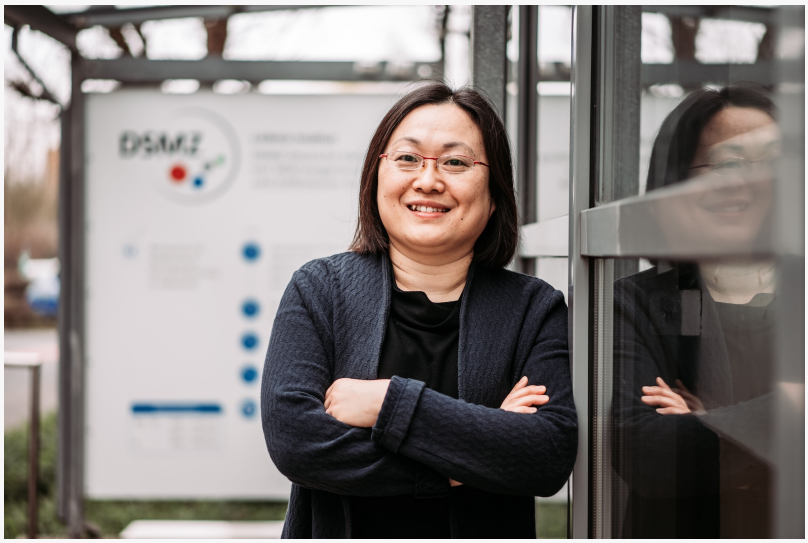
ZNM - Zusammen Stark! Awards €81,400 Grant to build 3D Centronuclear Myopathy Muscle Model

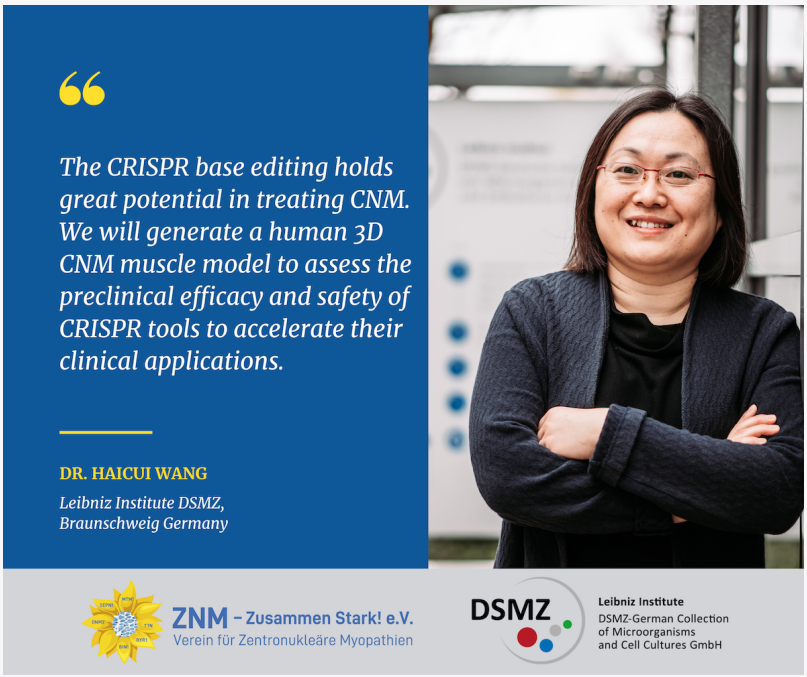
Joint press release ZNM – Zusammen Stark! e. V. / Leibniz Institute DSMZ

BRAUNSCHWEIG, NIEDERSACHSEN, GERMANY, November 6, 2023 /EINPresswire.com/ -- ZNM - Zusammen Stark! e. V. an association dedicated to supporting patients with rare muscle conditions known as centronuclear myopathies (CNM), is proud to announce the allocation of an €81,400 grant to a groundbreaking research initiative led by Dr. Haicui Wang, a distinguished scientist from the Department Human and Animal Cell Lines of the Leibniz Institute DSMZ-German Collection of Microorganisms and Cell Cultures GmbH. This significant grant, entirely funded by generous donations, aims to advance potential treatments for CNM by creating several muscle models (each with a specific CNM mutation) and then attempting treatment using the CRISPR technology.

Centronuclear myopathy is a group of rare inherited diseases caused by mutations in specific genes, including MTM1, DNM2, BIN1, RYR1, and TTN. The genetic diversity among individual CNM patients presents unique challenges in developing a unified



Dr. Haicui Wang, head of the working group Rare Disease Models at the Leibniz Institute DSMZ



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therapy strategy. Currently, gene therapy approaches for CNM involve replacing disease-causing genes or functional compensation through related genes. In this regard, the innovative use of CRISPR-base editors opens new possibilities for precise mutation correction.

3D CNM Muscle Model to Test the Efficacy and Safety of CRISPR Treatment for CNM Patients
"The CRISPR gene editing tools hold great potential in treating genetic diseases including CNM. Thus, the objective of this project is to create and use a human 3D CNM muscle model to evaluate the efficacy and safety of precise mutation correction using CRISPR-base editing before testing it in patients to accelerate their clinical applications", highlighted Dr. Wang, scientist at the Leibniz Institute DSMZ and principal investigator of the project. To achieve this, Dr. Wang will create ten 3D CNM muscle models with mutations drawn from four CNM genes: MTM1, DNM2, BIN1, and RYR1, investigate muscle function impairment and, ultimately, attempt to treat the mutated CNM muscle model by delivering base editors in mRNA form.
Dr. Wang's innovative research aims to improve our understanding of CNM's genetic basis and provide a platform for developing potential therapies. Furthermore, it promises to enhance the genotype-phenotype correlation, aiding in future CNM diagnosis and mechanistic studies.

Hope for a Brighter Future in the Community
Dr. Holger Fischer, President of ZNM - Zusammen Stark!, expressed the organization's enthusiasm for supporting this project: "We are thrilled to fund Dr. Wang's research initiative. CNM are rare conditions, and finding effective treatments has been challenging. Dr. Wang's project represents a significant step towards potential therapies for CNM patients. We are proud to support this groundbreaking work."
This grant continues ZNM - Zusammen Stark!'s commitment to advancing research and improving the lives of individuals and families affected by CNM. It is part of their broader mission to connect affected individuals, support their families, and invest in research that can make a difference.

About Centronuclear Myopathies
Centronuclear myopathies are very rare muscle diseases. The most affected children with centronuclear myopathies cannot walk; they need a feeding tube and a ventilator to support their breathing. This, however, does not stop their will to learn, play, and discover the world, as other children their age.

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About ZNM – Zusammen Stark! e.V.
ZNM – Zusammen Stark! e. V. (CNM – Together Strong!) is a self-help association for myotubular myopathy and other centronuclear myopathies (CNM). We represent 258 individuals from 92

families with a CNM in Germany, the Netherlands, Austria, Switzerland, and Belgium. As a self-help organization, our main goal is to connect those affected and their families and to support each other in our daily lives. This is mainly done through our yearly family conference, webinars, and our closed Facebook support group. We also actively invest in research to find a treatment for these conditions. For more information, please visit www.znm-zusammenstark.org/en/

About Leibniz Institute DSMZ-German Collection of Microorganisms and Cell Cultures
The Leibniz Institute DSMZ-German Collection of Microorganisms and Cell Cultures is the world's most diverse collection of biological resources (bacteria, archaea, protists, yeasts, fun-gi, bacteriophages, plant viruses, genomic bacterial DNA as well as human and animal cell lines). Microorganisms and cell cultures are collected, investigated and archived at the DSMZ. As an institution of the Leibniz Association, the DSMZ with its extensive scientific services and biological resources has been a global partner for research, science and industry since 1969. The DSMZ was the first registered collection in Europe (Regulation (EU) No. 511/2014) and is certified according to the quality standard ISO 9001:2015. As a patent depository, it offers the only possibility in Germany to deposit biological material in accordance with the requirements of the Budapest Treaty. In addition to scientific services, research is the second pillar of the DSMZ. The institute, located on the Science Campus Braunschweig-Süd, accommodates more than 85,000 bioresources and biomaterials and has around 220 employees. www.dsmz.de

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