Prevention of renal and bladder damage in children with spina bifida by means of early injections with Botulinum-Toxin-A (Botox): a pilot study.

No registrations found.

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

Summary

ID

NL-OMON27522

Source
NTR

Brief title
Early Botox in SB patients.

Health condition

Spina Bifida

Sponsors and support

Primary sponsor: Erasmus MC, Dept. Urology
Source(s) of monetary or material Support: Jaap Schouten Foundation

Intervention

Outcome measures

Primary outcome

End filling pressure of the bladder(determined by means of urodynamic testing) after 5 years

of follow-up.

Secondary outcome

- Clinical parameters:

o Number of urinary tract infections

o Frequency of intermittent catheterisation

o Bladder volumes (with intermittent catheterisation)

- Urodynamic parameters:

o Bladder capacity

o Bladder overactivities

o Compliance of the bladder

- Number and nature of operative interventions to the urinary tract.

Study description

Background summary

Background of the study:

Children with severe spina bifida almost always have serious bladder function problems, possibly resulting in renal damage. Taking into account (recent) discovered properties of botulinum toxin A it can be assumed that early treatment
with Botox in the bladder of children with spina bifida may have a protective effect on bladder function. This can result in
less operations and less incontinence later on in life, which undoubtedly will increase the quality of life of these children.

Also (further) deterioration of the renal function can be delayed.

Objective of the study:

- Can early Botox-injections in the bladder of patients with meningomyelocèle (MMC) prevent deterioration of the bladder
and renal function?

- Can early Botox-injections in the bladder of patients with meningomyelocèle (MMC) decrease the need for an operation
(ileocystoplastic with appendicovesicostoma)?

Study design:

Open, controlled, prospective pilot study.

Study population:

All newborns with MMC can participate in the study. There will be made no distinction in gender and/or ethnical background.

Nature and extent of the burden and risks associated with participation, benefit and group relatedness:

With dosages of <10 U/kg hardly any side effects are described in literature. A relative frequent side effect in “non neurogenic” adults is urinary retention. In case of urinary retention the patient should be catheterized temporarily (approximately 4 weeks) to fully empty the bladder. Patients in this study however are already catheterized as of birth, so a temporarily retention is not relevant. Another known side effect is a urinary tract infection shortly after the injection. This side effect however is rather a result of the necessary cystoscopy than of the Botox itself. Besides this, the risk of a urinary tract infection can be strongly reduced by applying antibiotics peri-operative. In current literature no prove can be found that repetitive narocsis has a negative influence on the development of children. Based on the hypothesis serious functional restrictions can be prevented. This probably weigh up to the currently unknown effects of anesthetics in children.

Study objective

Vroegtijdige Botox-injecties in de blaas bij MMC patiënten kunnen verdere achteruitgang van de blaas- en nierfunctie voorkomen.

Study design

Primary outcome: after 5 years of follow-up

- Clinical parameters: continuous

- Urodynamic parameters: at visits with urodynamic testing (age 2, 8, 17, 26, 35, 44, 53, and 62 months)

- Number and nature of operative interventions to the urinary tract: continuous.

Intervention

Botulinum toxin A will be injected in the bladder at the age of approximately 5 months during a short-term narcosis (10 minutes). These injections will be repeated every 9 months during the study period.

Contacts

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Eligibility criteria

Inclusion criteria

The study group exists of newborns with MMC who are treated with the standard treatment

(CIC and anticholinergics). The minimal age at inclusion is 3 months. This is because of the minimal diameter of the urethra which is necessary for cystoscopy.

The historical control group exists of MMC patients who have not been treated with Botox and who were born between 1998 and 2008.

Exclusion criteria

- Patients with swallowing or breathing problems for which medical treatment is or was necessary.

- Muscle diseases such as myasthenia gravis, amyotrophic lateral sclerosis or the Syndrome of Eaton-Lambert.

For the historical group the database of (parents of) patients who object against the use of their medical records for research purposes.

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):	01-06-2014
Enrollment:	10
Type:	Anticipated

Ethics review

Positive opinion
Date: 13-05-2014
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	NL4397
NTR-old	NTR4594
Other	NL46292.078.13 : A301223

Study results

Summary results
Not applicable.