

Orally inhaled heparin in patients with cystic fibrosis (CF)

Submission date 25/07/2008	Recruitment status No longer recruiting	☒ Prospectively registered
		☐ Protocol
Registration date 02/09/2008	Overall study status Completed	☐ Statistical analysis plan
		☐ Results
Last Edited 08/08/2016	Condition category Nutritional, Metabolic, Endocrine	☐ Individual participant data
		☐ Record updated in last year

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

Contact name
Dr Mark J Main

Contact details
Vectura Limited (UK)
1 Prospect West
Chippenham
United Kingdom
SN14 6FH
+44 (0)1249 667 700
mark.main@vectura.com

Additional identifiers

Protocol serial number
VR496/005

Study information

Scientific Title
A phase I/II randomised, placebo-controlled, double blind trial to assess the safety, tolerability, pharmacodynamics and exploratory efficacy of heparin inhalation in patients with cystic fibrosis (CF)

Study objectives

Orally inhaled Heparin is expected to provide advantages over currently available treatments for cystic fibrosis (CF) in a patient convenient delivery system.

Ethics approval required

Old ethics approval format

Ethics approval(s)

1. Multi-Centre Research Ethics Committee for Wales (UK) gave approval on 18th March 2008
2. Irish Ethics Committee (St Vincent's Healthcare Group Ltd Ethics) approved the study on 11th November 2008

Added 20/04/2010

3. Polish Ethics Committee (Bioethics Committee of the Medical University, Lodz) final approval gained on the 14th July 2009 (previously stated as the 21st April 2009)
4. Italian Central Ethics Committee (Comitato Etico, Azienda Ospedaliera Universitaria Integrata, Verona, approved on the 13th April 2010

Added 07/06/2010

5. Bellberry Human Research Ethics Committee, Dulwich, South Australia approved on the 20th April 2010

Primary study design

Interventional

Study design

Randomised double blind placebo controlled trial

Study type(s)

Treatment

Health condition(s) or problem(s) studied

Cystic fibrosis

Interventions

Patients will be randomised to receive one of three daily dose levels of heparin treatment or matching placebo; to be self-administered by inhalation by the patient twice daily for four consecutive weeks. Nominal Daily Doses to be studied are: 11400 IU, 22800 IU and 45600 IU.

For each patient there will be a screening period of 4 weeks, a treatment period of 4 weeks with a follow-up period of 2 weeks.

Intervention Type

Drug

Phase

Phase I/II

Drug/device/biological/vaccine name(s)

Heparin inhalation powder

Primary outcome(s)

Safety and tolerability.

Timepoints:

Five visits to the trial centre are included: screening, baseline, week 2, week 4 and, for follow-up, week 6.

Key secondary outcome(s)

Assessment of:

1. Sputum properties (i.e., rheological viscoelasticity/physicochemical measurement parameters)
2. Sputum inflammatory markers
3. Exhaled breath condensate pH
4. Blood plasma inflammatory markers
5. Blood coagulation
6. Visual Analogue Scale (VAS) parameters
7. Sputum microbiology
8. Pulmonary function parameters including FEV1 and forced vital capacity (FVC)
9. Response to the Cystic Fibrosis Questionnaire

Timepoints:

Five visits to the trial centre are included: screening, baseline, week 2, week 4 and, for follow-up, week 6.

Completion date

30/11/2010

Eligibility

Key inclusion criteria

Amendment as of 20/04/2010:

Point one below has been amended as follows:

1. Male or female, aged 16 years or older

Current information as of 21/09/2009:

1. Male or female, aged 18 years or older
2. Non-smoker
3. Written informed consent obtained prior to any trial specific procedures
4. Confirmed diagnosis of CF lung disease (i.e., respiratory clinical symptoms and positive sweat test or disease inducing mutations) by CF expert/investigator
5. Forced expiratory volume in one second (FEV1) at 40 - 90% of predicted value for age, sex and height at screening and baseline
6. FEV1 value at Baseline is within +/-15% of value at screening
7. Regular mucus production due to CF
8. Ease of sputum expectoration as defined by VAS score of ≤ 80 mm
9. Inflammatory markers above upper limit of normal range.
10. Adequate contraceptive measures.
11. Able to comply with all protocol requirements
12. Able to use inhalation device.

Amended as of 17/04/2009:

Please note that point 5 of the below criteria has been amended to read:

5. Forced expiratory volume in one second (FEV1) at 40 - 90% of predicted value for age, sex and height at screening and baseline

Initial information at time of registration:

1. Male or female, aged 18 years or older
2. Non-smoker
3. Written informed consent obtained prior to any trial specific procedures
4. Confirmed diagnosis of CF lung disease (i.e., respiratory clinical symptoms and positive sweat test or disease inducing mutations) by CF expert/investigator
5. Forced expiratory volume in one second (FEV1) at 40 - 80% of predicted value for age, sex and height during six months prior to screening
6. FEV1 within 10% of best value during six months prior to screening
7. Regular mucus production due to CF

Participant type(s)

Patient

Healthy volunteers allowed

No

Age group

Adult

Lower age limit

18 Years

Sex

All

Key exclusion criteria

Initial information at time of registration:

To be eligible for inclusion into this trial, each patient must not violate any one of the following exclusion criteria at the time of screening, at the time of assessment or as specifically described below:

1. Any contraindication to Monoparin® considered clinically relevant
2. Increased bleeding risk
3. History of heparin-induced thrombocytopenia
4. Patients with bleeding diathesis
5. Evidence of portal hypertension (e.g., hypersplenism or known grade III/IV oesophageal varices)
6. Clinically significant liver disease
7. Pregnancy at screening, or lactation
8. Previous thoracic or scheduled major surgery during trial
9. Any regular anticoagulant therapy (e.g., warfarin, aspirin) in the two weeks prior to screening
10. Modification of medication to treat respiratory disease between screening and baseline (Day 1)

Added 17/04/2009:

11. Diagnosis or history of aspergilloma

Added 21/09/2009:

12. Clinically significant serious disease or organ system disease not currently controlled / stable on present therapy

13. Planned hospitalisations which could interfere with trial compliance

14. Unable for any other reason to satisfactorily comply with the protocol (e.g., attendance for trial visits, treatment or assessments)

Date of first enrolment

01/11/2008

Date of final enrolment

30/11/2010

Locations

Countries of recruitment

United Kingdom

England

Ireland

Italy

Poland

Study participating centre

Vectura Limited (UK)

Chippenham

United Kingdom

SN14 6FH

Sponsor information

Organisation

Vectura Limited (UK)

ROR

<https://ror.org/000ydq217>

Funder(s)

Funder type

Industry

Funder Name

Vectura Limited (UK)

Results and Publications

Individual participant data (IPD) sharing plan

IPD sharing plan summary

Not provided at time of registration