N- acetylcysteine treatment for skin picking in children and young adults with PWS: A randomized, doubleblind, placebo-controlled, cross-over trial.

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To evaluate the effects of N-acetylcysteine (NAC; Fluimucil® in Dutch) on skin picking behaviour in children and young adults with PWS.

Ethical review	Approved WMO
Status	Pending
Health condition type	Chromosomal abnormalities, gene alterations and gene variants
Study type	Interventional

Summary

ID

NL-OMON54933

Source
ToetsingOnline

Brief title
NAC-treatment for skin picking in PWS

Condition

- Chromosomal abnormalities, gene alterations and gene variants
- Hypothalamus and pituitary gland disorders

Synonym
Prader-Willi syndrom

Research involving
Human

Sponsors and support

Primary sponsor: Stichting Kind en Groei

Source(s) of monetary or material Support: Stichting Kind en Groei

Intervention

Keyword: N-acetylcysteine, Prader-willi syndrome, Skin picking

Outcome measures

Primary outcome

Change in number and size of the skin picking lesions.

Secondary outcome

Change in repetitive/compulsive behaviour, measured by:

• Repetitive Behaviour Scale (RBS)

• Skin Picking-Symptom Assessment Scale (SP-SAS)

• Clinical Global Impression Scale (CGI)

Change in quality of life, measured by:

• Pediatric Quality of Life Inventory (PedsQL)

Change in cortisol concentration in hair samples.

Change in safety parameters (laboratory parameters and medical assessments).

Study description

Background summary

Patients with PWS suffer from behavioural problems in addition to their physical abnormalities. The onset of these behavioural problems is during childhood. Children with PWS might present with significant maladaptive behavioural and emotional characteristics including temper tantrums,

inappropriate social behaviour and self-injurious behaviour. Skin picking is the most common form of self-injurious behaviour in patients with PWS and can lead to severe wounds and infections. Several studies demonstrated that N-acetylcysteine is a promising treatment for patients with problems in obsessive- compulsive spectrum. N-acetylcysteine restores the extracellular glutamate concentration in the nucleus accumbens and this mechanism might play a role in the prevention of compulsive behaviours. An open label pilot study showed that N-acetylcysteine treatment in patients with PWS showed a reduction in skin-picking behaviour. Therefore, the aim of this study is to investigate the effect of N-acetylcysteine versus placebo in children and young adults with PWS who are suffering from skin picking behaviour.

Study objective

To evaluate the effects of N-acetylcysteine (NAC; Fluimucil® in Dutch) on skin picking behaviour in children and young adults with PWS.

Study design

A randomized, doubleblind, placebo-controlled, cross-over trial: daily dose of n-acetylcysteine versus placebo for three months each. With a washout period of three months between cross-over.

Intervention

Daily oral administration of acetylcysteine or placebo:
<30kg:
4 weeks: 600mg
8 weeks: 1200mg

>30kg < 59.99 kg:
4 weeks 600mg
4 weeks 1200mg
4 weeks 1800mg

>60kg:
4 weeks 600mg
4 weeks 1200mg
4 weeks 2400mg

Study burden and risks

Burden: once a day oral intake of tablet(s) for two periods of 3 months. During this time there will be maximal 5 outpatient hospital visits for the measurements of the skin picking lesions and the body locations of the lesions. Four of the five hospital visits will include blood sample draw, all the visits

include blood pressure and heart rate measurement.

Risks: Based on literature data, there are no severe side effects or adverse events expected. The most common side effects of NAC, which appear in 0,01-1% of patients are hypersensitivity reactions to NAC, like itching, skin rash, headache and mild gastrointestinal complaints like nausea, cramps, diarrhoea, flatulence. The patients and their parents are expected to be very motivated to participate in this study, considering the negative impact of their skin-picking behaviour on the quality of life of the patients and family.

Contacts

Public
Stichting Kind en Groei

Westzeedijk 106
Rotterdam 3016AH
NL
Scientific
Stichting Kind en Groei

Westzeedijk 106
Rotterdam 3016AH
NL

Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Children (2-11 years)
Elderly (65 years and older)

Inclusion criteria

- Prader-Willi syndrome (genetically proven)
- Age between 6 - 25 years
- Skin picking behaviour for at least one year, according the following DSM-5 criteria:
1. Repeated picking of the skin and damaging it.
2. Multiple attempts to stop the behaviour.
3. Picking of the skin provokes significant stress or limitations in social, professional functioning, or limits other important areas of functioning.
4. Picking of the skin is not attributed to the direct effect of drugs or to any other medical condition (e.g. scabies).
5. Picking of the skin is not explained by another mental disorder (e.g. psychosis or body dysmorphic disorder).

Exclusion criteria

- Severe psychiatric problems, as active psychosis or behavioural problems that hinder study participation.
- Previous treatment with N-acetylcysteine in the last 3 months.
- Current or recent (past 3 months) DSM-5 substance abuse or dependence.
- Initiation of pharmacotherapy, psychotherapy, or behaviour therapy from a mental health perspective in the last 3 months.
- Treatment with investigational medication or depot neuroleptics within 3 months.
- Need for medication other than NAC with possible psychotropic effects or unfavourable interactions with NAC.
- Asthma (given the possible worsening).
- Active peptic ulcer considering the possible gastrointestinal- and vomiting side effects.

Study design

Design

Study phase:	2
Study type:	Interventional
Intervention model:	Crossover
Allocation:	Randomized controlled trial
Masking:	Double blinded (masking used)

Control:	Placebo
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Pending
Start date (anticipated):	01-03-2021
Enrollment:	35
Type:	Anticipated

Medical products/devices used

Product type:	Medicine
Brand name:	Fluimucil
Generic name:	N-acetylcysteine
Registration:	Yes - NL outside intended use

Ethics review

Approved WMO Date:	04-08-2020
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	06-04-2021
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	EUCTR2019-000735-61-NL
CCMO	NL74197.078.20