Mesenchymal stromal cells for treatment of drug resistant pediatric juvenile idiopathic arthritis

No registrations found.

Ethical review	Positive opinion
Status	Recruiting
Health condition type	-
Study type	Interventional

Summary

ID

NL-OMON21350

Source
NTR

Brief title
MSC-JIA

Health condition

juvenile idiopathic arthritis

Sponsors and support

Primary sponsor: University Medical Center Utrecht
Source(s) of monetary or material Support: ZonMw (The Netherlands Organisation for Health Research and Development)

Intervention

Outcome measures

Primary outcome

Total number of adverse events in the 3 months prior to MSC infusion and the number of adverse events 3 months after MSC infusion.

To offer an effective alternative for that category of JIA patients that is therapy-resistant.

For this effectiveness the ACR Pediatric 70 criteria should be achieved.

The ACR Pedi 70 criteria are defined as improvement of >= 70% in at least 3 of 6 core response variables used to assess disease activity with no more than 1 variable worsening by >= 30%.

Secondary outcome

- The ACR Pediatric 30 criteria should be met.

The ACR Pedi 30 criteria are defined as improvement of ≥ 30% in at least 3 of 6 core response variables used to assess disease activity with no more than 1 variable worsening by ≥ 30%.
Core response variables are:

1. Physician global assessment of overall disease activity

2. Parent or patient global assessment of overall well-being

3. Functional ability (CHAQ)

4. Number of joints with active arthritis

5. Number of joints with limited range of motion

6. Index of inflammation: ESR or CRP

- Radiological (MRI) improvement of most active large joint.

- Improvement in laboratory parameters or biomarkers.

Study description

Background summary

The main objective is to offer a safe alternative for that category of JIA patients that is therapy-resistant. We hypothesize that intravenous administration of MSC in therapy refractory JIA patients is safe and has the potential to have clinical relevant effect as measured by the ACR Pedi 30.

Aims:

1. Total number of adverse events in the 3 months prior to MSC infusion and the number of adverse events 3 months after MSC infusion.

2. The ACR Pediatric 30 criteria should be met

3. Radiological (MRI) improvement of most active large joint.

4. Improvement in laboratory parameters or biomarkers.

Study objective

To find out if intravenous MSC is a safe treatment for children with therapy-resistant JIA

Study design

52 weeks after the (first) MSC injection the primary outcome will be measured and the third MRI will be made.

Also all data and materials for the secondary outcomes are collected within this time frame. This will also be the end of the study for the individual.

Intervention

1 to maximal 3 iv MSC infusions.3 MRI scans, 4 extra visits to the hospital and 3 extra venapunctures when compared to standard treatment in the typical JIA patient

Contacts

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Eligibility criteria

Inclusion criteria

Inclusion criteria: In order to be eligible to participate in this study, a subject must meet all of the following criteria:

Patients (4-18 years of age) diagnosed with juvenile idiopathic arthritis according to the ILAR-criteria with active arthritis resistant to intra-articular steroids and systemic use of methotrexate and for whom no on-label indication exists for (not yet used) biologicals.

The patient is followed for adverse events via the
Pharmachild database.

Informed consent signed.

Exclusion criteria

Concurrent use of biological response modifiers.

Concurrent infection, febrile illness or malignancy.

Pregnancy.

No signed informed consent.

Study design

Design

Study type:	Interventional
Intervention model:	Other
Allocation:	Non controlled trial

Masking:	Open (masking not used)
Control:	N/A , unknown

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	01-08-2014
Enrollment:	6
Type:	Anticipated

Ethics review

Positive opinion

Date:	04-09-2013
Application type:	First submission

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
NTR-new	NL3923
NTR-old	NTR4146
Other	2012-002067-10 : EUDRACT
ISRCTN	ISRCTN wordt niet meer aangevraagd.

Study results