



The Foundation for Casey's Cure, Inc
162 Wellington Drive
LaGrange, GA 30241



SCIFAM Conference, July, 2022
Nashville, TN

Hello to all of our donors and friends (whose emails we have)!

Casey and I have just returned from our first SciFam Conference where patient advocacy groups like The Foundation for Casey's Cure and others are able to listen to and meet with the researchers working on the cutting edge science that could someday bring a treatment or even a cure for rare diseases like Casey's.

Speaking as a mom, I couldn't be more excited.

#1 News:

Casey and I are back from our first neuromuscular conference this week where Casey was able to once again see Jennifer Roggenbuck from the Ohio State University Wexner Medical Center, Columbus OH lab. Jennifer took a sample of Casey's right lower leg muscle and skin sample in 2021. I was finally able to meet her!

Casey and I have had to sign a confidentiality agreement in order to participate in this particular session. I am unable to explain anything more than this: In examining Casey's tissues, Jennifer has been able to identify a quality that she hadn't expected to see in Casey's tissues. Truthfully, I'm not sure if that is good news or bad, but **I know that it means that Casey's sample is adding to the body of science** work that is hard to come by in the HMERF world, simply because HMERF patients are so rare. The recognition that Casey's tissues have added something special to this body of work on Hereditary Myopathy with Early Respiratory Failure shows that even small patient advocacy groups CAN make a difference in the research that will ultimately unlock a treatment or a cure.

So when you ask yourself, "is my donation actually making a difference?" we can answer with a resounding YES!

No Dedicated Funding

We've also learned that Jennifer Roggenbuck's lab doesn't have any routine dedicated funding for her work. I can only reason that because HMERF samples provide such a small pool of affected patients, her work at Ohio State is funded on an ad-hoc basis with monies left over from other studies. Again, the reason is because there are so few HMERF patients.



What DOES matter is that your donations enable The Foundation for Casey's Cure to make a contribution to her lab and allow us to urge other HMERF patients to donate samples, which makes an incredible difference in her ability to study HMERF and how the protein coding can be altered to allow a genetic answer to be found. Her next step is to analyze tissue using a mass spectrometer so she can view the samples at the chemical level. (To those of you who happen to be NCIS fans of the character Abby Schuto, the mass spectrometer yields those case altering results and I can only hope the same will happen here!)

#2 News:

We also had the opportunity to meet Dr. Johan Lindqvist, who has been using Casey's tissues to study the HMERF 'trigger mechanism'. To see his surprise at actually meeting Casey was amazing! I'm told that seeing the face of the tissue donor brings home to the researcher that his work really matters to someone's life. In this instance, it certainly seemed that way.

His Work

Dr. Lindqvist is an associate of Dr. Henk Granzier in the Granzier Lab at Arizona Medical School at Arizona State University.



You may remember that Dr. Granzier was one of the very first researchers who, in 2019, demonstrated interest in Casey's tissue samples. Since Jennifer Roggenbuck works in conjunction with Dr. Granzier's lab she was able to share Casey's sample with the Granzier Lab.

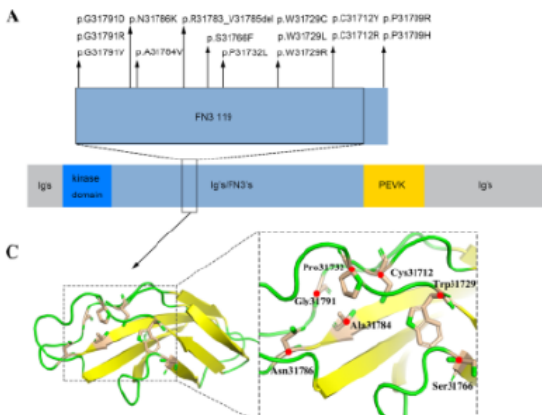
Using Casey's Tissues

As an associate of the lab, Dr. Lindqvist has been using Casey's tissues to study the 'trigger mechanism' within the cells/DNA that cause the muscle-making protein to stop working. Dr. Lindqvist's work is focused on identifying what stops this process from happening, setting the muscular dystrophy in motion. He is looking into several causal issues but has yet been unable to make any determination.

Meeting Casey

His surprise at actually meeting Casey was amazing! To see the face of the tissue donor brings home to the researcher that his work really matters to someone's life. (A little background here; Science knows that HMERF can strike between the ages of 30 and 70. The 'when' is the triggering mechanism and determining how it is triggered is where his research is currently focused.) We'd been told of the emotional impact meeting a donor can have on a researcher but to see the surprise and the interest was, well, quite extraordinary. When he realized that Casey was one of the providers of the tissue samples he's been working with to ascertain this triggering mechanism, he quickly suggested that he may need more tissue samples in order to continue his work. We were very excited to hear that! Getting more of Casey's tissues into the hands of the lab that is specifically working with her samples seems to me that her tissues and the money that lets her travel there is more important than ever!

#3 News:



There may be a possibility to have Casey's DNA re-coded and aligned to the current DNA map used by the researchers today. (Casey's original DNA test was conducted in 2017 and while an update to the encoding of her disease won't change her diagnosis, it will put it in today's current genome map. And as her mom, I cross my fingers that one piece of DNA coding update may just help the researchers! Who knows?!)

We can only hope we can continue to raise money to get Casey's tissues to more researchers so that a treatment for her is something that is closer than I can imagine.

In closing, We want to thank each and every one of you for your financial and emotional support. To the untrained eye, Casey still looks very un-affected by her disease. But Casey's challenges are increasing in incremental stages, making time of the essence.

We have some events happening in the coming months: Raffles, a Bike Ride, and someone has suggested we have a cake auction! So stay tuned because if she has to go Arizona, I'll be sure to let you all know... (As an official transplant to Georgia, "y'all" is just not something I can pull off without having my friends erupt in laughter...!)

Please stay safe and thank you again for your continuing help. Casey's tissue from our small non-profit IS adding to the body of science that will one day, hopefully sooner than later, find a treatment for her muscular dystrophy. The clock is ticking...

On behalf of the entire Foundation, thank you.

Chris Duane
Casey's Mom & Founder
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Hi!

We've sent this letter to you because we didn't have an email address on file but we didn't want to you miss out on everything you have made possible! Our goal is to bring you up to date on just how much your donations have accomplished as well as to inspire you to continue your financial support for research on rare muscular diseases like Casey's muscular dystrophy at www.caseys-cure.org.

Did you know that your donation inspires Casey? Because of your donation, she is living her life as if she has a future without the complicated end-of-life issues that face her. And she has YOU to thank for that! So this is a big shout out from us! THANK YOU!

As her mom, and on behalf of the rest of the Foundation's Family of supporters and fans, please accept our deepest gratitude and hope that you will make another contribution this year so Casey can actually get out to Arizona, donate that tissue that Dr. Lindqvist has requested that will bring his work to an outcome worthy of medical investigation from the grant and pharmaceutical communities.

Thank you so much! And please reach out if you have any questions. Your questions are welcomed at any time! :)

Thank you again,
Christine Duane
Executive Director and Casey's mom...

PS. If you'd like us to reach you by email, please send it to me at Contact@caseys-cure.org. I'll add you right on! (And we know how much we all hate our mailboxes filled with junk, so you can be sure that your email is safe with us and it will never be shared by us to anyone else.)