

# From QPatch to QPatch Compact – NMD Pharma breaks new ground in neuromuscular diseases

NMD Pharma is devoted to discovering and delivering life-transforming therapies for people living with neuromuscular diseases. As a research-based company, they are determined to be in control of their research and quality. Sophion's automated patch clamp instruments have been a part of NMD Pharma's research, and all the knowledge generated along the way has been an important part of NMD Pharma's journey. NMOPHARMA



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Martin Gruwier Broch-Lips, Head of biological research Anders Riisager, Senior Scientist

NMD Pharma is a spin-off from the Department of Biomedicine at Aarhus University in Denmark. Based on 15 years of basic research, NMD Pharma achieved an orphan drug designation for NMD670. They have grown into a clinical stage biotech company and have achieved proof of mechanism in a Phase IIa study in patients with myasthenia gravis.

NMD Pharma is a clinical-stage biotech company with a focus on discovering and delivering life-transforming therapies for people living with neuromuscular diseases. Until recently, NMD Pharma has been using the QPatch® regularly to generate data for their research. Today, NMD Pharma is using Sophion's new QPatch Compact instrument and finds its flexibility to be well-suited to their current research projects.

# Making a difference through electrophysiology

Neuromuscular diseases are serious and often rare conditions with limited treatment options. NMD Pharma is hoping to change that treatment landscape. "We are working within our true core competencies – electrophysiology, physiology, and pharmacology – to produce therapies for people with neuromuscular diseases such as myasthenia gravis," says Martin Gruwier Broch-Lips, Head of Biological Research at NMD Pharma.

The company began after one of its founders, CEO Thomas Holm Pedersen, was doing research on activity-related regulation of the CIC-1 channel and found that it regulates the excitability of active muscle tissue. What if patients with neuromuscular diseases could benefit from treatment targeted at this ion channel?

"Already from the beginning our mission was clear," says Martin Gruwier Broch-Lips. "We are dedicated to developing novel treatments to improve muscle function and quality of life of patients with neuromuscular diseases". We are a research-based company and the idea for our first treatment was generated through basic research," explains Martin Gruwier Broch-Lips. "We have progressed and have now completed phase IIa studies in myasthenia gravis patients, so this clinical proof of mechanism is transformative for us as a company."

# QPatch's high throughput capabilities

NMD Pharma's in-house molecular pharmacology and screening team conducts experiments by testing molecules on specific targets and describing them in their screening cascades using Sophion automated patch clamp technology. "We have been working with the QPatch 16 since early on in our journey," says Senior Scientist Anders Riisager. "We started by setting up a screening cascade to identify active molecules on our targets. Initially, we wanted to utilize the throughput of the QPatch as the initial part of our screening funnel."



# From QPatch to QPatch Compact

After testing many compounds, NMD Pharma's research quickly developed into using the QPatch for counter-screening against other targets. "This meant we could test compounds on the human isoform of our targets instead of testing solely on rodent muscle tissue," explains Anders Riisager. "This was how we were using the QPatch for years until we recently began working on the QPatch Compact."

#### **Greater control of experiments with QPatch Compact**

NMD Pharma made the switch to the QPatch Compact for several reasons. "Sophion's QPatch sets the standard for automated patch clamp. However, we no longer needed the high throughput of the QPatch, and we were looking for even greater control over our experiments," says Anders Riisager.

"With manual patch clamp, you have full control on every parameter, but the tradeoff is a very low throughput and particularly time-consuming experimentation," explains Anders. "The QPatch Compact is an elegant compromise that gives us some key automation along with the control we need." The team was also interested in some of the newer capabilities from the QPatch II that were also implemented in the QPatch Compact. "It was a great opportunity. Plus, we were always testing new targets, so the QPatch Compact was the perfect fit," says Anders Riisager.

#### Adaptive voltage clamp and innovative software

Adaptive voltage clamping and Sophion's Analyzer software were also key considerations for the team. "The QPatch Compact's dynamic voltage clamp feature was a big selling point for us," says Anders Riisager.



"We wanted to work with individual cells and be able to halt experiments and adjust the protocol if a patch is stable. This flexibility in the workflow is really attractive for us with the QPatch Compact."

Anders Riisagei

"The QPatch Compact user interface and software is very powerful in getting throughput, and it enables you to set up protocols and templates in your analysis and then it quickly gives you the data points you need." Anders Riisager

The team still has the QPatch 16 onsite, and they also use manual patch clamp for asking very specific questions. "The QPatch Compact is excellent for ensuring versatility," says Martin Gruwier Broch-Lips. "One of our priorities is understanding translation – the steps between in vitro, in situ, and in vivo in rodents and then in humans – and that needs a dynamic setting. QPatch Compact enables us to ask better questions."

#### **Enabling efficient CRO collaboration**

It's a top priority for the NMD Pharma team to own their research and control their quality, but they also work with CROs. "Using Sophion's automated patch clamp technology is considered the industry standard," says Anders Riisager. "We collaborate with different CROs, who are also working on Sophion systems, so it's also beneficial for us to have those capabilities in-house. It enables us to transfer custommade protocols or to test and validate CRO work very smoothly and easily."

Being in control of their own experiments helps NMD Pharma fully understand their research. "If we can understand the capabilities of the QPatch platform and survey the quality of the data output through in-house experiments using the QPatch Compact, then we can also expand our research and ask CROs to do larger library screenings for us at the right high quality."

The automated patch clamp technology from Sophion enables researchers to set up protocols for an easier handover for additional screening. "We own the research and the quality. All that is done in-house, so we understand what we are asking from a scientific point of view," says Martin Gruwier Broch-Lips. "We believe that's important when it comes to generating drugs for all diseases, including rare ones."

#### Partnering with Sophion from the very start

From early on, Sophion's automated patch clamp instruments have been an important part of NMD Pharma's research and data package. "The whole platform that Sophion offers can help you understand channel function better, cheaper and faster than if you had to do it another way," says Martin Gruwier Broch-Lips. "You get the whole package with Sophion – it's not just the instruments," adds Anders Riisager.

For all types of research, it's critical to have cell lines in the right condition so that you can optimize experiments and ensure reliable data. "Sophion has enabled us to succeed when it comes to cell lines and also analyzer software and service. Our business moves quickly, and Sophion has helped us through every issue we've run into, always getting us up and running again quickly," says Anders Riisager. "It's a pleasure to work together. They are fast to respond and get people to our sites if needed."

The potential also exists for NMD Pharma to grow with Sophion. "With our existing QPatch we have the possibility for screening more compounds, but the QPatch Compact is enabling us to ask detailed questions," says Martin Gruwier Broch-Lips. "Down the line, there is also the Qube for when you have large libraries of compounds."

"We have always had a great collaboration with Sophion, and all the knowledge we have generated together along the way has been an important part of our journey."

Martin Gruwier Broch-Lips



NMDPHARMA® Neuromuscular disorders

For more information about NMD Pharma and to make contact, click here: <u>nmdpharma.com/</u>

#### What's next for NMD Pharma

Today NMD Pharma is developing its own cell lines internally and the company has an ambition to expand its pipeline within neuromuscular diseases. "Electrophysiology will remain one of our core competencies and the QPatch Compact is a part of that," says Martin Gruwier Broch-Lips. "We will continue our research in pharmacology and understanding disease mechanisms. I imagine that in five years time, we will still be using the QPatch Compact, and we will have more targets in our pipeline. Our mission continues to be developing novel treatments to improve the function of patients with neuromuscular disease."

"I'm sure I speak for all of us in NMD Pharma when I say that we want to be a company that is helping neuromuscular patients' long term," says Martin Gruwier Broch-Lips. "We are building this company for the future, so want to keep producing great results and new medications for people with rare neuromuscular diseases."

Sophion is keen to work with startups like NMD Pharma and to help grow possibilities within scientific research. Collaboration is a win-win, giving Sophion a deeper understanding of processes and enabling further instrument optimization.



Sophion Bioscience is a leading global life science company founded in 2000 by a group of passionate electrophysiologists. We specialize in developing and manufacturing automated patch clamping and cell line solutions. With our complete technical, biological, and application support, we help our partners pioneer ion channel research and drug discovery. Through the continued development of our QPatch Compact, QPatch, and Qube 384 platforms, we offer uncompromised data quality in a user-friendly environment, from assay setup to advanced data analysis. We are headquartered in Denmark and have subsidiaries in the United States, Japan and China. For more information, visit **Sophion.com** 

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