

A mitoPodCast Interview with Dr. Neal Sondheimer

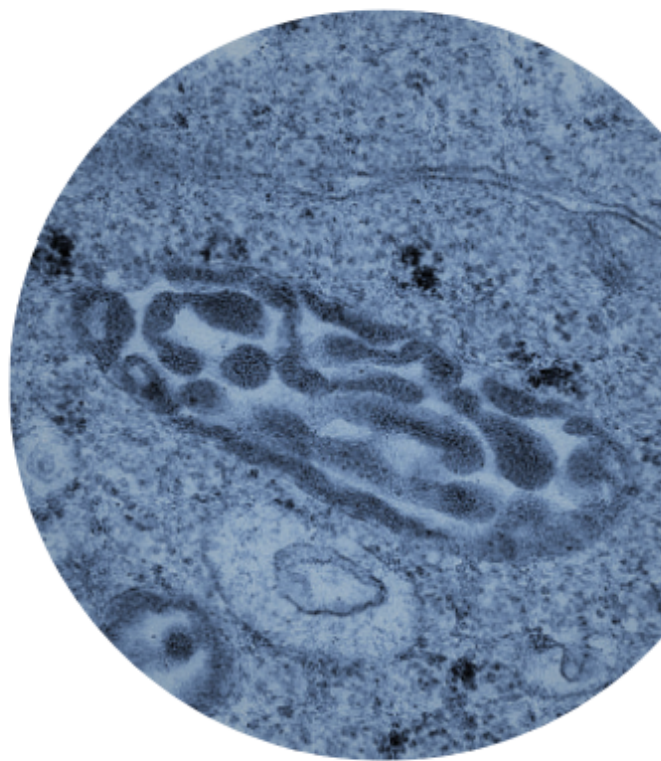


MEET DR. SONDHEIMER

Dr. Sondheimer is a staff physician and geneticist at Toronto's Hospital for Sick Children. He is also an Associate Scientist with the SickKids Research Institute's Genome and Genome Biology Research Program. After completing an MD/PhD at the University of Chicago, he pursued clinical training in clinical biochemistry, genetics and pediatrics at the Children's Hospital of Philadelphia.

WHAT IS HETEROPLASMY?

Heteroplasmy refers to the presence of different allele copies of mitochondrial DNA (mtDNA) in a cell. Some of these copies are disease-causing. Part of Dr. Sondheimer's work involves the analysis of patient mtDNA to see if there are disease-causing variants.



HETEROPLASMY AS A THERAPY

If normal mtDNA is present in patient cells, one could make that mitochondrial genome take over the cell. This way, one could possibly reduce or eliminate the symptoms of disease. One would need to make the "bad" copies of mtDNA replicate less or the "good" copies replicate more. Dr. Sondheimer's lab is attempting to use a class of small molecules to achieve this.

HIS NEXT STEPS

While heteroplasmy alteration has been done in patient cell lines, Dr. Sondheimer's lab wishes to see if it can be used as a therapy in patients. However, structuring clinical trials for mitochondrial disease patients has its challenges as these diseases are complex. Nevertheless, his lab is working hard to make this a reality.



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ACCELERATING DRUG DEVELOPMENT

Working with Health Canada and the United States' FDA, we need to find the right balance between balancing patient safety and accelerating drug development. Patients and their families demand a better strategy as patients cope with these often fatal diseases.

PROMISING NOVEL THERAPIES?

Mitochondrial disease doesn't just affect one organ system. There are some promising developments in direct gene and mRNA therapies that allow for multiple organ systems to be targeted. Enhancing mitochondrial quality control to select for better performing mitochondria in cells is another exciting avenue.



IMPORTANCE OF COLLABORATION

In order to find solutions to mitochondrial diseases, we have to be well-organized and work as a team to advocate for resources and to organize clinical trials. Not only does a collaborative network enable this, it allows for patients to seek out assistance from clinicians and for those clinicians to direct information and resources to researchers who are working to develop new therapies.

FINAL MESSAGE AND GETTING IN TOUCH

Those with family members who have mitochondrial disease are part of a large community that is ready to provide support. Please reach out, you are not alone!

If you have questions about Dr. Sondheimer's work, please visit this [website](#).

