

This information is for parents of boys with Duchenne muscular dystrophy.

Welcome to the Dubowitz Children's Neuromuscular Centre (DNC). This is a brief overview of our natural history studies and clinical trials that are running here at Great Ormond Street Hospital for Children.

### On-going Recruiting Studies

**Heart Protection Study:** A double-blind randomised multi-centre, placebo-controlled trial of therapies in preventing the development of cardiomyopathy. Sponsored by the British Heart Foundation

- Boys aged 7-12

**FOR-DMD:** A double-blind randomized trial to find optimum steroid treatment

- Boys aged 4-7 Open in the USA and Europe.

**GSK Imaging:** Evaluation and optimisation of muscle imaging biomarkers- MRI scan involved and upper limb physio assessment – no drug involved.

- Non-ambulant boys aged 11-17

**AFM Natural History Study:** Outcome measures in DMD. No drug involved.

- Boys aged 5-18 years

**Prosensa PRO053:** A Phase I/II, open-label, dose escalating with 48-week treatment study to assess the safety and tolerability, pharmacokinetics, pharmacodynamics and efficacy of PRO053 Prosensa exon 53 skipping

- Ambulant Boys aged 5 and above

**Summit Utrophin-SMT C1100:** A Phase I, open-label, single and multiple oral dose, safety, tolerability and pharmacokinetic study

- Ambulant Boys aged 5 to 11 years

**PTC124-GD-020-DMD:** A phase III efficacy & safety study of Ataluren in patients with nonsense mutation dystrophinopathy.

- Ambulant boys aged 7-16

## On-going Recruiting Studies

**Genethon-Pre-U7:** Study of clinical and radiological changes in boys theoretically treatable with exon 53 skipping – no drug involved. Opening January 2014 for boys aged 6 to 16

**Prosensa PRO045:** A phase IIb, open-label study to assess the efficacy, safety, pharmacodynamics and pharmacokinetics of multiple doses of PRO045. Prosensa exon 45 skipping

## Upcoming Studies

**Skip NMD (Sarepta)** A phase I/IIa clinical trial in Duchenne muscular dystrophy using systemically delivered morpholino antisense oligomer to skip exon 53" (SKIP-NMD) Please see - <http://www.skip-nmd.eu/>

**Summit Utrophin-SMT C1100:** A Phase II Double bind placebo Trial

- Ambulant Boys aged 5 to 11 years

## On-going Studies closed to recruitment

**Drisapersen GSK DMD 114349:** An open-label extension study of the long-term safety, an open-label extension study of the long-term safety, tolerability and efficacy of GSK2402968 Prosensa exon 51 skipping- suspended

**PTC124-GD-19-DMD:** An open-label study for previously treated Ataluren patients with nonsense mutation dystrophinopathy

If you would like more information or have any questions please do not hesitate to contact our research nurse Naomi Antcliff on [Naomi.antcliff@gosh.nhs.uk](mailto:Naomi.antcliff@gosh.nhs.uk)

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