Effectiveness of Somatostatin Analogues in Patients with hereditary hemorrhagic telangiectasia and symptomatic gastrointestinal bleeding, the SAIPAN-trial: a multicenter, randomized, open-label, parallelgroup, superiority trial.

Published: 01-05-2019
Last updated: 21-12-2024

To determine if SSA are effective in decreasing transfusion requirements and improving quality of life while being cost-effective.

Ethical review	Approved WMO
Status	Completed
Health condition type	Gastrointestinal vascular conditions
Study type	Interventional

Summary

ID

NL-OMON46190

Source
ToetsingOnline

Brief title
SAIPAN

Condition

- Gastrointestinal vascular conditions

Synonym
Hereditary hemorrhagic teleangiectasia (HHT), Rendu-Osler-Weber syndrome

Research involving
Human

Sponsors and support

Primary sponsor: Radboud Universitair Medisch Centrum
Source(s) of monetary or material Support: ZonMW

Intervention

Keyword: hereditary hemmorhagic teleangiectasia., HHT, Octreotide, Sandostatin

Outcome measures

Primary outcome

'Successful response', defined as a decrease of >=50% in the amount of units

intravenous iron and/or blood transfusions given.

Secondary outcome

The percentual or mean/median difference between the half year prior to

inclusion and the treatment period of a half year between the treatment and

observational arm in:

- blood and intravenous iron requirements

- PROM*s: quality of life (SF-36, EQ-5D), level of fatigue (MFI-20), epistaxis

severity (ESS tool), and patient satisfaction

- hemoglobin and ferritin levels

- number of endoscopic treatments

- cost-effectiveness

- Safety

Study description

Background summary

Rendu-Osler-Weber (ROW or HHT) is an autosomal dominant hereditary disease

which affects 1 / 5-8000 individuals. It is characterized by ateriovenous malformations (AVMs) and telangiectasias in multiple organs, including the gastrointestinal tract. Patients can be transfusion dependent due to severe gastrointestinal bleeding from those telangiectasias. Endoscopy is not as effective due to the recurrent character of the telangiectasias. Based on literature in patients with non-ROW AVMs and telangiectasias, Octreotide might be beneficial for these patients to decrease their transfusion needs.

Study objective

To determine if SSA are effective in decreasing transfusion requirements and improving quality of life while being cost-effective.

Study design

International, multicenter, open label RCT with a parallell observational control group. (Phase III)

Intervention

Self-administerend subcutaneous injection with 0.1 mg short-acting Octreotide twice daily for 26 weeks.

Study burden and risks

Self-administered subcutaneous Octreotide-injection. The risks are the known side effects of subcutaneous Octreotide treatment.
Furthermore, the participants has to visit the hospital 4 times for check-ups with bloodsamples.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

- Patients older than 18 years with written informed consent.
- Diagnosis of HHT: either confirmed by genetic testing or the Curacao criteria (definitediagnosis).
- Presence of IDA in combination with the presence endoscopic proven GI AVM manifestations / telangiectasias confirmed within the last 12 months (upper and/or lower endoscopy and/or capsule endoscopy).
- Endoscopic refractory: at least 1 endoscopic APC / laser /other endoscopic treatment modality performed in the past 5 years.
- Substantial transfusion dependency: at least 4 blood units and / or intravenous iron in the 6 months prior to study inclusion with a:
o At least one serum ferritin below < 30 ug/l within the last 6 months requiring iron infusion above or equal to 1 g and/or
o Hemoglobin below 5.6 mmol/l (9.0 g/dl) or are in need of transfusions
due to anemia related symptoms within the last 6 months requiring blood transfusion above.

Exclusion criteria

- liver cirrhosis child-pugh C.
- symptomatic cholecystolithiasis (possible side-effect octreotide).
- pregnancy or nursing women or women having a pregnancy wish during the study period.

Study design

Design

Study phase:	3
Study type:	Interventional
Intervention model:	Parallel
Allocation:	Randomized controlled trial
Masking:	Open (masking not used)

Primary purpose: Prevention

Recruitment

NL

Recruitment status:	Completed
Start date (anticipated):	20-12-2019
Enrollment:	18
Type:	Actual

Medical products/devices used

Product type:	Medicine
Brand name:	Sandostatin
Generic name:	Octreotide
Registration:	Yes - NL outside intended use

Ethics review

Approved WMO Date:	01-05-2019
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	03-06-2019
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	24-10-2019
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
EudraCT	EUCTR2018-004179-11-NL
CCMO	NL68092.091.18
Other	NTR nummer volgt