Muscular Dystrophy Campaign: Putting Some Financial Muscle Behind Finding a Cure

Marita Pohlschmidt

Introduction

The Muscular Dystrophy Campaign grew from the efforts of Lord John Walton and Professor Fred Nattrass, leading neuromuscular clinicians who teamed up with families of children with muscular dystrophy. Our goal, originally, was to fund research aimed at finding a treatment for one condition—Duchenne muscular dystrophy.

Over the years the charity has expanded its mission, providing information, support, and equipment to patients as well as campaigning for better health care services for those with these conditions. We continue to fund research on Duchenne muscular dystrophy, which affects approximately 1 in every 3,500 male infants, but we now also support research into more than 60 different kinds of muscle and nerve diseases. As such, we are considered the leading voice on neuromuscular research in the U.K. The Muscular Dystrophy Campaign also organizes and sponsors scientific meetings and conferences to facilitate collaboration and communication between scientists and clinicians, which we believe is essential for promoting the quick translation of research findings into clinical trials and new treatments.

Research Goals

The Muscular Dystrophy Campaign funds three particular research areas: basic science, the development of therapeutic approaches, and projects to improve the clinical management of muscle disease. Funding research on muscle stem cells forms part of our research strategy, and the U.K. has some of the best muscle stem cell researchers in the world. We continue to fund research on Duchenne muscular dystrophy, which affects approximately 1 in every 3,500 male infants, but we also support research into more than 60 different kinds of muscle and nerve diseases. As such, we are considered the leading voice on neuromuscular research in the U.K. The Muscular Dystrophy Campaign also organizes and sponsors scientific meetings and conferences to facilitate collaboration and communication between scientists and clinicians, which we believe is essential for promoting the quick translation of research findings into clinical trials and new treatments.

These characteristics are the reason why muscle stem research has the potential to develop into an efficient treatment for people with muscular dystrophy and related muscle diseases. However, most of the conditions we support are genetic, and the major challenge and the next steps would be to investigate ways to repair the genetic defect. Even if it would be possible to use an individual's own stem cells for replacing wasted muscle tissue, all those resulting muscle fibers would still carry the same genetic defect.

Muscle stem cell research is still in its infancy, and at the moment we fund four different projects studying the biology of satellite cells at a total cost of just over $919,000 (£580,000). One such project is being undertaken in the laboratory of Dr. Jenny Morgan at University College London’s Institute of Child Health. Dr. Morgan is working on the impact of the muscle environment—the extracellular matrix— on the ability of satellite cells to divide and repair. Her research is also directed at understanding why satellite cells behave differently in individuals with muscular dystrophy, investigating whether it is the muscle environment in dystrophic muscle that causes satellite cells to eventually fail to repair damage or the satellite cells themselves that fail.

Another researcher we fund is Dr. Peter Zammit at King’s College London. Dr. Zammit’s research is looking at the different types of satellite cells in the hope of identifying and isolating the cells that have the greatest ability to regenerate muscle. He is also exploring the role that muscle stem cells have in the progression of a particular type of muscular dystrophy called facioscapulohumeral muscular dystrophy.
A Strong Voice
The Muscular Dystrophy Campaign is also a strong advocate for stem cell research and has increasingly been the first point of contact for the media. During the past few years we have been vocal in some very high-profile news stories, presented by *BBC Online* and the *Sunday Times*, among others. Although we have not funded this type of research ourselves, we support embryonic stem cell research, as these cells have potential to divide almost infinitely and can specialize into many cell types.

We are careful, however, not to raise false hopes among supporters and patients. We communicate stem cell research for muscle disease as a potentially powerful treatment that still has a long way to go before it comes into the clinic. Feedback from our supporters is that they understand this, and a cure is not something that they expect to happen tomorrow.

Meet Marita Pohlschmidt, Ph.D.
Dr. Marita Pohlschmidt is director of research at the Muscular Dystrophy Campaign, where she oversees an annual U.S. $1.88 million (£1.2 million) budget invested in ways designed to increase the understanding of muscle function and muscle disease and to ensure an efficient bench-to-bedside transition of promising technology into a clinical benefit. Before she joined the charity, Dr. Pohlschmidt also worked as a scientist in the field of molecular genetics for more than 15 years and was involved in the development of a gene therapy approach for Duchenne muscular dystrophy.