ABLIVA Delivering mitochondrial health

Newsletter

December 2022

"The FALCON study will evaluate the effects of KL1333 in patients who have primary mitochondrial disease and suffer from chronic fatigue and muscle weakness".

The FALCON has taken flight

After an extensive start-up period, we were thrilled, on December 5, to announce the start of our Phase 2 study, the FALCON study, with our lead candidate KL1333. This study will evaluate the effects of KL1333 in patients who have primary mitochondrial disease and suffer from chronic fatigue and muscle weakness. There are currently no approved medicines for these patients, so we are hopeful that patients will enroll in our study and help us determine whether KL1333 gives them more energy and helps their muscles work better.

We saw our first evidence of these effects in a Phase 1b study in patients last year, where patients treated with KL1333 had improvements that were not seen in placebo-treated patients. We were also pleased, in that study, to observe that patients that had more drug in their body did better on the clinical assessments.

Taken together, our data suggests that KL1333 is having an effect on patients, and we are looking forward to testing this in a larger population of patients during the FALCON study.

In this edition of the newsletter, our Chief Medical Officer, Magnus Hansson, and our Clinical Project Manager, Fia Ence, will tell you more about KL1333 and our FALCON study.

While focused on study start up, we also prioritized meetings with patients, physicians, researchers, and study sites, traveling to the UK, France, Italy, and Denmark in the past few months. The last two weeks took members of the team to Cambridge, UK, for the Mitochondrial Medicine – Therapeutic Development meeting (where we presented a poster) and then I was invited to present to Scandinavian biotechs and investors at the Nordic American Life Science Conference in New York last week.

As I reflect on this year, I am thankful for the bright and driven team at Abliva, a promising lead molecule and a second program full of potential, our successful raise in the midst of a difficult market, and all of you, who accompany us on this important journey. We are developing medicines for patients who suffer from the devastating and debilitating primary mitochondrial diseases. They have nothing. They need our help!

Wishing you all a festive holiday and a Happy New Year,

Ellen Donnelly
CEO

Interview

Magnus and Fia on the FALCON study start

In early December, it was announced that the company's FALCON study with KL1333, in patients with mitochondrial diseases, had been initiated. After extensive preparations including applications to regulatory authorities in different countries, completion of the study protocol, negotiations with the study sites and training of the study staff, this long-awaited, potentially registrational, Phase 2 study has been launched. At the center of this work are Abliva's Chief Medical Officer, Magnus Hansson, and Fia Ence, the company's Clinical Project Manager.

Hi there! First of all, congratulations on the start of the study. What factors had to come into place to accomplish this?

"The study has now been approved by several regulatory authorities and ethics committees in Europe and the US. In addition, the extensive logistics required are in place for, among other things, tablet management, opportunities for remote virtual visits for participating patients, and study-specific apps where the patients themselves fill in several of the study's efficacy measures at home so that we can evaluate effects on the symptoms of the disease in daily life."

Can you briefly tell us about the study design? What is required to participate in the study?

"The study is aimed at patients with mitochondrial disease who suffer from chronic fatigue and muscle weakness. In the study, we are investigating the effects of KL1333 on these particular disease expressions with the hope of being able to show that patients feel less fatigue, that it has less of an impact on their daily life, and that their muscles work better. To participate in the study, patients will first undergo a first visit to the clinical study site and then go through a screening period of 8-12 weeks, where we ensure that they meet the criteria required to participate in the study. This is followed by a treatment period of 48 weeks, where patients are randomized to either receive tablets with KL1333 or placebo (tablets with identical appearance but without active compound). The study ends with a five-week follow-up period. During the course of the study, no one (doctors, patients, or colleauges at Abliva) will know whether patients are receiving KL1333 or placebo."

When do you think the first patient will be dosed?
“As we recently announced, we have activated the first clinical sites, and more sites will be activated at the beginning of next year. We have a screening period of 8 to 12 weeks, which means that a couple of months will pass between the first screened patient and the first dosed patient. We expect the first patient to be dosed in the first quarter of next year.”

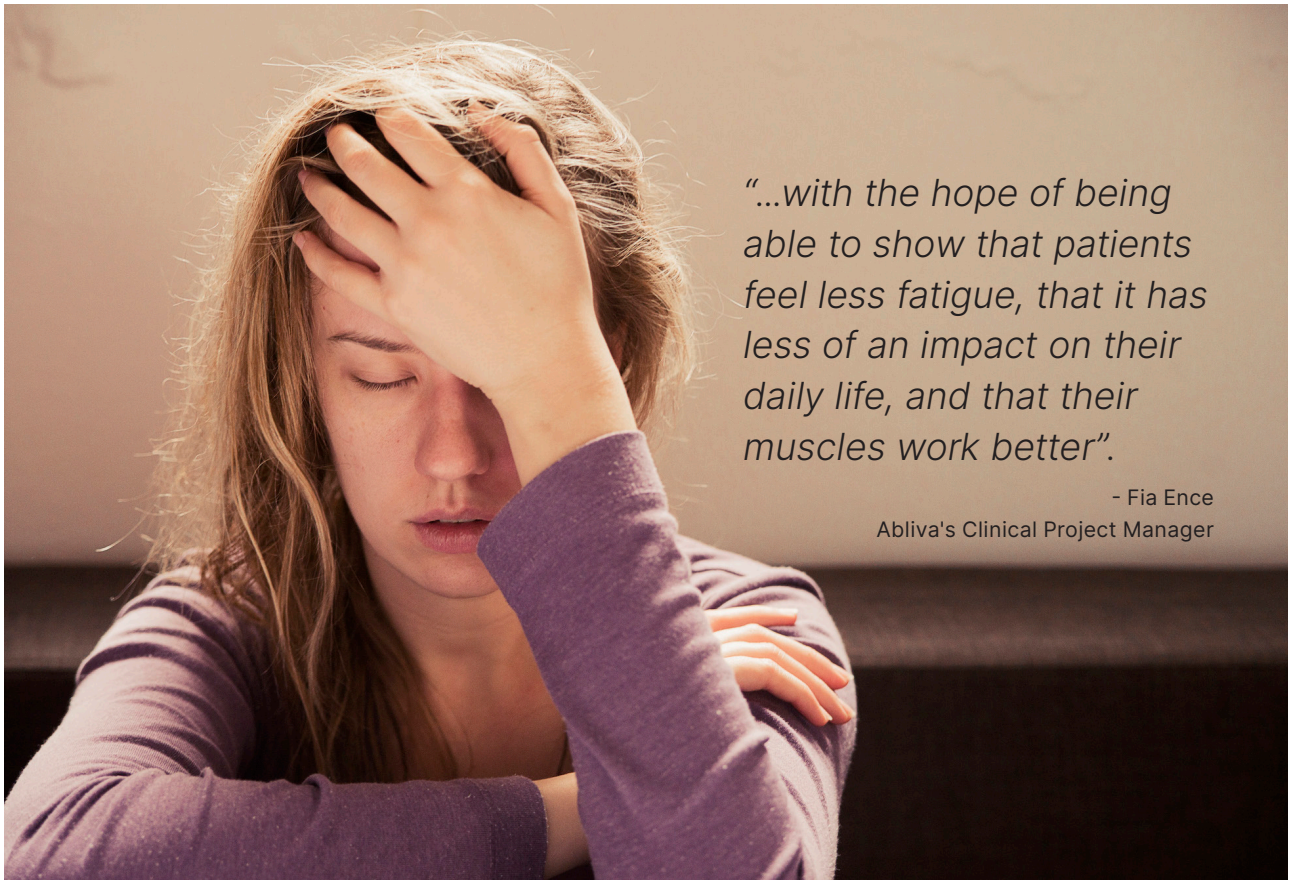
In which countries are the study sites currently active located, and why?
"The sites that have now been initiated are located in Denmark and the UK. It was in these two countries that we first received complete approval from all authorities and ethics committees. We are progressing the discussions in the other countries as well and expect to have most sites activated during January-February."

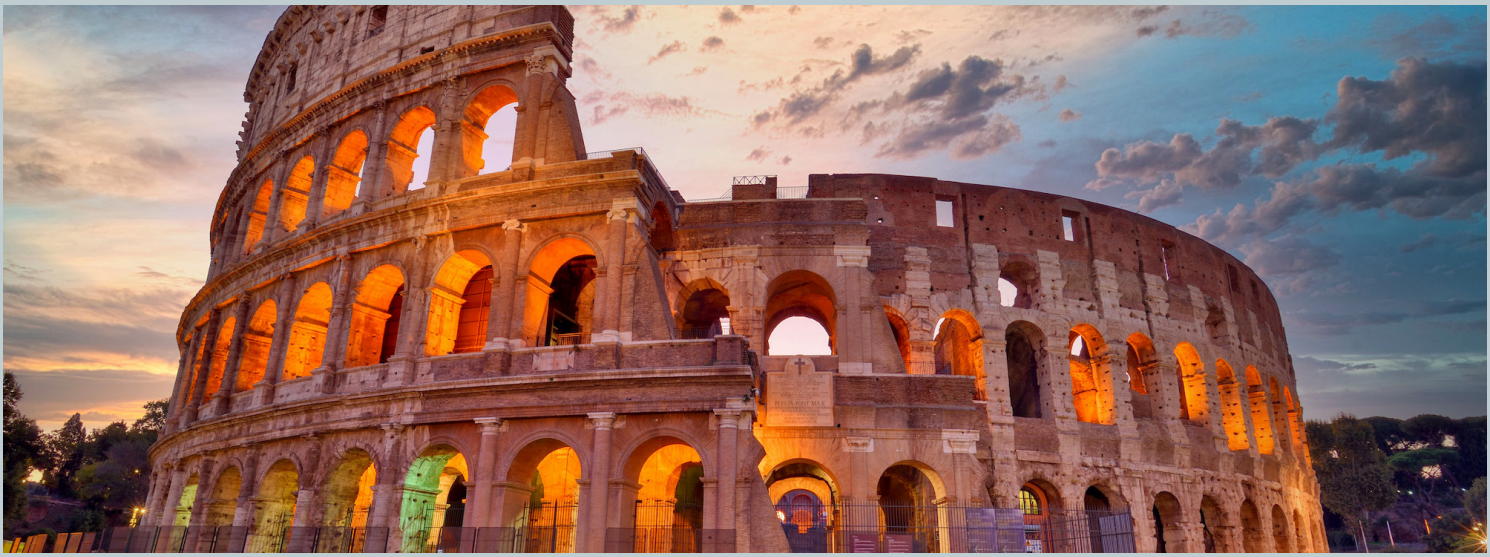
Normally, pharmaceutical companies do both Phase 2 and 3 studies before applying for market approval and registration. Why is it that the FALCON study in particular may be registrational?
"For rare diseases such as mitochondrial diseases, there is a limited number of patients, so it is not possible to do studies on thousands of patients as in the development of, for example, blood pressure medications. When we have discussed the development plan with authorities, they recommended that we design the study in a way that allows it to support registration if it shows positive results. This made the preparation phase more extensive as there were important activities that had to take place to allow for registration, including the validation of the first specific outcome measure of efficacy for chronic fatigue in mitochondrial disease. This is also one of the reasons for the long treatment time of 48 weeks."

What is next in the study, and what is the next big milestone?
"Now that we have started the study we will look towards the interim analysis which we expect in late 2023 or early 2024. Approximately half of the study sites that we have selected for the full study will be activated in the first stage. These sites will recruit the first 40 patients required for the interim analysis. When all 40 patients have been treated for at least 6 months, there will be a calculation of how large the study needs to be to be able to show statistically reliable results for both of our primary endpoints (although it is enough that either of them is positive in the end for the study to be successful). After that, we will activate the remaining study sites to recruit a total of 120-180 patients, and thus complete the study."

Information about the FALCON study is now available at ClinicalTrials.gov.

ClinicalTrials.gov Identifier: NCT05650229.





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:

Mitocon - Italian Meeting on Mitochondrial Diseases
Rome, Italy
October 8 - 9, 2022.

Mitochondrial Medicine - Therapeutic Development
Cambridge, UK
November 21 - 23, 2022.

Aktiespararna Västra Blekinge/Bromölla
Olofström, Sweden
November 8, 2022.

Aktiespararna Sydöstra Skåne
Tommarp, Sweden
November 17, 2022.

Aktiespararna's Stora Aktiedagen Stockholm
November 28 - 30, 2022.
View the presentation: https://youtu.be/XDOgwgNDmRU

Nordic American Life Science Conference
New York, US
December 7 -8, 2022.