

## SUMMARY OF FINAL REPORT

### Investigating balance therapies in cerebellar ataxia: Evaluating therapeutic outcome measures and feasibility testing of a novel intervention

**Principal researchers: Professor Brian Day, Dr Paola Giunti, Prof Jonathan Marsden, Dr Lisa Bunn**

**Original project:** 1 October 2006 - 31 March 2011

#### **Background and aims:**

Balance dysfunction is a prominent and disabling feature of spinocerebellar ataxia (SCA). It leads to falls and difficulties with activities of daily living (Van de Warrenburg et al., *Move Dis* 20:497-508, 2005). Our long-term aim was to provide a physical training protocol that people with SCA can use to alleviate their balance-related problems and improve their quality of life.

To achieve this long-term aim we devised a serial plan that consists of three broad stages:

1. Identification of the pathophysiological mechanisms underlying balance dysfunction in SCA. Application of this knowledge to design a novel therapy.
2. Identification and validation of outcome measures that, as a result of a balance-dysfunction intervention, best reflect the anticipated i) physiological changes, ii) functional benefits, and iii) quality-of-life improvements. Piloting the planned intervention (designed in stage 1) in a small sample to establish feasibility and compliance (including drop-out rates) of the procedure.
3. Execution of a randomised clinical trial (with known statistical power based on the data of stages 1 and 2) in order to assess the effectiveness of the piloted intervention using the validated outcome measures.

#### **Results:**

Investigations of sensory control mechanisms of balance in people with ataxia were made in order to identify the pathophysiological mechanisms underlying balance dysfunction in these people. An appropriate therapy was then designed based on

these findings. The therapy design was also informed by the researcher's experience of working with people with ataxia and by involvement of a patient support group (focus group format). It became evident from the group discussions that fatigue would potentially be a major limiting factor for compliance with therapy. For this reason design was directed towards achieving a safe, feasible, home-based intervention, which could be used in the future by community therapists.

The home-based intervention was developed and tested in a small pilot trial of six people with SCA6 and six healthy controls. The effects of the intervention on balance, daily living tasks and quality of life were measured. No adverse effects (falls, significant increases in fatigue or other) were experienced and the general feedback from subjects was that the therapy was challenging but manageable in the home environment.

Although a pilot study, initial results suggest there may be some improvements in balance with the intervention. These results support the design of a larger, randomised clinical trial and such a trial is required in order to confirm the benefits of the intervention and before it can be implemented in the NHS and beyond. More information will be released once the data has been published.

**Benefits to people with ataxia arisen/likely to arise from this research:**

This study presents the next stage in development of a future balance training intervention with a feasible delivery mechanism. It now requires evaluation by a larger clinical trial in order to provide sufficient evidence based for use within the NHS and beyond.

The study has established recommendations of use of a range of outcome measures with the potential to measure treatment effects for balance specific and a potentially a wider range of interventions (in accordance with the scale's construct validity).

The study supports the use of home-based delivery of therapy for patients with cerebellar degeneration.

**Publications arisen from this project:**

Manuscript in preparation.

**Conferences/ meetings where this research has been presented:**

- Presented in part at the World Congress of Physical Therapy, June 2011 (Amsterdam).
- Presented in part at an Ataxia study day for physiotherapists interested in neurology, April 2011 (Bristol).
- Plan to submit an abstract for a platform/poster presentation at the European Society of Physical and Rehabilitation Medicine, June 2012 (Athens).

**If the grant awarded funded a PhD studentship, has the student obtained their PhD? If not please give details of current status.**

Lisa Bunn PhD in neuroscience gained 2010 from the wider study of balance mechanisms in cerebellar disease.

**For more support or information please contact: Ataxia UK, Lincoln House,  
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