster speed and during a shorter time than the usual medicine is given to you. You will receive either four infusions if on a 4 week infusion schedule, or five infusions if on a 3-week infusion schedule, and a follow-up visit.

What are the possible benefits and risks of participating?
The benefit of being in this study is that your infusions will go faster than normal, and your visit to the doctors office will take less time than normal.
The risks of being in this study are that you may experience more side effects than normal. It is felt that the faster you get the medicine, usually the risk of side effects goes up. The most common side effects when people take IV immunoglobulin are headache, chills, migraine, dizziness, fever, nausea/vomiting, fatigue, faster heart rate, Itching, upper abdominal pain, rash /hives, increased blood pressure and cough.

Where is the study run from?
This study is being run at six different centers in the USA.

When is the study starting and how long is it expected to run for?
This study started in May 2011 and is expected to last until September 2012.

Who is funding the study?
Octapharma.

Who is the main contact?
Barbara Pyringer
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Contact information

Type(s)
Scientific

Contact name
Dr Wolfgang Frenzel

Contact details
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Additional identifiers

EudraCT/CTIS number
2011-005015-82

IRAS number

ClinicalTrials.gov number
NCT01313507

Secondary identifying numbers
NGAM-05 (extension of study NGAM 01)

Study information

Scientific Title
Clinical study to evaluate the safety and tolerability of immunoglobulin intravenous (human) 10% (NewGam) administered at high infusion rates to patients with primary immunodeficiency diseases: a prospective, open label, non-controlled, non-randomised, multicentre, phase III study

Study hypothesis
To assess the safety and tolerability of NewGam when administered at infusion rates from 0.08 mL/kg/min to 0.14 mL/kg/min

Extension of study NGAM 01 registered under http://www.controlled-trials.com /ISRCTN05425999

Ethics approval required
Old ethics approval format

Ethics approval(s)
1. Research and Clinical Trials Administration Office, Rush University Medical Parkway, approval granted on 04/03/2011
2. Western IRB, approval granted on 16/03/2011
3. St. Louis University Biomedical Institutional Review Board, approval granted on 05/04/2011
4. Institutional Review board, Seattle Childrens Hospital, approval granted on 22/12/2011
5. Office of Research Administration, University of California Irvine, approval granted on 08/04 /2011

Study design
Prospective open-label non-controlled non-randomised multicentre Phase III study

Primary study design
Interventional

Secondary study design
Other

Study setting(s)
Hospital

Study type(s)
Treatment

Participant information sheet
Not available in web format, please use the contact details below to request a patient information sheet

Condition
Primary immunodeficiency (PID) diseases

Interventions
Patients will receive NewGam every 3 or 4 weeks (+/-3 days) following the same dosing interval as in the main study NGAM 01. Patients who complete the present study will receive either 5 (at 3-week intervals) or 4 (at 4-week intervals) infusions of NewGam.

Intervention Type
Other

Phase
Phase III

Primary outcome measure
1. Occurrence of adverse events (AEs)
2. Occurrence of AEs temporally associated with the study treatment
3. Proportion of infusions with one or more temporally associated AEs
4. AEs by infusion rate
5. Short-term tolerance parameters including vital signs (blood pressure, heart rate, temperature, respiratory rate)
6. Laboratory parameters (haematology, clinical chemistry, direct Coombs' test, urinalysis, and tests for viral safety)

Secondary outcome measures
Quality of Life:
For QoL assessments, each patient will continue using the same questionnaire as before. That is, the parent or guardian of patients who were below 14 years of age when they entered the main study NGAM 01 will continue using the Child Health Questionnaire-Parent Form (CHQ-PF50), and patients who were ≥ 14 years of age when they entered the main study will continue using the SF-36 Health Survey

Overall study start date
15/03/2011

Overall study end date
30/06/2012

Eligibility

Participant inclusion criteria
1. Completion of the main study NGAM 01
2. At each of the last three infusions in the main study NGAM 01, administration of NewGam at the maximum infusion rate of 0.08 mL/kg/min and without the need for premedication
3. For adult patients: freely given written informed consent. For patients below the legal age of majority: freely given written informed consent from parents/legal guardians and written informed assent from the child/adolescent in accordance with the applicable approvals
4. For female patients of child-bearing potential, a negative result in a urine pregnancy test conducted at the screening visit
5. Willingness to comply with all aspects of the protocol, including blood sampling, for the duration of the study

Participant type(s)
Patient

Age group
Adult

Sex
Both

Target number of participants
20-35

Participant exclusion criteria
1. Any condition or circumstance that would have led to the exclusion of the subject from the NGAM 01 study
2. Administration of any immunoglobulin infusion other than NewGam between conclusion of the NGAM 01 study and the beginning of the present study
3. A deviation of the subjects treatment interval of more than 7 days between the last infusion of NewGam in the NGAM 01 study and the first infusion of NewGam in the present study

Recruitment start date
15/03/2011

Recruitment end date
30/06/2012

Locations

Countries of recruitment
Austria

United States of America

Study participating centre
Oberlaaer Str. 235
Vienna
Austria
1100

Sponsor information

Organisation
Octapharma AG (Switzerland)

Sponsor details
Seidenstrasse 2
Lachen
Switzerland
8853

Sponsor type

Industry

ROR
https://ror.org/002k5fe57

Funder(s)

Funder type
Industry

Funder Name
Octapharma AG (Switzerland)

Results and Publications

Publication and dissemination plan
Not provided at time of registration

Intention to publish date

Individual participant data (IPD) sharing plan

IPD sharing plan summary
Not provided at time of registration

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Basic results				No	No
Basic results				No	No