Transition of care in women with Turner syndrome: Oxandrolone follow-up study

Published: 12-05-2009
Last updated: 06-05-2024

The aim of the study is to assess the long-term effects (at adult age) of the TOS study medication (growth hormone and estrogen with or without Ox), with specific attention to adult height and body proportions, body composition, virilization,...

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Endocrine disorders congenital
Study type	Observational invasive

Summary

ID

NL-OMON33605

Source
ToetsingOnline

Brief title
Turner Oxandrolone follow-up study

Condition

- Endocrine disorders congenital

Synonym
Turner Syndrome

Research involving
Human

Sponsors and support

Primary sponsor: Universitair Medisch Centrum Sint Radboud
Source(s) of monetary or material Support: industrie: Pfizer,Pfizer

Intervention

Keyword: growth hormone, oxandrolone, Turner syndrome

Outcome measures

Primary outcome

The main study parameters are: height, body proportions, body composition, symptoms and signs of virilization, glucose tolerance, lipid profile, cardiac conduction abnormalities, liver and thyroid function, (neuro)psychological function and quality of life. The effects of Ox are analyzed to a background of genotypic variation of the sex chromosomes and the growth hormone receptor.

Secondary outcome

Not applicable

Study description

Background summary

Turner syndrome is the result of complete or partial absence of one X-chromosome. Besides short stature and gonadal dysgenesis, Turner syndrome is associated with a wide range of abnormalities affecting nearly every organ system. In 1991 the Dutch multicentre Turner Oxandrolone Study (TOS) started: a randomized double-blind placebo controlled study on the effect of the androgen oxandrolone (Ox) in combination with authentic biosynthetic human growth hormone and low-dose estrogens on growth and metabolic parameters in girls with Turner syndrome (CEOM-nr 9202-0029). In TOS, the girls were followed until a mean of 1.5 years after cessation of growth hormone and Ox/placebo. Preliminary analysis suggests that Ox enhances growth at cost of mild virilization and deceleration of breast development, especially in the high-dose group. The beneficial and possible adverse effects of Ox potentially carry on into adulthood, i.e. far beyond the scope of the original pediatric study. The present study represents a long term follow-up of TOS. The girls who participated in the original TOS have now reached an adult age.

Study objective

The aim of the study is to assess the long-term effects (at adult age) of the TOS study medication (growth hormone and estrogen with or without Ox), with specific attention to adult height and body proportions, body composition, virilization, metabolic cardiovascular risk profile, neuropsychological function and quality of life. Our hypothesis, is that permanent beneficial effects of auxiliary treatment with Ox outweigh short-lived adverse effects.

Study design

This is an observational study and represents the long-term follow-up of a multicentre double-blind placebo controlled trial.

Study burden and risks

Participating subjects are investigated during a single, whole-day visit to the RUNMC which comprises of standardized history taking and physical examination including standardized photographs, sampling of blood, urine, hair and buccal mucosa, (neuro)psychological investigations, voice frequency analysis and audiometry, bone mineral density and body composition measurement and electrocardiography. The risks associated with these investigations for the participants are minimal. If the investigations yield abnormalities relevant to the patient, the patient will be referred to their own physician for adequate treatment.

Contacts

Public
Universitair Medisch Centrum Sint Radboud

Postbus 9101
6500 HB Nijmegen
NL
Scientific
Universitair Medisch Centrum Sint Radboud

Postbus 9101
6500 HB Nijmegen
NL

Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

Inclusion criteria are completion of the original TOS protocol at least 6 months prior to the study visit and age >=18 years. Main inclusion criteria of the original protocol (TOS) were: a) the diagnosis of TS should be confirmed by lymphocyte chromosomal analysis. Any chromosomal pattern which is known to be associated with TS characteristics is acceptable, except for evidence of a Y-chromosome on blood analysis. b) Chronological age between 2 and 15.99 years. c) Well-documented growth rate during the previous year. d) Bone-age lower than 12.0 years.

Exclusion criteria

Exclusion criteria are: a) Patients who have participated in another experimental drug study within two months of entry into the present study. b) Malignant or severely disabling disease. c) Serious suspicion of psychiatric illnesses. d) Pregnancy or current fertility treatment. Main exclusion criteria of the original protocol: a) Any endocrine or metabolic disorder, with the exception of thyroidal illnesses adequately treated and/or substituted. b) Growth failure due to disorders of urinary, cardiopulmonary, gastro-intestinal and nervous systems; nutritional/vitamin deficiencies and chondrodysplasias. c) Patients with hydrocephalus. d) Patients who have participated in another experimental drug study within two months of entry. e) Patients receiving any kind of drug that may interfere with GH therapy. f) Previous GH, sex hormone, or anabolic steroid treatment. g) Presence of any persistent abnormality at general pediatric and biochemical screening (including scoliosis, liver function tests, kidney function tests, electrtolytes, blood count, urine glucose, protein and sediment). h) Serious suspicion of psychosocial dwarfism (emotional deprivation) or psychiatric illnesses.

Study design

Design

Study type:	Observational invasive
Intervention model:	Parallel
Allocation:	Randomized controlled trial
Masking:	Double blinded (masking used)
Control:	Placebo
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	28-07-2009
Enrollment:	133
Type:	Actual

Medical products/devices used

Registration:	No

Ethics review

Approved WMO

Date:	12-05-2009
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)

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
CCMO	NL26203.091.09