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

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- 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...

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Neurological disorders congenital
Study type	Interventional

Summary

ID

NL-OMON40616

Source
ToetsingOnline

Brief title
Early Botox in SB patients.

Condition

- Neurological disorders congenital
- Neurological disorders NEC
- Bladder and bladder neck disorders (excl calculi)

Synonym
Neurogenic bladder function disease in spina bifida patients, spastic bladder in spina bifida patients.

Research involving
Human

Sponsors and support

Primary sponsor: Erasmus MC, Universitair Medisch Centrum Rotterdam
Source(s) of monetary or material Support: Jaap Schouten Foundation

Intervention

Keyword: early botox, prevention, renal and bladder damage, spina bifida

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

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.

Study objective

- 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.

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.

Study burden and risks

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.

Contacts

Public
Erasmus MC, Universitair Medisch Centrum Rotterdam

Wytemaweg 80
Rotterdam 3015 CN
NL
Scientific
Erasmus MC, Universitair Medisch Centrum Rotterdam

Wytemaweg 80
Rotterdam 3015 CN
NL

Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Children (2-11 years)

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

The exclusion criteria for the study group are:
- 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 phase:	4
Study type:	Interventional
Intervention model:	Other
Allocation:	Non-randomized controlled trial
Masking:	Open (masking not used)
Control:	Active
Primary purpose:	Prevention

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	15-10-2014
Enrollment:	10
Type:	Actual

Medical products/devices used

Product type:	Medicine
Brand name:	BOTOX
Generic name:	Botulinum toxin type A
Registration:	Yes - NL outside intended use

Ethics review

Approved WMO Date:	06-01-2014
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	24-04-2014
Application type:	First submission

Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)

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
EudraCT	EUCTR2013-003842-18-NL
CCMO	NL46292.078.13