ABLIVA Delivering mitochondrial health

Newsletter

October 2021

Two clinical assets next year

We are excited this month as NV354, a compound that came from our research group, has received positive feedback from the UK regulators and will move into the clinic next year, joining KL1333 in our clinical pipeline. With the advancement of these two programs, we are pleased to welcome two clinical experts to our team, Dr. Sam Lindgren and Dr. Joy Chukwujindu.

Preparations for our upcoming Phase 2/3 study with KL1333 in mito patients continue to plan; I refer you to Fia's interview for more details.

During World Mitochondrial Disease Week, we worked to increase awareness towards mito diseases. We highlighted a number of our programs in our daily videos so I encourage you to check them out at abliva.com.

In addition, this week I had the opportunity to meet many of you in person during an intense four days in Stockholm and Lund. I truly enjoyed our discussions and thank you for your candid questions and feedback. We will hold more events in the future so please watch out for those announcements.

Join us on an exciting journey ahead!

Ellen Donnelly
CEO

"...I get to be part of a fantastic and creative team, focused on developing new medicines to improve the life of patients with mito disease."

Interview

Fia Ence on KL1333's path forward

Abliva's drug candidate KL1333 has advanced rapidly over the past six months. Preparatory activities for the upcoming registrational Phase 2/3 study in patients with mitochondrial diseases are in full swing. Abliva's Clinical Project Manager, Fia Ence, is on top of the situation.

Hello Fia! You are part of the clinical development team at Abliva. What is your specific role in that team?
"Within the clinical development team, I am responsible for clinical operations – this means that I work closely with the Abliva team to design and operationalize the clinical study that we will run with KL1333 in primary mitochondrial disease patients. I am also the liaison for the contract research organization that helps us run the study, called a CRO. I really enjoy my job because I get to be part of a fantastic and creative team, focused on developing new medicines to improve the life of patients with mito disease".

Abliva's lead candidate, KL1333, has shown favorable pharmaceutical properties in several prior studies, and recently demonstrated a clinical effect in a cohort of patients. How will you use these results going forward?
"These studies are all critically important as they de-risk our program as we finalize the design of our upcoming study. First, we've now dosed over 110 patients and healthy volunteers with our compound, so we have a good understanding of the safety profile. The recent data from our PMD patient study helped confirm the appropriate patients for the study and gave us important information about the appropriate dose in the upcoming study. Finally, the strong data on both the fatigue endpoints as well as the functional endpoint provided justification for a second primary endpoint in the study. This is really great news as we now have two 'shots on goal' for a successful study".

What preparatory activities for the upcoming Phase 2/3 study are ongoing and how far along are you?
"As you can probably imagine, starting up a global, registrational study is a great deal of work and requires sequential activities over many months. First, we selected a CRO earlier this year. It is important to us to partner with a CRO that understands the rare disease space and can be all quality standards. We are now working with the CRO to operationalize the study and set-up all the logistical aspects. We also continue our discussions with regulators to ensure we run a well-designed study – a recent example was our End of Phase 1 (EOP1) meeting with the FDA earlier this summer".

Looking at the next few months, what will you and the team be focusing on in the KL1333 program?
"We are in the process of selecting the sites that will participate in the study. Once we have selected our sites, we will start working on site initiation and applying for regulatory approval in each country. In parallel to this we will start preparing our vendors so that they are ready to go. For instance, we will pack and distribute the drug product, set-up a study database, prepare all training material and many other things that will all come together to allow for the start of inclusion of patients next year"

"...this treatment has the potential to modify the course of the disease and make a difference in many patients' lives. "

Interview

Alvar Grönberg sees great potential in NV354

NV354, a compound originating from research at Abliva, recently received positive feedback from the UK regulators. This means it will move into the clinic next year, joining KL1333 in the company's clinical pipeline. Alvar Grönberg, Abliva's Director Preclinical Development, sees great potential in the project.

Hello Alvar! You have an impressive background in drug development, with a focus on non-clinical work. What are your main responsibilities at Abliva?
"Hi! At Abliva, I oversee the planning of, and the delivery of the preclinical experiments that both support our late-stage assets as well as help us design new drug candidates for the portfolio. This is important because it is the preclinical data package that demonstrates to regulators that our drugs have the necessary profile (are safe and have the potential to be efficacious) to be trialed in healthy volunteers and then in patients. I find the preclinical space very invigorating because it is scientifically and intellectually stimulating and exciting. The studies that we need to perform cover a very broad area of disciplines, from chemistry to advanced biology, and give rise to completely novel data".

Just a few weeks ago Abliva released the news that a second program, NV354, will move to clinical development. What was the trigger point for this decision?
"After completion of the preclinical program we requested a meeting with the UK regulatory authority, the MHRA, to discuss key aspects of the program. Our discussion went well and thus NV354 will move forward into the clinic in 2022".

What additional activities are needed before you can start a clinical Phase 1 study, your first study in humans, with this program?
"While we have completed the core set of activities required to support clinical development, we will continue to build and strengthen the preclinical package over time. Right now our focus, however, is manufacture of clinical trial material and assembly of the clinical trial application for submission to the regulators. This document, once approved, will allow us to dose our first healthy volunteer with NV354".

NV354 comes from research conducted at Abliva. If the drug candidate reaches the market, how will this hopefully change the lives of patients with mitochondrial diseases?
"NV354 is designed to compensate for a specific type of mitochondrial dysfunction that is common in the primary target group of patients. NV354 has properties that will allow convenient oral dosing and efficient delivery of the active moiety to all organs in the body including the affected nervous system. This means that this treatment has the potential to modify the course of the disease and make a difference in many patients' lives.

We are also excited about possibilities to expand the use of NV354 into other uses within mitochondrial medicine and other indications.

The mode of action and properties of NV354 are complementary to those of KL1333, which is our second drug candidate already in clinical development. Together, these drugs have the potential to bring treatment options to patients who currently have nothing.".

World Mitochondrial Disease Week 2021

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. This year, 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 Mito 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: Developing Drugs in Rare Diseases – Orphan Drug Designation

Day 7: Fatigue in Mito Disease: Understanding The Patient Experience

Learn more: https://abliva.com/poster/presentationer/world-mitochondrial-disease-week-2021/

New collaboration

Strengthening the clinical development team

The team at Abliva is fortunate to include a number of drug development experts who support the portfolio in specialized areas such as regulatory affairs, statistics, manufacturing, safety, and a host of other areas. The company has recently strengthened its team with the addition of two clinical experts, Dr. Sam Lindgren and Dr. Joy Chukwujindu.

Joy Chukwujindu is a fully registered UK General Medical Council (GMC) physician with specialist registration in pharmaceutical medicine, and also holds a Master of Business Administration (MBA). She has worked at several large and medium pharmaceurical companies and Contract Research Organizations (CROs) for more than 25 years and has extensive experience in all aspects of global pharmacovigilance across all phases of drug development and post-marketing. Joy is also skilled in real time safety monitoring of clinical trial data. Joy will support the safety management of the KL1333 phase 2/3 study.

Sam Lindgren is a MD, PhD, with a Master of Business Administration (MBA), who trained as a cardiologist at Lund University Hospital. He brings more than 25 years of big pharma experience of clinical drug development, translational medicine, pharmacovigilance, strategy development and senior leadership roles from AstraZeneca, Novartis, Lundbeck and Novo Nordisk. Sam will support the clinical development of both the NV354 and KL1333 programs.

Fireside chat in Stockholm and Lund

At the end of September, Abliva arranged two informal meetings, ”fireside chats”, with the company’s CEO Ellen Donnelly. Ellen, who is American and lives in Boston, was able to meet colleagues, shareholders and others interested in the company for the first time after taking over as CEO.

Upcoming events

7 - 8 Oct

Biotech in Europe Forum

20 - 21 Oct

BioStock Life Science Summit

See previous presentations here: https://abliva.com/posts/events-and-presentations/