



Clinical Trials Unit Projects 2008

<http://www.bsped.org.uk/professional/projects/index.htm>

STUDIES RECRUITING NOW

AdDIT

(Adolescent Type 1 Diabetes Intervention Trial)

A randomised, double blind, placebo controlled trial of ACEI and statin therapy in adolescents with Type 1 Diabetes

- Principal Investigators (UK): David Dunger, Julie Edge, Julian Hamilton-Shield, Tim Cheetham, Tim Barrett, Rakesh Amin
- Project Co-ordinator (UK): Stella Silvester

Aim: To determine whether intervention with an ACE inhibitor, a statin or combination therapy will reduce albumin excretion and cardiovascular risk in high risk adolescents (age 11-16 yrs) with newly diagnosed T1D.

Recruitment start date: November 2008

Trial status: Screening to identify subjects with high rates of albumin excretion has been underway since June 2008 in 6 sites & multiple sub-sites in the UK, as well as a further 6 centres in Australia & Canada. 3000 children will be screened in order to recruit 500 for the intervention (250 in the UK). The distribution of drugs to pharmacies and the first participant randomisation is expected in November 2008.

NESGAS

(North European Small-for-Gestational Age Study)

A randomised, multi-centre, multi-national trial evaluating the safety and efficacy of GH treatment at varying doses in short children, born small-for-gestational-age (SGA)

- Principal Investigators (UK): Professor David Dunger, Dr Malcolm Donaldson, Dr Jeremy Kirk
- Project Manager (UK): Emma-Jane Gault
- Researchers (UK): Catherine Fullah (Cambridge); Ann McGovern (Manchester); Sheena McGowan (Glasgow); TBA (Birmingham)

Aim: To determine the safety & efficacy of 3 different GH treatment regimens in SGA children - high dose, low dose & dose titration according to IGF-I levels.

Start date: 30/09/2004

Trial status: 300 short children, born SGA, aged 4 - 8.99 years (9.99 years in boys) are required to be enrolled across 7 European countries, 100 in the UK. Currently, 100 patients have been enrolled across all European sites.

THYROTOXICOSIS STUDY

A randomised study of two anti-thyroid drug treatment regimens in young people with thyrotoxicosis

- Chief Investigator: Dr Tim Cheetham
- Project Manager: Diane Picton

Aim: To compare the advantages and disadvantages of the two main anti-thyroid drug treatment regimens in young people with hyperthyroidism by 1) determining the amount of time that patients have abnormal biochemical indices whilst on treatment and 2) determining remission rates 1 year post-treatment. **Start date:** 01/09/2004

Trial status: Recruitment began in January 2005 and will continue for 4 years with a view to recruiting 128 participants throughout the UK. Currently 31 patients have been recruited at 6 sites and a further 4 sites have signed up to the study. The trial is on-going and referrals are welcome. Please contact Adelyn Thomason for details.

MOCA

(Metformin in Obese Children & Adolescents)

A multi-centre randomised placebo controlled trial of metformin in obese children and adolescents aged 8-18 years with insulin resistance and/or impaired glucose tolerance.

- Chief Investigator: Dr Catherine Hall
- Project Manager: Dr Debbie Kendall

Aim: To study the effects of metformin treatment compared to placebo at a dose of 1.5g per day for 6 months. **Start date:** May 2005 **Trial status:** 140 children are required and currently 78 have been recruited. The trial is on-going and referrals are welcome. Please contact Adelyn Thomason for details.

NATIONAL TURNER REGISTER

Establishment of a national register of UK women with Turner syndrome (TS) aged ≥ 16 yrs

- Chief Investigator: Professor David Dunger
- Project Manager: Emma-Jane Gault

Aim: To monitor the provision of medical care for TS patients in adolescence & adulthood. **Start date:** 02/12/2004

Trial status: The project has MREC approval (MREC 03/6/75) and an amendment is underway to allow patients to refer themselves to the register, removing the need for local R&D approval (NB SSI form NOT required). The register is ongoing and currently, data have been collected on 33 women at 8 centres.

ONGOING STUDIES - RECRUITMENT CLOSED

GROWTH HORMONE REGISTER

Establishment of a national register of all children prescribed growth hormone (GH) therapy.

- Chief Investigator: Professor David Dunger
- Project Manager: Adelyn Thomason

Aim: To develop a register of all children prescribed GH therapy including age at start of therapy, sex, indication for therapy, diagnostic category, and region. The data will inform NICE deliberations on GH indications.

Start date: 01/08/2003 **Trial status:** Data collection has now ended. Over 30 consultants reported to the register quarterly for 5 years and data has been collected on approximately 2000 children. Analysis of the data is underway.

UK GRID STUDY

UK Genetic Resource Investigating Diabetes

- Chief Investigator: Professor David Dunger
- Project Manager: Barry Widmer

Aims: 1) To establish cell lines from 10,000 patients with type 1 diabetes diagnosed under 16 years and 10,000 ethnically matched controls to obtain a permanent source of DNA. 2) High throughput genotyping of the DNA samples to compare allele frequencies in cases with controls to map and identify genes for type 1 diabetes. 3) Biochemical and functional analyses of genes & their expression to differentiate between the functions and role of alleles predisposing to and protective of disease.

Start date: 01/01/2001 **Trial status:** Over 10,000 samples have been collected from over 100 UK centres. The DNA samples are available to the wider scientific community through the JDRF/Wellcome Trust Access Committee.

UK TURNER STUDY

A randomised, double-blind, placebo controlled study of growth promoting treatment in TS

- Chief Investigator: Dr Malcolm Donaldson
- Project Manager: Emma-Jane Gault

Aim: To determine the impact (if any) on final height of oxandrolone and the timing of pubertal induction (12 vs 14 yrs) in girls with TS receiving GH treatment

Start date: 24/11/1999

Trial status: 102 girls were enrolled at 40 UK hospitals. The study is nearing completion and results are expected in 2009. Currently, 42 girls have completed treatment and have achieved a mean FH of 152.0cm.

CTU INFORMATION

PROPOSED STUDIES

• Androgen replacement study

Chief Investigator: Professor Chris Kelnar

A randomised comparison of endogenous vs. exogenous testosterone in stimulating male pubertal development (induction & progression) in boys with hypogonadotropic hypogonadism

• Diabetic Ketoacidosis (DKA) study

Chief Investigators: Dr Julie Edge, Dr Jeremy Allgrove, Professor David Dunger

A study comparing two different insulin doses in DKA - 0.05 units/kg/hour vs 0.1 units/kg/hour

• Prader Willi study

Chief Investigator: Dr Malcolm Donaldson

A randomised trial of GH therapy in children with Prader Willi syndrome aged 1-3 years, examining its impact on body composition and motor development

• Thyroxine study

Chief Investigator: Dr Malcolm Donaldson

A randomised controlled trial of initial thyroxine treatment comparing liquid vs. tablet form for neonates with congenital hypothyroidism

• Transition study

Chief Investigator: Professor Chris Kelnar

A randomised controlled trial to determine the optimisation of GH during the transition phase from achievement of adult height to attainment of peak bone mass

• UK Turner Study II

Chief Investigator: Dr Malcolm Donaldson

A randomised controlled trial of oral (contraceptive pill and HRT) versus transdermal oestrogen replacement for adolescents and young women with Turner syndrome

• UK Turner Study III

Chief Investigator: Dr Malcolm Donaldson

A randomised controlled trial of oral versus transdermal oestrogen therapy for pubertal induction in girls with Turner syndrome

Hormone replacement for the induction, progression and completion of puberty in hypogonadal males and females

An over-arching study or collection of studies encompassing hormone replacement for pubertal development in hypogonadal boys and girls

CONTACTS

The CTU currently has staff at the following 4 sites across the UK:

BSPED CLINICAL TRIALS UNIT, CAMBRIDGE

University of Cambridge Department of Paediatrics, Box 116, Level 8, Addenbrooke's Hospital, Hills Road, Cambridge CB2 0QQ

• Prof. David Dunger (Unit Director)

Tel: (01223) 762 943 Email: dbd25@cam.ac.uk

• Barry Widmer (Data Co-ordinator)

Tel: (01223) 763 130 Email: bw215@cam.ac.uk

• Adelyn Thomason (Research Administrator)

Tel: (01223) 769 386 Email: at494@medschl.cam.ac.uk

• Diane Picton (Clinical Trials Monitor)

Tel: (01223) 762 944 Email: dp223@medschl.cam.ac.uk

• Catherine Fullah, East Anglia (Research Nurse)

Tel: (01223) 763 405 Email: caf41@cam.ac.uk

GLASGOW

Royal Hospital for Sick Children, Yorkhill, Glasgow, G3 8SJ

• Emma-Jane Gault (Research Assistant/Monitor)

Department of Child Health

Tel: (0141) 201 6952 Email: ej.gault@clinmed.gla.ac.uk

• Sheena McGowan, Glasgow (Research Assistant)

Department of Child Health

Tel: (0141) 201 0502 Email: s.mcgowan@clinmed.gla.ac.uk

• Sarah Casey, Glasgow (Clinical Trials Pharmacy Technician)

Pharmacy Department

Tel: (0141) 201 9367 Email: sarah.casey@nhs.net

MANCHESTER

• Ann McGovern, Manchester (Clinical Trials Practitioner)

2nd Floor Colwyn House, Royal Manchester Children's Hospital, Pendlebury, Manchester, M27 4HA

Tel: (0161) 922 2707 Email: ann.mcgovern@mmcmm.nhs.uk

BIRMINGHAM

• Research Nurse - to be appointed