CBR Neurogenetics Clinic – Becker Muscular Dystrophy Newsletter August 2024

GRASP BMD – defining the endpoints in Becker muscular dystrophy

The team at the CBR Neurogenetic Research Clinic are looking for participants with a genetic diagnosis of Becker muscular dystrophy (BMD) to participate in a natural history study called Defining the Endpoints in Becker Muscular Dystrophy. In clinical trials the 'endpoints' are the key objective measures that are predicted to show whether the treatment is working.

By participating in GRASP BMD you'll be part of a multi-site international study that aims to include eighty men from sites in the UK, USA, and New Zealand.

This study is designed to find better ways to record how Becker muscular dystrophy impacts people over time.

We'll be exploring the natural history of BMD, how the disease progresses over time, and determining the feasibility and suitability of clinical outcome assessments.

These analyses will evaluate the responsiveness of the measures, validity, and help define an ideal clinical trial population. Blood biomarkers will also be investigated.

The New Zealand GRASP BMD Team
Sarah Mollet Research Physiotherapist, Dr Melanie Glenn Neurologist & Sub-Investigator, Miriam Rodrigues Neurogenetic Research Lead, Sarah Nagar GRASP BMD Study Co-ordinator, A/ Prof Richard Roxburgh Neurologist & Principal Investigator GRASP BMD

The study visits will take place at the CBR Neurogenetic Clinic at the University of Auckland. The first (baseline) visit takes place over two days. Subsequent visits will take just one day and are 12 months apart. All travel and accommodation costs will be covered.

As part of each study visit you'll see a physiotherapist, have your heart checked, see a doctor, complete some questionnaires, and provide a blood sample.

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We're working with Optimal Clinical Trials to research an investigational treatment to understand if it can help people living with Becker muscular dystrophy. The treatment, Sevasemten (formerly known as EDG-5506) aims to limit muscle breakdown and disease progression in people with the condition.

People diagnosed with BMD may be eligible for this study if they:
- Are biologically male, aged between 18 to 50 years (inclusive)
- Are able to walk 100 metres with or without a mobility device like a cane or walker
- Have not taken oral steroids for management of BMD in the last 6 months

The study will take approximately 18 months and include at least seven in-clinic visits. Study treatment and all study tests and procedures are provided at no cost. Participants will have all transport arranged – there will be no cost to participants. Trial participants may have the opportunity to continue taking the study medication after the trial is finished. Participants will also be helping researchers learn more about the condition and aiding in the development of a potential new treatment for BMD.

Vamorolone

It's an exciting time for treatment development in Becker muscular dystrophy. Outside of New Zealand a phase two clinical trial of vamorolone, which is an improved substitute for corticosteroids such as prednisone or deflazacort, is taking place in the US and Italy. We're watching this trial closely and look forward to reporting the results as they're released.

Resources for families with Becker Muscular Dystrophy

https://beckermusculardystrophy.com/ is a great online resource for people living with Becker muscular dystrophy. Although it leads with a disclaimer that the information is designed for adults with BMD living in the USA much of what it contains is relevant to families here in New Zealand too. The website, sponsored by Edgewise Therapeutics, includes useful information about the clinical trial process as well as Becker-specific information.

Participating in research

Lisa Fraser, Research Assistant, is currently updating Pūnaha Io the New Zealand Neurogenetic Registry & Biobank for people with BMD so don't be surprised if she contacts you!

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You're welcome to update your contact details at any time or if you have family members with Becker muscular dystrophy who would also like to enroll please contact Miriam Rodrigues - email neurogenetics@adhb.govt.nz

Recruitment for both GRASP BMD and Grand Canyon is taking place through Pūnaha Io the New Zealand Neurogenetic Registry & Biobank.

Best wishes from the teams at the University of Auckland's CBR Neurogenetics Clinic and Pūnaha Io - the New Zealand Neurogenetic Registry & Biobank!