ISRCTN32265704 https://doi.org/10.1186/ISRCTN32265704

Study regarding the safety of Gammanorm in autoimmune diseases

Submission date	Recruitment status	[X] Prospectively registered
15/06/2016	No longer recruiting	[_] Protocol
Registration date	**Overall study status**	[_] Statistical analysis plan
05/07/2016	Completed	[_] Results
Last Edited	**Condition category**	[_] Individual participant data
01/07/2016	Skin and Connective Tissue Diseases	[_] Record updated in last year

Plain English summary of protocol

Background and study aims
Immunotherapy is a drugs therapy that can be used to treat diseases that affect the immune system. Gammanorm® is a immunoglobulin and contains antibodies against bacteria and viruses. It is used as a replacement antibody therapy in both adults and children to treat a number of immune system diseases, for example, severe combined immunodeficiency, common variable immunodeficiency (CVID), IgG subclass deficiencies and myeloma or chronic lymphatic leukaemia with severe secondary hypogammaglobulinaemia and recurrent infections. However, it can also be used “off label” in treating other conditions (that is, conditions it has not been licenced to treat). The main name of this study is to assess the safety of Gammanorm® for “off-label” uses.

Who can participate?
Adults aged at least 18 and treated with Gammanorm® for an autoimmune disease, such as chronic inflammatory demyelinating polyradiculoneuropathy, multifocal motor neuropathy, dermatomyositis , polymyositis, inclusion body myositis, idiopathic thrombocytopenic purpura , and necrotizing myopathy.

What does the study involve?
Participants are treated with Gammanorm® from between a few months to several years, depending on their condition, typically being given the drug twice a week. The follow-up period for each patient varies, depending on how long they receive Gammanorm® , but is for the maximum of two years.
During this period, information on adverse effects of taking the drug, whether the participants stick to the treatment regime and how well the treatment works is collected.

What are the possible benefits and risks of participating?
Not provided at time of registration

Where is the study run from?
1. Hospital Center University Rouen (France)
2. University Hospital Of Martinique (France)

When is the study starting and how long is it expected to run for?
January 2015 to December 2020

Who is funding the study?
Octapharma France

Who is the main contact?
Dr Jean-Christophe Delain

Contact information

Type(s)
Scientific

Contact name
Dr Jean-Christophe Delain

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Additional identifiers

EudraCT/CTIS number

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers
GAN-08

Study information

Scientific Title
Prospective, observational, multicenter study regarding the safety of Gammanorm in autoimmune diseases

Acronym
Immunorm Study

Study objectives
The marketing authorization of SCIg (human normal immunoglobulin), especially Gammanorm® is currently restricted to immunosubstitution indications. However, pharmacovigilance show SCIg use in immunomodulation indication, in the absence of a therapeutic alternative to replacing IVIg tolerance reasons. Thus the main objective of this study is to regulate this practice in accordance with our obligations evaluating the safety of treatment with Gammanorm® in various off-label uses.

Ethics approval required
Old ethics approval format

Ethics approval(s)
No Ethical approval is needed in such study. Only the "CCTIRS" (evaluation of collected data) and "CNIL" (evaluation and safety of the data/database) is needed in France for an observational project.

Study design
Observational prospective, multicenter study

Primary study design
Observational

Secondary study design
Longitudinal study

Study setting(s)
Hospital

Study type(s)
Treatment

Participant information sheet

Health condition(s) or problem(s) studied
Autoimmune diseases which needs an immunoglobulin treatment - including dermatomyositis, polymyositis, inclusion body myositis, MMN, CIDP

Interventions
The study will be conducted in adult patients (>18 years old) treated with Gammanorm® for a autoimmune disease, such as chronic inflammatory demyelinating polyradiculoneuropathy, multifocal motor neuropathy, dermatomyositis , polymyositis, inclusion body myositis, idiopathic thrombocytopenic purpura , necrotizing myopathy or any other off-label use. The main objective is safety so the appearance of adverse events will be noted.

Gammanorm® is a subcutaneous polyvalent immunoglobulins preparation at 16,5% (165mg/ml. Typical dosage is around 2 g/kg/month. Typical frequency of administration is twice a week. Treatment duration depends on the patient and could be anywhere from a few months to several years. The follow-up period also depends on how long the patient has been treated for but the maximum follow-up period is two years.

Intervention Type

Primary outcome measure
Occurrence of adverse events, by counting and evaluating these events as soon as they occur.

The physician completes the Adverse event declaration page in the eCRF and this adverse event is automatically declared in pharmacovigilance.
information coklected includes:

1. Type of event
2. The outcomes
3. Severity
4. Duration
5. The link with Gammanorm
6. Actions (stop of the treatment, corrective treatment,...)

Secondary outcome measures
1. Efficacy of treatment:
1.1. For ITP (Immune Thrombocytopenic Purpura) : SMOG Score and echography of spleen
1.2. For NMM (Multifocal motor neuropathy) and CIDP (Chronic Inflammatory Demyelinating Polyneuropathy) : Rankin modified score, and MRC (Medical research council of Great Britain) and ONLS (Overall Neuropathy Limitation Scale)
1.3. For dermatomyositis, Polymyositis or Inclusion body myositis or necrotic myositis : a muscular testing, a myostis functional scale
2. Treatment compliance and therapeutic regimen, monitored using the following data:
2.1. Dosage for a month
2.2. Number of perfusions during a week
2.3. Dosage (in g/kg)
2.4. Speed infusion
2.5. Batch numbers
2.6. Number of vials since the last visit
3. Evaluate patient satisfaction (LQI scale) and quality of life (SF12 scale)

Measured at baseline and then every 3 months and at study end.

Overall study start date
22/01/2015

Completion date
31/12/2020

Eligibility

Key inclusion criteria
1. Adult patient
2. Patient with an autoimmune disease like CIDP, MMN, Polymyositis, Dermatomyositis, Inclusion body myositis, necrotizing myopathy or other autoimmune dideases
3. Patient treated by Gammanorm®
4. Patient had given oral consent after patient information note

Participant type(s)
Patient

Age group
Adult

Sex
Both

Target number of participants

80

Key exclusion criteria
1. Patient with Gammanorm® and with primary or secondary immunodeficiency
2. Patient does not wish to participate

Date of first enrolment
01/09/2016

Date of final enrolment
01/09/2018

Locations

Countries of recruitment
France

Study participating centre
Hospital Center University Rouen
1 Rue de Germont
Rouen
France
76000

Study participating centre
University Hospital Of Martinique
France
97221

Sponsor information

Organisation
Octapharma France

Sponsor details
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Sponsor type
Industry

ROR
https://ror.org/02xqq7c82

Funder(s)

Funder type
Industry

Funder Name
Octapharma France

Results and Publications

Publication and dissemination plan
A publication is expected after the clinical study report of the study : expected end of 2020
abstracts and posters will be made to communicate on progress: depending of inclusions

Intention to publish date
31/12/2020

Individual participant data (IPD) sharing plan

IPD sharing plan summary
Available on request