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Metformin in Tuberous Sclerosis Complex

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

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

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Additional identifiers

EudraCT/CTIS number
2011-001319-30

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers
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

Study design
Prospective double-blind randomised parallel group placebo-controlled trial

Primary study design
Interventional

Secondary study design
Randomised controlled trial

Study setting(s)
Hospital

Study type(s)
Treatment

Participant information sheet
Not available in web format, please use the contact details below to request a patient information sheet

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 measure
Mean renal angiomyolipoma volume 12 months after initiation of intervention

Secondary outcome measures
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

Overall study start date
01/09/2011

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

Age group
Adult

Sex
Both

Target number of participants
100

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
England

United Kingdom

Study participating centre
Department of Paediatric Neurology
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Sponsor information

Organisation
University Hospitals Bristol NHS Foundation Trust (UK)

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Sponsor type
Hospital/treatment centre

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

Publication and dissemination plan
Not provided at time of registration

Intention to publish date

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