

Metformin in Tuberous Sclerosis Complex

Submission date 09/02/2011	Recruitment status No longer recruiting	☒ Prospectively registered ☐ Protocol
Registration date 11/05/2011	Overall study status Completed	☐ Statistical analysis plan ☒ Results
Last Edited 09/03/2021	Condition category Genetic Diseases	☐ Individual participant data

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

Contact name
Dr Finbar O'Callaghan

Contact details
Department of Paediatric Neurology
Level 6 UHB Education Centre
Upper Maudlin Street
Bristol
United Kingdom
BS2 8AE
+44 11 7342 0202
finbar.ocallaghan@bristol.ac.uk

Additional identifiers

Clinical Trials Information System (CTIS)
2011-001319-30

Protocol serial number
Version 1

Study information

Scientific Title

A prospective, double-blind, randomised, parallel group, placebo-controlled trial of metformin treatment for renal angiomyolipomas in tuberous sclerosis complex

Acronym

MiTS

Study objectives

Metformin reduces the size of renal angiomyolipomas in people with tuberous sclerosis complex (TSC).

Ethics approval required

Old ethics approval format

Ethics approval(s)

Not provided at time of registration

Primary study design

Interventional

Study design

Prospective double-blind randomised parallel group placebo-controlled trial

Study type(s)

Treatment

Health condition(s) or problem(s) studied

Tuberous sclerosis complex, renal angiomyolipoma

Interventions

Participants will be randomised to two groups: Placebo or Metformin

Metformin 500mg twice daily for 6 months. If this dose is tolerated then after 6 months the dose will be increased to metformin 500mg three times per day for a further 6 months. Renal angiomyolipoma volume will be measured by magnetic resonance imaging at day 0 (baseline) and 12 months.

Intervention Type

Drug

Phase

Not Applicable

Drug/device/biological/vaccine name(s)

Metformin

Primary outcome(s)

Mean renal angiomyolipoma volume 12 months after initiation of intervention

Key secondary outcome(s)

1. Incidence of side effects
2. Mean subependymal giant cell astrocytoma volume 12 months after initiation of intervention
3. Health related quality of life 12 months after initiation of intervention
4. Cognitive ability, development and adaptive behaviour 12 months after initiation of intervention
5. Epilepsy outcome
6. Facial angiofibroma appearance 12 months after initiation of intervention

Completion date

31/08/2014

Eligibility

Key inclusion criteria

1. Clinically definite diagnosis of TSC (modified Gomez criteria)
2. Age between 10 to 65 years
3. One or more renal angiomyolipoma of at least one centimetre in largest diameter
4. Signed informed consent

Participant type(s)

Patient

Healthy volunteers allowed

No

Age group

Adult

Sex

All

Total final enrolment

55

Key exclusion criteria

1. Serious intercurrent illness or uncontrolled disease which could compromise participation in the study
2. Impairment of renal function
3. Use of x-ray contrast medium containing iodine within the last 30 days
4. Multiple renal angiomyolipomas where individual lesions cannot be distinguished (and as such cannot be accurately measured)
5. Renal haemorrhage within the preceding year
6. Known conservatively managed renal aneurysm(s) greater than 10mm
7. Liver insufficiency
8. Acute or chronic disease which may cause tissue hypoxia e.g. cardiac/respiratory failure, recent myocardial infarction, shock
9. Diabetes
10. Treatment with any injected or oral hypoglycaemic drug

- 11. Use of an investigational drug within the last 30 days
- 12. Pregnant or intending to become pregnant during the study period
- 13. Breastfeeding

Date of first enrolment

01/09/2011

Date of final enrolment

31/08/2014

Locations

Countries of recruitment

United Kingdom

England

Study participating centre

Department of Paediatric Neurology

Bristol

United Kingdom

BS2 8AE

Sponsor information

Organisation

University Hospitals Bristol NHS Foundation Trust (UK)

ROR

<https://ror.org/04nm1cv11>

Funder(s)

Funder type

Government

Funder Name

National Institute for Health Research (NIHR) (UK) - Research for Patient Benefit (RfPB) programme (ref: PB-PG-0909-20131)

Results and Publications

Individual participant data (IPD) sharing plan

IPD sharing plan summary

Not provided at time of registration

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article	results	14/01/2021	09/03/2021	Yes	No