ISRCTN15591075 https://doi.org/10.1186/ISRCTN15591075

A preliminary safety and efficacy evaluation of direct delivery of autologous bone marrow-derived cells in Egyptian patients with type 1 diabetes mellitus

Submission date
30/03/2017

Recruitment status
No longer recruiting

Registration date
02/05/2017

Overall study status
Completed

Last Edited
02/05/2017

Condition category
Digestive System

[ ] Prospectively registered
[ ] Protocol
[ ] Statistical analysis plan
[ ] Results
[ ] Individual participant data
[ ] Record updated in last year

Plain English summary of protocol

Background and study aims
Type 1 diabetes is a lifelong condition where the pancreas is unable to produce insulin, causing a person to have high or uncontrolled levels of blood sugar in their blood. It is a complex disease resulting from the autoimmune destruction of the majority of islet β-cells and requires patients to inject themselves with insulin to control their blood sugar. It is a major cause of morbidity (diseases) and mortality (death) worldwide. There is an option for stem cells to treat patients who are insulin dependent due to diabetes. Bone marrow is a diverse source of stem cells and transplantation could help repair and regenerate tissue that has been damaged. The normal way to deliver the stem cells (through injections into the arteries) has many problems. There is a possibility that taking stem cells from bone marrow and injecting it back into the person's pancreas could be feasible. The aim of this study is to evaluate the safety of a new strategy (designed to enhance the localization of where cells are injected to the diseased pancreas) by a single administration of autologus bone marrow derived mononuclear cells (A-BMMNCs) (that have certain types of stem cells) using a computed tomography (CT) scan-guided direct delivery route and to explore whether this treatment leads to improved β-cell function in diabetes patients.

Who can participate?
Males and females aged two to 30 years old with confirmed Type 1 diabetes.

What does the study involve?
Participants undergo a clinical examination and provide blood samples. They then undergo a bone marrow transplant done using a new strategy that uses a single administration of stem cells from bone marrow to a certain part of the pancreas (directly to the tail, not to the blood vessels that supply the pancreas). This is guided by a CT scanning machine. Participants are followed up for one year to see if there was any improvement or decrease in their β-cell function and diabetes symptoms.

What are the possible benefits and risks of participating?
Participants may benefit from being able to decrease or stop injecting insulin and having less diabetes complications. There is a risk of acute pancreatitis.

Where is the study run from?
Wadi EL-Neel Hospital (JCI) (Egypt)

When is the study starting and how long is it expected to run for?
January 2014 to June 2015

Who is funding the study?
Wadi EL-Neel Hospital (JCI) (Egypt)

Who is the main contact?
Professor Hazem Khamis

Contact information

Type(s)
Scientific

Contact name
Prof Hazem Khamis

Contact details
Wadi El-neel Hospital (JCI Accredited hospital)
Kobri Elkobba
Cairo
Egypt
19303

Additional identifiers

Protocol serial number
0000006555

Study information

Scientific Title
A preliminary safety and efficacy evaluation of direct intrapancreatic tail delivery of autologous bone marrow-derived cells in Egyptian patients with type 1 diabetes mellitus

Study objectives
The aim of this study is to evaluate the safety of a new strategy (designed to enhance localization of the injected cells to the diseased pancreas along with avoidance of nonspecific organ entrapment) by a single administration of autologus bone marrow derived mononuclear cells (A-BMMNCs) via a computed tomography (CT) scan-guided direct transgastricintrapancreatic (DTI) delivery route and to explore whether this treatment leads to improved β-cell function in insulin dependent diabetes mellitus (IDDM) patients.

Ethics approval required
Old ethics approval format

Ethics approval(s)
Wadi El-neel Ethical Committee, 13/01/2014, ref: 0000006555

Study design
Prospective single centre interventional non randomised study

Primary study design
Interventional

Study type(s)
Treatment

Health condition(s) or problem(s) studied
Type 1 diabetes mellitus

Interventions
At baseline, participants undergo a clinical examinations and provide blood samples to assess for HbA1c, fasting C-peptide (FCP) and 2hour-C-peptide (2h-CP) levels (as indirect measures of endogenous insulin secretion), levels of islet cells and insulin antibodies. Insulin doses for each participant are determined.

Participants then undergo an autologus bone marrow transplant derived mononuclear cells. This is done using a new strategy (designed to enhance localisation of the injected cells to the diseased pancreas along with the avoidance of nonspecific organ entrapment) by a single administration of bone marrow-derived mononuclear cells (BM-MNCs) via a computed tomography (CT) scan-guided direct transgastricintrapancreatic (DTI) delivery route.

During the procedure, participants are assessed for bleeding from the bone marrow aspiration and transgastric puncture sites, abdominal pain and other clinical complaints (i.e. nausea, vomiting, pain and fever). Blood amylase levels are measured to exclude acute pancreatitis complications. Haematocrit values are obtained only in suspected bleed cases.

Participants are followed up between month one-two, three-five, six-eight, and after eight months (around 12 months) for any clinical complaints. After six months, if islet cells and insulin antibodies are abnormal and serologically positive before the transplantation, there are re-measured to record any improvement or decline in these antibodies. If the patients are negative for islet cell or insulin antibodies before the transplantation, the test is not repeated after six months (as it is not recommended as routine clinical practice). Participants are also followed up to measure their HbA1c, FCP, 2h-CP, insulin doses levels as well as their frequency of hospitalisation for hypo- and hyper-glycemic attacks (common acute short-term complications of uncontrolled diabetes that can lead to hospitalization and account for the majority of costs associated with diabetes care). Participants are encouraged to self-monitor blood glucose at least twice a day.

Intervention Type
Drug

Phase

Not Applicable

Drug/device/biological/vaccine name(s)
Insulin, autologus bone marrow derived mononuclear cells

Primary outcome(s)
Safety is measured using the morbidity, mortality, and unwanted side effects (through patient records) during the procedure and at 12 month follow up.

Key secondary outcome(s))
1. Serological changes in positive islet cell and insulin antibody levels are measured using blood samples at baseline and six months
2. β-cell function is measured using blood samples(HbA1c and in FCP and 2h-CP levels) at baseline, month one-two, month three-five, month six-eight and months eight to 12
3. Abdominal pain and other clinical parameters are measured using clinical examination during the procedure and at month one-two, month three-five, month six-eight and month 12

Completion date
13/06/2015

Eligibility

Key inclusion criteria
1. Males and females aged between 2 to 30 years of age
2. Confirmed T1D based on the American Diabetes association (ADA) 2015 criteria for diagnosis
3. Body mass index (BMI) of ≤ 29.9

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Mixed

Sex
All

Key exclusion criteria
1. Active infections
2. Any chronic or acute illness
3. Antibodies to hepatitis B surface antigen
4. Hepatitis C
5. Human immunodeficiency virus or evidence of diabetic complications at baseline
6. Pregnant, are breast-feeding or intend to become pregnant during the study
7. Clinically relevant uncontrolled medical conditions not associated with diabetes (such as hematologic, renal, hepatic, neurologic, cardiac and respiratory conditions)
8. Evidence of active malignancy or a prior history of active malignancy

Date of first enrolment
13/02/2014

Date of final enrolment
13/04/2014

Locations

Countries of recruitment
Egypt

Study participating centre
Wadi El-neel (JCI Accredited Hospital)
Kobri Elkobba
Cairo
Egypt
19303

Sponsor information

Organisation
Wadi EL-Neel hospital (JCI)

Funder(s)

Funder type
Hospital/treatment centre

Funder Name
Wadi EL-Neel Hospital (JCI)

Results and Publications

Individual participant data (IPD) sharing plan
The datasets generated during and/or analysed during the current study are/will be available upon request from Raghda Moustafa Shahin at Raghda.shahin@fop.usc.edu.eg

IPD sharing plan summary
Available on request