



A Personal Connection to Advancing Duchenne and Becker Research

Alyson Fiorillo, PhD, Assistant Professor in the Department of Cellular, Molecular, and Genetic Medicine at Virginia Commonwealth University (VCU) School of Medicine, is studying how inflammation, microRNAs, and dystrophin biology influence Duchenne muscular dystrophy (DMD) and Becker muscular dystrophy (BMD), helping pave the way for the next generation of therapies.

Grappling with unknowns has appealed to Dr. Fiorillo from a young age. “I’ve always liked to know the why of things,” she says. “I like puzzles. I like figuring things out. I just like delving into something and understanding it thoroughly.”

Her connection to the neuromuscular disease community also began years ago. Before starting graduate school, Dr. Fiorillo spent several summers volunteering at [MDA Summer Camps](#), where she was inspired by the friendships, traditions, and sense of community that defined camp life.

“The camp was so amazing,” Dr. Fiorillo says. “These kids knew the counselors; they knew all the camp songs; they knew each other. It really was an amazing week.”

Dr. Fiorillo earned her doctorate in molecular biology at Northwestern University in Evanston, Illinois, in 2011, then completed postdoctoral training under renowned muscular dystrophy researcher Eric Hoffman, PhD, at Children's National Hospital in Washington, DC. Along the way, her scientific interests increasingly aligned with the patients she first met at MDA Summer Camps.



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Building the Fiorillo and Heier Labs

Dr. Fiorillo and her husband, Christopher Heier, PhD, both established independent laboratories in 2017. Although each initially pursued separate research, their work naturally converged on the biology of muscular dystrophy.

The COVID-19 pandemic unexpectedly accelerated that collaboration.

With limited access to the laboratory and young children at home, the pair alternated childcare responsibilities while keeping their research programs moving forward. What began as a necessity evolved into a productive scientific partnership that helped launch several new research initiatives, including the development of the first mouse model for BMD. Today, the two labs operate as one — they co-mentor graduate students and technicians and hold joint lab meetings, which lets students draw on both labs' expertise and move projects forward faster than either program could alone.

The Fiorillo and Heier Labs are based in the Department of Cellular, Molecular, and Genetic Medicine at VCU, where the team studies both DMD and BMD and collaborates closely with clinicians and researchers across the institution. The laboratory's work focuses on understanding disease mechanisms and identifying therapeutic strategies to improve patient outcomes.

Understanding the biology behind disease

One major focus of Dr. Fiorillo's research centers on microRNAs — small molecules that regulate gene expression.

During her postdoctoral work, Dr. Fiorillo helped identify several microRNAs that can suppress production of dystrophin, the protein that is missing in DMD and is partially functional in BMD. That discovery raised an important question: Could blocking these microRNAs improve current therapies, or even increase dystrophin production on its own? This work was supported by an MDA Research Grant in 2023.

Today, Dr. Fiorillo's lab pursues several research directions, including two that address the question about microRNAs. One investigates whether inhibiting disease-associated microRNAs can reduce inflammation in muscle tissue, while the other explores whether targeting those same molecules can improve the effectiveness of exon-skipping therapies.

Her team has also discovered that some of these microRNAs resemble viral RNA molecules closely enough to activate inflammatory immune pathways, opening another potential avenue for therapeutic intervention.

Expanding BMD research

While significant therapeutic advances have been made for DMD in recent years, Dr. Fiorillo believes BMD deserves greater research attention.

Her laboratory, along with Dr. Heier's, developed the first genetically engineered mouse model that mimics the most common BMD mutation, creating an important tool for studying disease progression and evaluating potential therapies. The work addresses a longstanding gap that has slowed preclinical research for BMD.

The team has since developed additional mouse models to better understand why different dystrophin gene mutations produce varying disease severity. They are also collaborating with experts in metabolomics and lipidomics at VCU to identify biological pathways that may contribute to disease progression and reveal new therapeutic targets.

"I think we need to have a multifaceted approach to treating DMD," Dr. Fiorillo says. "And I think BMD patients also really need more treatments that are specifically tailored toward them, especially as dystrophin-restoration therapies produce a Becker-like dystrophin, making treated DMD patients increasingly resemble BMD patients at the molecular level."

Looking ahead

Although finding effective treatments is the ultimate goal of muscular dystrophy research, Dr. Fiorillo says meaningful progress comes through sustained, foundational work.

She remains particularly optimistic about advances in next-generation exon-skipping therapies, which she believes could significantly improve treatment effectiveness over the coming years. At the same time, she emphasizes the importance of continued investment in therapies specifically designed for BMD, especially as more individuals with DMD are surviving longer with partially restored dystrophin expression.

Just as important as the science itself is mentoring the next generation of researchers.

“I really love figuring out the puzzle,” Dr. Fiorillo says. “But also seeing young scientists grow and learn and come up with even better ideas than I might is highly rewarding.”

Despite today’s challenging research environment, Dr. Fiorillo says staying connected to the neuromuscular community helps keep the work in perspective.

“The Muscular Dystrophy Association meetings, in particular, are always quite rewarding because you really see the progress in the field year to year,” she says. “You see the impact on the patients and their families. I think that’s where we should keep our focus.”

Resources

- [The Fiorillo Lab, Virginia Commonwealth University](#)
- [The Heier Lab, Virginia Commonwealth University](#)
- [MDA Clinical & Scientific Conference](#)
- [MDA Summer Camp](#)