ISRCTN13744381 https://doi.org/10.1186/ISRCTN13744381

Heart muscle changes in facioscapulohumeral muscular dystrophy type 1 applying cardiac magnetic resonance

Submission date
13/06/2017

Recruitment status
No longer recruiting

Registration date
25/07/2017

Overall study status
Completed

Last Edited
08/04/2020

Condition category
Nervous System Diseases

[ ] Prospectively registered
[ ] Protocol
[ ] Statistical analysis plan
[X] Results
[ ] Individual participant data

Plain English summary of protocol

Background and study aims
Facioscapulohumeral muscular dystrophy (FSHD) is a genetic disease that causes weakness in the muscles of the limbs, shoulders and face. Patients may suffer from shortness of breath, dizziness and loss of consciousness. In the researchers' patient group heart problems seem to be less common, but problems including sudden death have been reported. It may be that an unknown injury of the heart leads to death or dangerous heart rhythm disturbances. Over the last couple of years cardiac (heart) MRI has become the gold standard method for looking at scars and other changes in the heart muscle (fat, inflammation). The aim of this study is to identify early heart muscle changes in patients with FSHD and their relationship with heart rhythm disturbances.

Who can participate?
Patients age 18 or over with FSHD, and healthy volunteers

What does the study involve?
All participants undergo a full examination by cardiologists including a cardiac MRI scan with both standard and new techniques to look for heart tissue injuries. Heart rhythm disturbances are assessed using an electrocardiogram (ECG).

What are possible benefits or risks of participating?
Participants receive a written report for their records including the basic assessment. The detection of early heart changes with MRI may help to improve treatment in the future. The MRI scan is prolonged by only about ten minutes. From a participant's perspective no other burden is created by this study.

Where is the study run from?
Charité University Medicine Berlin (Germany)

When is the study starting and how long is it expected to run for?
September 2015 to May 2017

Who is funding the study?
Bayer Health Medical Care

Who is the main contact?
Prof. Jeanette Schulz-Menger

Contact information

Type(s)
Scientific

Contact name
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13125

Additional identifiers

EudraCT/CTIS number

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers
FSHD-CMR (internal study code)

Study information

Scientific Title
Cardiac involvement in facioscapulohumeral muscular dystrophy type 1 patients with preserved ejection fraction – assessment by cardiovascular magnetic resonance

Study objectives
A prospective diagnostic trial to evaluate the efficacy of gadobutrol-enhanced cardiovascular magnetic resonance (CMR) at identifying myocardial tissue injury in facioscapulohumeral muscular dystrophy type 1 patients with preserved left ventricular function. This proof of concept trial is intended to extend the indications for CMR.

Ethics approval required
Old ethics approval format

Ethics approval(s)
Ethics board of the Charité University Medicine Berlin Campus Mitte, 03/09/2015, ref: EA1/169/15

Study design
It is a prospective diagnostic trial to evaluate the efficacy of gadobutrol-enhanced CMR to identify myocardial tissue injury in Facioscapulohumeral Muscular Dystrophy Type 1. This proof of concept trial is intended to extend the indications for CMR.

Primary study design
Observational

Secondary study design
Cohort study

Study setting(s)
Hospital

Study type(s)
Diagnostic

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

Health condition(s) or problem(s) studied
Facioscapulohumeral muscular dystrophy type 1

Interventions
After inclusion, the study participants undergo a CMR. Furthermore, an age- and gender-matched healthy control group is identified. They receive the same CMR protocol.

CMR is applied at a 1.5 T Scanner (MAGNETOM AvantoFit®, Siemens Healthcare, Erlangen, Germany) using a 32 channel surface coil. Cine imaging is performed applying state of the art steady state precession sequences to determine the global cardiac performance. For myocardial tissue differentiation, parametric T1 and T2 mapping, fat/water separated imaging and focal fibrosis imaging (Late Gadolinium Enhancement, LGE) are performed.

Intervention Type
Other

Primary outcome measure
Myocardial tissue injuries, detected using magnetic resonance imaging visually (qualitative) and quantitative including the presence and extent of lesions like fat and scar, measured at a single timepoint

Secondary outcome measures
Heart rhythm disturbances, assessed using ECG and ECG monitoring at a single timepoint

Overall study start date

03/09/2015

Completion date
15/12/2018

Eligibility

Key inclusion criteria
1. Genetically confirmed diagnosis of FSHD1
2. Age ≥18 years (no upper limit)
3. Age- and gender-matched healthy control group

Participant type(s)
Mixed

Age group
Adult

Lower age limit
18 Years

Sex
Both

Target number of participants
52

Total final enrolment
52

Key exclusion criteria
Known vascular, cardiac diseases (e.g. coronary artery disease, significant valvular disease, myocarditis), malign diseases or known contraindications for CMR or Gadolinium- based contrast-media

Date of first enrolment
03/09/2015

Date of final enrolment
31/12/2016

Locations

Countries of recruitment
Germany

Study participating centre

Working Group on Cardiovascular Magnetic Resonance
Experimental and Clinical Research Center, a joint cooperation between the Charité University Medicine Berlin and the Max-Delbrueck Center for Molecular Medicine, and HELIOS Klinikum Berlin Buch, Department of Cardiology and Nephrology
Lindenberger Weg 80
Berlin
Germany
13125

Sponsor information

Organisation
Charité University Medicine Berlin

Sponsor details
Working Group Kardiale MRT Lindenberger Weg 80
Berlin
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13125

Sponsor type
University/education

Website
http://www.cmr-berlin.org

ROR
https://ror.org/001w7jn25

Funder(s)

Funder type
Industry

Funder Name
Bayer Health Medical Care

Results and Publications

Publication and dissemination plan
Results of this study shall be published in a high-ranking peer-reviewed journal.

Intention to publish date
31/08/2017

Individual participant data (IPD) sharing plan
The datasets generated during and/or analysed during the current study are not expected to be made available due to data protection laws in Germany. However, upon request methodology and dataset structure can be shared.

IPD sharing plan summary
Not expected to be made available

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article	results	29/04/2019		Yes	No