

## News Release

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FOR IMMEDIATE RELEASE

Dr. Nicolau Receives 2022 Development Grant from American Neuromuscular Foundation

**Rochester, Minn. (December 08, 2022)-** The American Neuromuscular Foundation (ANF), is excited to announce the 2022 Development Grant Recipient, Stefan Nicolau, MD, for his research project “CRISPR/Cas9 correction of a common Duchenne muscular dystrophy (DMD) deletion.” Dr. Nicolau is a research fellow at the Abigail Wexner Research Institute at Nationwide Children’s Hospital in Columbus, OH.

Dr. Nicolau’s team is working to develop a new method to correct a common DMD mutation using CRISPR/Cas9 gene editing. “This method makes use of a process called homology-independent targeted integration (HITI), which is a more efficient way of introducing DNA into muscle cells. Unlike most previously described gene editing methods for DMD, this also allows production of the full-length dystrophin protein, rather than a shortened isoform,” he explained.

The team’s short term research goals are to develop and optimize a gene editing system based on HITI to correct a common DMD mutation. This will be achieved through a series of experiments in patient-derived cells and in a mouse model of DMD.

They are also hopeful that their research will help advance HITI and other gene therapy approaches for DMD towards clinical use. “Although we are planning to initially develop this method for correction of one particular mutation, we anticipate that it can be adapted to correct a broader range of mutations in the same region,” he said.

Following completion of the research, Dr. Nicolau will present his findings at an AANEM Annual Meeting. “I am very grateful to the ANF for offering me this opportunity as an early career investigator. I look forward to being able to share results of the research with the ANF and the NM community at large in the coming years.”

### **About American Neuromuscular Foundation (ANF)**

Based in Rochester, MN, ANF is a nonprofit organization dedicated to strengthening the global effort to cure neuromuscular disease. As part of its mission to promote the advancement of scientific research and education, the ANF offers annual development grants intended as seed money to help launch research into muscle and nerve disorders by promising new researchers. For more information about ANF, visit [neurmuscularfoundation.org](http://neurmuscularfoundation.org).

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