ABLIVA Delivering mitochondrial health

Newsletter

September 2022

"We have recharged our batteries for the busy period ahead".

Exciting times ahead

Hi! I hope you all had a lovely summer and early autumn. We have recharged our batteries for the busy period ahead, with the preparations for the start of the FALCON Phase 2/3 study in patients with primary mitochondrial diseases treated with KL1333.

The annual World Mitochondrial Disease Week ran from 18 to 24 September with the aim of spreading knowledge among healthcare professionals, policymakers, industry, and the general public about mitochondrial diseases. We participated this year by releasing videos on our website with a focus on mitochondrial disease patients and on our portfolio (see all videos here: https://abliva.com/sv/poster/presentationer/world-mitochondrial-disease-week-2022/). In this newsletter, we will hear more about the FALCON study from our Clinical Project Manager, Fia Ence. We will also hear directly from a patient, Daniela Gallo Castro, who suffers from Kearns-Sayres syndrome.

Abliva has participated in many clinical, scientific and investor meetings aimed at raising awareness for the research area and our efforts in developing new therapies for mitochondrial disease patients. The autumn will offer additional opportunities to spread our message. We look forward to seeing you at future events!

Best wishes,

Ellen Donnelly
CEO

"We will be evaluating the effects of KL1333 on the patients' fatigue and muscle weakness in hope to improve these symptoms".

Interview

Abliva's Clinical Project Manager, Fia Ence

This year, Abliva is looking forward to the start of the Phase 2/3 study with KL1333, the FALCON study. The study will include adult patients with systemic primary mitochondrial diseases suffering from fatigue and myopathy. Here, our clinical project manager, Fia Ence, will tell you more about this important study and what it will feel like to patients who participate in that study.

You are about to start a clinical study to evaluate the efficacy of KL1333. Can you tell us a bit more about the study?
"We will be enrolling adult patients suffering from fatigue and muscle weakness, amongst other symptoms, and who have a genetically confirmed mitochondrial DNA mutation. We will be evaluating the effects of KL1333 on the patients' fatigue and muscle weakness in hope to improve these symptoms. Patients enrolling into the study will take one pill of KL1333 or placebo twice daily for approximately one year. The study is double-blind, meaning that neither we, nor the physician, nor the patient will know whether they are receiving placebo or the active study drug. This is important to be able to evaluate the actual effects of KL1333 without any bias. The study will run in the US and in a handful of European countries."

The patients in the study will, among other symptoms, suffer from fatigue and muscle weakness, making it a bit difficult for them to participate in a clinical trial. How will you facilitate their participation in the study?
"We have reduced the number of visits to the site and made sure that most assessments can also be done in the patient's home, meaning that we will offer home nurses to come to the patient's home for three of the eight visits. In total there will be five visits to the site during the 64 weeks that the study is ongoing. We have also developed an app where patients can respond to the questionnaires, which they will do prior to their visits to lessen the burden on the day at the site. In addition to that, we work with a travel agency that will be available to provide travel to and from the site."

Before you can start recruiting patients, what additional activities need to be performed?
"Currently, we are working on rather detailed preparations to ensure that we will capture high-quality data during the study. We are submitting applications to the ethics committees, finalizing discussions with regulatory authorities, we are setting up data systems for data capture, and we are training site staff and home nurses in the protocol and the different assessments. Everything to make sure that, once recruitment starts, everything will run smoothly."

FALCON

"He (the doctor) asked me for how long I had not had any eye movement. I had absolutely no idea what he was talking about".

Interview

Daniela suffers from a primary mitochondrial disease

The patients sit at the core of everything we do at Abliva. We meet with them frequently to understand their disease and their symptoms, so that we can better design our therapeutics. Meet Daniela Gallo Castro, PhD, clinical trial coordinator, and mitochondrial disease ("mito") patient.

Can you briefly introduce yourself and tell us a little bit about your connection to primary mitochondrial disease?
"My name is Daniela, and I am 36 years old. I live in Switzerland, but I'm Uruguayan and Italian - I have lived in both countries. I have a mitochondrial disease that is called Kearns-Sayres syndrome."

When did you learn that you that you have a mitochondrial disease, and how was it discovered?
"I learned about my mitochondrial disease when I was 31 years old, so almost five years ago. It was just by accident because I had some eye issues, like an infection, and I went to the ophthalmological hospital that we have here. At the end of the visit, the doctor asked me to do the test where you follow the doctor's finger with your eyes. And I could tell there was something wrong because he kept on asking me to look at his finger. And I thought: "I AM looking at the finger." He told me that I had no eye movement and asked me for how long I had not had any eye movement. I had absolutely no idea what he was talking about.

My disease was diagnosed at 31, but I started having symptoms when I was 12 years old. I only had one droopy eyelid, and no one had an answer for that. After he saw that I did not have any eye movement, he sent me to another ophthalmologist that was much more of an expert in rare conditions. She was amazing because immediately after just checking my eyes and after some more controls, she said "I really believe you have a mitochondrial disease. And we will need to do other checks for that". So, I went to a neurologist, and I saw another ophthalmologist at the hospital as well. I also saw the mitochondrial disease expert and a geneticist. And finally, they did a muscle biopsy that showed the ragged-red fibers, which is a clear indication of mitochondrial disease. Then they did the DNA testing and it showed that I have a deletion in my mitochondrial DNA. At that point it was confirmed."

How is the disease affecting your everyday life, and what disease expressions are most debilitating for you?
"I live with chronic fatigue, and it is something very present in my life. I almost need one nap daily just to function. In my case, besides the chronic fatigue, is a lot of eye symptoms, general muscular weakness, especially in my legs, in my arms, and my neck. But I have learned to sense my energies and work accordingly."

How has the support from the healthcare been for you?
"Now, I have a bunch of specialists, and they are constantly talking to each other and addressing each other. Even for issues that are not related to mito, they will go and ask my mito specialist about them first to see if this treatment or that treatment can work or not. So, I feel very blessed in that sense because the health care system is really helping me a lot."

World Mitochondrial Disease Week 2022, Sep 18 – 24

World Mitochondrial Disease Week is an initiative from the patient organization International Mito Patients (IMP) to raise awareness of mitochondrial disease (mito) on a global scale through educational, fundraising and advocacy activities. Abliva participated by releasing videos focused on increasing the understanding of the community to rare disease development and to the development of new therapies for primary mitochondrial diseases. Click below to watch.

Day 1: Introducing Mitochondrial "Mito" Disease

Day 2: Designing Therapies for Mito Disease, and Abliva's NV354

Day 3: Living with Mitochondrial Disease

Day 4: Drug Development in Mito Disease, and Abliva's KL1333

Day 5: Abliva's upcoming Phase 2/3 study with KL1333

Day 6: A Patient-Centric Approach to Drug Development

Day 7: Mitochondrial Disease in Children - Meet Adrian, father of Rebecca

Learn more: https://abliva.com/posts/events-and-presentations/world-mitochondrial-disease-week-2022/

Spreading the word

Communicating our mission, our strategy and our data to the external community (patients, physicians, researchers, investors) is critical as we work to build the premier company in mitochondrial medicine. Primary mitochondrial disease is an area unknown to many, so we aim to educate and inform as we work to develop therapeutics to treat these patients. Our recent events have included:

Bioblast 2022, Innsbruck, Austria
June 29 - 30, 2022. Presentation available here:
https://youtu.be/2--Bt1T7Zu0

Aktiespararna Digitalt, Life Science
August 31, 2022. Presentation available here:
https://youtu.be/iHgYjMfQoK8

mitoNICE, Nice, France
September 15 - 17, 2022.

Aktiespararna's Aktiedagen Lund, Sweden
September 26, 2022. Presentation available here:
https://youtu.be/ajZ9_40hK-s

Nordic Life Science Days 2022, Malmö, Sweden
September 28 - 29, 2022.

Upcoming events

8 - 9 Oct
Mitocon - Italian Meeting on Mitochondrial Diseases
Rome, Italy

14 Nov
LSX Inv€$tival Showcase
London, UK

21 - 23 Nov
Mitochondrial Medicine
Cambridge, UK

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