**Research Communications Group Application**The information on this form will only be shared with staff at Muscular Dystrophy UK. We will use the information to contact you about your application, and to ensure we keep a balanced membership of the Group, in terms of expertise, geographic location and demographic. Your details will be securely stored on our database; you can read our Privacy Policy at: <https://www.musculardystrophyuk.org/privacy-cookies/>.

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| **Name** |
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| **Address:** |
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| **Email address:** |
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| **Telephone:** |
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| **Do you have any potential conflicts of interest?** *For example, involvement with charitable or commercial organisations. If yes, please provide details.* |
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| **Education and Qualifications**  *Please provide details below of your undergraduate education, post-graduate studies, and any other qualifications relevant to this volunteering role.* |
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| **Why would you like to volunteer?**  *Please tell us why you’d like to join the group. We’re also interested to hear about any experience you have communicating science to a lay audience and any experience of muscle-wasting conditions.* |
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| **Written Task**  In order for us to see if you’re suitable for the role, and for you to assess if this role is something you would enjoy, we kindly ask you to complete the following written task.  We don’t expect you to spend longer than 30 minutes – 1 hour. |
| *Instructions:*  Imagine that a family of a child with Duchenne muscular dystrophy found the following press release on the internet. They have shared it with us because they don’t fully understand what it means.  Please summarise below the release (max. 250 words), explaining the key findings and implications of the research for the family.  **Press release: New drug molecules hold promise for treating fatal child disease**  New drug molecules hold promise for treating fatal child disease Scientists at the Universities of Exeter and Nottingham have identified a way to “rescue” muscle cells that have genetically mutated, paving the way to a possible new treatment for rare childhood illness such as Duchene Muscular Dystrophy (DMD). The study published in the Proceedings of the National Academies of Science, USA, used novel drugs being developed at the University of Exeter, which “metabolically reprogramme” the cellular energy production centres in muscle cells, by providing them with a fuel source to generate metabolic energy.  DMD is a genetic condition caused by a mutation in a gene called dystrophin and results in progressive irreversible muscular degeneration and weakening. Its symptoms include muscle atrophy leading to a loss of the ability to walk in children for which there is no known cure and there is no known cure. Currently, the condition is treated with steroids, such as prednisone, but they can stop working and side-effects are common. The research, funded by the Medical Research Council (UK) and United Mitochondrial Disease Foundation in the USA, was led by Professors Nate Szewczyk in Nottingham and Matt Whiteman in Exeter focussed future alternative ways to improve muscle performance when the dystrophin gene is missing or is defective.  The research team comprising of scientists from Australia, USA, The Netherlands and Germany as well as the UK first used microscopic worms (C. elegans) and then mice with specific genetic mutations affecting muscle strength, that match mutations that cause DMD in humans. The team found that these animals had defects in gait, movement, and muscle strength, and had marked defects in the structure their muscle mitochondria, the tiny organelle responsible for cellular energy regulation. The animals also had lower levels metabolic enzymes used for the generation of the gasotransmitter hydrogen sulfide in their muscle, as well as lower levels of the gas itself. Treating these animals with a compound called NaGYY which replaced the lost hydrogen sulfide partially reversed some of the muscle and mitochondrial defects in the same way the standard of care drug prednisone did. However, specifically targeting mitochondria with hydrogen sulfide using the compound AP39, exhibited the same effects but at 3.7 million-fold lower dose.  Professor Nate Szewczyk of the Ohio Musculoskeletal & Neurological Institute, USA commented “Steroids are very effective and safe drugs but their use over a long period of time causes effects wear off and they can have some very unpleasant and life-changing side effects. The compounds we’ve used in our study are not steroids and they work in a very similar way to these drugs give the same improvement in muscle function, but at a much, much lower dose and because they are not steroids, they are unlikely to produce steroidinduced side effects such as weaker muscle and decreased ability to fight infection”.  PhD student Rebecca Ellwood added “Life first emerged on earth in a sulfide rich environment and thrived for billions of years before it was replaced by the oxygen we have today. Our cells and our mitochondria have maintained the ability to both make and use very small amounts of sulfide to keep healthy. Our study now shows that in DMD models, this metabolic pathway is defective, offering a potential for therapeutic intervention to correct this defect”. Professor Matt Whiteman, of the University of Exeter Medical School, who developed AP39, said: “We’re really excited that our findings show that a deficit in muscle sulfide may contribute to the development of Duchenne Muscular Dystrophy. Rectifying this deficit may lead to new treatment approaches for this and other currently incurable diseases, without relying on potentially harmful steroids. At Exeter we are developing more advanced approaches to target muscle mitochondria, and we aim to spin-out a new biotech company called 'MitoRx Therapeutics' to develop these newer approaches for clinical use during 2021.”  Dr Kate Adcock, Director of Research and Innovation at the charity Muscular Dystrophy UK, said: “We welcome research that increases our understanding of molecular pathways that could both contribute to the symptoms of Duchenne muscular dystrophy and offer potential new therapeutic targets. Although a long way from patient studies, this research has shown interesting results in animal models of Duchenne muscular dystrophy and it is encouraging to see these early-stage studies for such a complex, rare condition.”  Full name of paper -- Mitochondrial hydrogen sulfide supplementation improves health in the C. elegans Duchenne muscular dystrophy model  **Notes to editors**: The press embargo on your article will lift on February 22, 2021 at 3:00 PM U.S. Eastern time  **Your summary:** |

I confirm to the best of my knowledge that the information given on this form is correct

**Keep Involved**

We'd love to share updates with you about MDUK’s work and opportunities to take part in events and other ways to help beat muscle-wasting conditions. Please click if you are happy to be contacted in the following ways:

Email

Text

Phone

*Our privacy policy is available* [*on our website*](https://www.musculardystrophyuk.org/privacy-cookies/) *or by phoning 0300 012 0172.*

Please email your completed form to our Research Communications Officer, Andrea Gubas at [a.gubas@musculardystrophyuk.org](mailto:a.gubas@musculardystrophyuk.org). If you have any questions, please contact the research team on 020 7803 4827 or at [research@musculardystrophyuk.org](mailto:research@musculardystrophyuk.org).