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Clinical study to evaluate the efficacy, pharmacokinetics and safety of immunoglobulin intravenous (human) 10% (NewGam) in patients with primary immunodeficiency diseases

Submission date 09/11/2009	**Recruitment status** No longer recruiting	[X] Prospectively registered [_] Protocol [_] Statistical analysis plan [_] Results [_] Individual participant data [_] Record updated in last year
Registration date 11/11/2009	**Overall study status** Completed	
Last Edited 12/11/2009	**Condition category** Haematological Disorders	

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

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Additional identifiers

Protocol serial number
NGAM-01

Study information

Scientific Title

Study objectives
To assess the efficacy of NewGam in preventing serious bacterial infections compared to historical control data.

Ethics approval required
Old ethics approval format

Ethics approval(s)
Saint Louis University Biomedical Institutional Review Board approved on the 15th September 2009 (ref: 16291)

Primary study design
Interventional

Study design
Prospective open-label non-controlled non-randomised multi-centre phase III study

Study type(s)
Treatment

Health condition(s) or problem(s) studied
Primary immunodeficiency diseases (PID)

Interventions
The treatment intervals with NewGam will be documented over 12 months: every 3 or every 4 weeks (+/- 3 days) following the same dosing interval as the previous commercial IVIG infusions. Therefore, it is anticipated that each patient will be administered either 17 (at 3-week intervals) or 13 (at 4-week intervals) infusions of NewGam.

Intervention Type
Drug

Phase
Phase III

Drug/device/biological/vaccine name(s)
NewGam

Primary outcome(s)
To assess the efficacy of NewGam in preventing serious bacterial infections compared to historical control data, measured throughout the 12-month treatment period.

Key secondary outcome(s)
1. To evaluate the safety of NewGam, measured throughout the 12-month treatment period
2. To determine the pharmacokinetic (PK) profile of NewGam, measured on the 9th (or soonest subsequent) NewGam infusion day for patients on the 3-week schedule, or on the 7th (or soonest subsequent) NewGam infusion day for patients on the 4-week schedule
3. To assess the effect of NewGam on quality of life (QoL) measures, measured using the Child Health Questionnaire (CHQ-PF50) (completed by a parent or guardian of patients less than 14

years of age) or the 36-item short form health survey (SF-36) in patients greater than or equal to 14 years of age. These will be completed at the first and last infusion visits and at intervals of 3 months, i.e. at the 5th, 10th and 14th infusion days for patients on the 3-week schedule, or on the 4th, 8th and 11th infusion days for patients on the 4-week schedule.

Completion date
01/04/2011

Eligibility

Key inclusion criteria
1. Aged greater than or equal to 2 years and less than or equal to 75 years, either sex
2. For minor patients, above a minimum weight based on the amount of blood required for testing: per individual, the trial-related blood loss (including any losses in the manoeuvre) should not exceed 3% of the total blood volume during a period of 4 weeks and should not exceed 1% at any single time (the total volume of blood is estimated at 80 ml/kg body weight)
3. Confirmed diagnosis of common variable immunodeficiency (CVID) or X-linked agammaglobulinaemia (XLA)
4. Previously treated with a commercial immune globulin intravenous (human) every 21 - 28 days for at least 6 infusion intervals at a constant dose between 200 and 800 mg/kg body weight
5. Availability of the immunoglobulin G (IgG) trough levels of the two previous infusions before enrolment, and maintenance of at least 5.5 g/l in the trough levels of these two infusions
6. Negative result on a pregnancy test (human chorionic gonadotrophin [HCG]-based assay in urine) for women of childbearing potential and use of a reliable method of contraception for the duration of the study
7. For adult patients: freely given written informed consent. For minor patients: freely given written informed consent from parents/legal guardians, and written informed assent from the child/adolescent in accordance with the applicable approvals.
8. Willingness to comply with all aspects of the protocol, including blood sampling, for the duration of the study

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Other

Sex
All

Key exclusion criteria
1. Acute infection requiring intravenous antibiotic treatment within 2 weeks prior to and during the screening period
2. Known history of adverse reactions to immunoglobulin A (IgA) in other products
3. Exposure to blood or any blood product or derivative, other than commercially available intravenous immunoglobulin (IVIG), within the past 3 months prior to enrolment
4. Ongoing history of hypersensitivity or persistent reactions to blood or plasma derived products, or any component of the investigational product

5. Requirement of any routine pre-medication for IVIG infusion
6. History of congenital impairment of pulmonary function
7. Severe liver function impairment (alanine aminotransferase [ALAT] 3 x upper limit of normal)
8. Presence of renal function impairment (creatinine greater than 120 µmol/L), or predisposition for acute renal failure (e.g. any degree of pre-existing renal insufficiency or routine treatment with known nephritic drugs)
9. History of autoimmune haemolytic anaemia
10. History of diabetes mellitus
11. Congestive heart failure New York Heart Association (NYHA) class III or IV
12. Non-controlled arterial hypertension (systolic blood pressure greater than 160 mmHg or diastolic blood pressure greater than 90 mmHg)
13. History of deep vein thrombosis or thrombotic complications of IVIG therapy
14. A positive result at screening on any of the following viral markers: human immunodeficiency virus (HIV), hepatitis C virus (HCV), hepatitis B virus (HBV)
15. Presence of any clinically relevant disease or unstable condition at screening, other than PID, which in the opinion of the investigator could interfere with the conduct of the study
16. Treatment with steroids (oral or parenteral, long-term, i.e. 30 days or more, not intermittent or burst, daily, greater than or equal to 0.15 mg of prednisone or equivalent/kg/day), immunosuppressive or immunomodulatory drugs
17. Planned vaccination during the study period
18. Treatment with any investigational agent within 3 months prior to enrolment
19. Known or suspected to abuse alcohol, drugs, psychotropic agents or other chemicals within the past 12 months prior to enrolment
20. Pregnant or nursing women

Date of first enrolment
01/12/2009

Date of final enrolment
01/04/2011

Locations

Countries of recruitment
Austria

Germany

Poland

United States of America

Study participating centre
Oberlaaerstrasse 235
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Sponsor information

Organisation
Octapharma AG (Switzerland)

ROR
https://ror.org/002k5fe57

Funder(s)

Funder type
Industry

Funder Name
Octapharma AG (Switzerland)

Results and Publications

Individual participant data (IPD) sharing plan

IPD sharing plan summary
Not provided at time of registration