ISRCTN55938644 https://doi.org/10.1186/ISRCTN55938644

Comparison of quality of life and satisfaction in primary immunodeficient patients treated with subcutaneous injections of Gammanorm administered using a pump or a syringe for rapid manual administration

Submission date
02/09/2015

Recruitment status
No longer recruiting

Registration date
15/12/2015

Overall study status
Completed

Last Edited
28/06/2019

Condition category
Haematological Disorders

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

Plain English summary of protocol

Background and study aims
Primary immunodeficiency disorders (PID) are caused by an inherited defect in the immune system, which makes a person more susceptible to infection. There are more than 200 of these disorders, the symptoms of which can vary greatly, ranging from very mild or non-existent (asymptomatic) to severe and debilitating. Gamma globulins are proteins found in the blood plasma. There are different types of gamma globulins, but the most important are immunoglobulins, also known as antibodies. The immune system uses antibodies to recognize and fight infections. In many of the PID's, people do not have enough of these gamma globulins in their blood, and so are given injections of immunoglobulins to help strengthen their immune systems. When a patient receives this treatment, the injections are often done by the patient themselves at home. The injections can be given using a syringe or an automatic pump (which delivers the dose automatically over a period of time). This study aims to find out whether patients are happier using a syringe or an automatic pump method for receiving their injections.

Who can participate?
Adults with primary immunodeficiency who have received injections of immunoglobulin at use using an automatic pump or syringe for at least one month.

What does the study involve?
Participants are randomly assigned into two groups, each of which receives the treatments in a different order. Each patient is treated for three months with the first treatment option, and then treated for 3 months with the second treatment option (i.e. syringe and then pump, or pump and then syringe). Patients complete a questionnaire about how satisfied they are with each of the treatment options at the end of each three month treatment period.

What are the possible benefits and risks of participating?
Participants will benefit from learning a new technique for administering their immunoglobulin treatment at home, which could be preferable than their current technique. Risks of participating involve the minor risks associated with repeated blood tests, such as pain, bruising or infection.

Where is the study run from?
1. University Medical Center Freiburg (Germany)
2. Klinikum St. Georg (Germany)
3. University Hospital of Wales (UK)
4. Derriford Hospital (UK)
5. The Royal London Hospital (UK)
6. University Hospitals Birmingham (UK)
7. Padova Hospital (Italy)
8. John Radcliffe Hospital, Oxford (UK)
9. Royal Free London Hospital (UK)
10. Campbell Town Hospital (Australia)
11. Canberra Hospital (Australia)
12. Università di Roma "Sapienza", Policlinic Umberto I, Rome (Italy)

When is the study starting and how long is it expected to run for?
November 2014 to December 2017

Who is funding the study?
Octapharma (Austria)

Who is the main contact?
Tatiana Lavrova
tatiana.lavrova@octapharma.com

Contact information

Type(s)
Scientific

Contact name
Dr Tatiana Lavrova

Contact details
Octapharma Pharmazeutika Prod.Ges.m.b.H.
Oberlaaer Strasse 235
A-1100 Vienna
Austria
1100

Additional identifiers

ClinicalTrials.gov (NCT)
NCT02503293

Clinical Trials Information System (CTIS)
2014-003746-27

Protocol serial number
GAN-06

Study information

Scientific Title
A randomised, cross-over study to compare quality of life and satisfaction in primary immunodeficient patients treated with subcutaneous injections of Gammanorm® 165 mg/mL administered with two different delivery devices: injections using pump or rapid push

Acronym
GAN-06

Study objectives
The administration of Gammanorm 165 mg/ml using a syringe is not inferior to the administration of Gammanorm 165 mg/ml using a pump regarding patient satisfaction.

Ethics approval required
Old ethics approval format

Ethics approval(s)
1. Central Ethics Committee, Albert Ludwig University(Germany), 09/02/2015, ref: 561/14(FF-MC)
2. Research Ethics Service, Wales (UK), 12/06/2015, ref: 15/WA/0047
3. Comitato Etico per la Sperimentazione Clinica della Provincia di Padova, 21/04/2016, ref: NRC AOP0707
4. ACT Health, Research Ethics and Governance Office, 21/06/2016, ref: ETH.6.16.113E
5. South Western Sydney Local Health District, 30/05/2016; ref: HREC/16/LPOOL/44
6. Ethics Committee University "Sapienza", 03/03/2017, ref: 4359

Study design
A non-inferiority comparative interventional multi-centre prospective longitudinal randomised open-label cross-over study

Primary study design
Interventional

Study type(s)
Quality of life

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

Interventions
Participants are randomly allocated to one of two groups, who receive the two different treatments in a different order (pump then syringe or syringe then pump). Treatment will be administered subcutaneously at home by the patient.

Pump treatment: The usual dose is 0.6 mL (100 mg) of Gammanorm® 165 mg/mL per kg of body weight once a week, which can be administered at several infusion sites.

Syringe treatment: The usual dose is 0.6 mL (100 mg) of Gammanorm® 165 mg/mL per kg of body weight per week. The weekly dose could be divided into three injections administered every other day at a single infusion site.

Participants use the pump treatment and the syringe treatment for a total of three months, with no wash-out period in between.

Intervention Type
Drug

Phase
Phase III/IV

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

Primary outcome(s)
Patient satisfaction is measured using the life quality index (LQI) questionnaire at the end of each 3 month treatment period.

Key secondary outcome(s))
1. Therapy-related problems and therapy setting is measured using LQI sub-scores at baseline, 3 months and 6 months
2. Patient satisfaction is measured using the Treatment Satisfaction Questionnaire for Medication (TSQM-11) at baseline, 3 months and 6 months
3. Patient preference is measured by patient answer at 6 months
4. Efficacy (clinical efficacy and residual levels of IgG) of Gammanorm® 165 mg/mL is measured by evaluation of IgE level at baseline, 3 months and 6 months
5. Systemic and local tolerability of Gammanorm® 165 mg/mL is measured by evaluation of the diary and AE Reporting during ongoing study
6. Burden of illness is measured using the PRISM test at at baseline, 3 months and 6 months
7. Burden of subcutaneous immunoglobulin treatment delivery device is measured using the PRISM test at at baseline, 3 months and 6 months
8. Costs measured by analyses of the patient diary at 6 months

Completion date
11/12/2017

Eligibility

Key inclusion criteria
1. Adult patients (≥ 18 years).
2. Presenting with primary immunodeficiency.
3. Having received subcutaneous injections of immunoglobulin at home using an automatic pump or syringe for at least 1 month at the time of inclusion.
4. For whom the investigator decides to maintain immunoglobulin replacement therapy with subcutaneous injections of Gammanorm® 165 mg/mL at home.
5. Women of childbearing potential must have a negative result on a pregnancy test (human

chorionic gonadotropine [HCG]-based assay) and need to practice contraception using a method of proven reliability for the duration of the study

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Adult

Lower age limit
18 years

Sex
All

Total final enrolment
30

Key exclusion criteria
Participating in another interventional clinical study and receiving investigational medicinal product within three months before study entry.

Date of first enrolment
25/06/2015

Date of final enrolment
14/07/2017

Locations

Countries of recruitment
United Kingdom

England

Wales

Australia

Germany

Italy

Study participating centre
University Medical Center Freiburg
Centre of Chronic Immunodeficiency

Breisacher Straße 117
Freiburg
Germany
79106

Study participating centre
Klinikum St. Georg
Delitzscher Street 141
Leipzig
Germany
04129

Study participating centre
University Hospital of Wales
Dept. of Biochemistry & Immunology
Heath Park
Cardiff
United Kingdom
CF14 4XW

Study participating centre
Derriford Hospital
Department of Immunology and Allergy
Eden Unit, level 7
Derriford Road
Plymouth
United Kingdom
PL6 8DH

Study participating centre
The Royal London Hospital
Barts Health NHS Trust
4th Floor Pathology & Pharmacy Building
80 Newark Street
London
United Kingdom
E1 2ES

Study participating centre
University Hospitals Birmingham
Department of Immunology

Mindelsohn Way
Edgbaston
Birmingham
United Kingdom
B15 2GW

Study participating centre
Padova Hospital (Azienda Ospedaliera di Padova)
Dipartimento di Medicina (DIMED)
Via N. Giustiniani, 2
Padova
Italy
35128

Study participating centre
John Radcliff Hospital
Department of Clinical Immunology
Oxford
United Kingdom
OX3 9DU

Study participating centre
Royal Free London Hospital
Department of Immunology
London
United Kingdom
NW3 2QG

Study participating centre
Campell Town Hospital
Unit Immunology and Allergy
Therry Road
Campell Town
Australia
NSW 2560

Study participating centre
Canberra Hospital
Yamba Dr.

Garran
Australia
ACT 2605

Study participating centre
Università di Roma "Sapienza"
Policlinic Umberto I
Viale del Policlinico, 155
Rome
Italy
00161

Sponsor information

Organisation
Octapharma Pharmazeutika Prod.Ges.m.b.H.

ROR
https://ror.org/022k50n33

Funder(s)

Funder type
Industry

Funder Name
Octapharma

Results and Publications

Individual participant data (IPD) sharing plan

IPD sharing plan summary
Not expected to be made available

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Basic results		08/02/2019	08/02/2019	No	No
HRA research summary			28/06/2023	No	No