NeuroVive PHARMACEUTICAL

Newsletter

June 2018

Summer greetings from NeuroVive

Summer is here, and NeuroVive will soon be ending yet another exciting and eventful six months. Some days ago, we finalized an exciting outlicencing deal with a potential value of USD 60 million including possible royalty payments. We have also reported positive findings from our research, strengthened our existing partnerships and formed new collaborations with world-class researchers, and completed a highly successful issue of new shares. Let me use this opportunity to thank our owners, old as well as new, for the trust you have shown us!

Erik Kinnman
CEO

NeuroVive is focused on a broad range of therapeutic areas – from traumatic brain injury and mitochondrial diseases to fatty liver and liver cancer. The common denominator is, of course, the mitochondria – the powerhouse of the cell. The positive trend in all of our areas is firm confirmation of the company's unique expertise in mitochondrial medicine – an area that is attracting increased attention from the pharmaceutical industry.

The agreement with BridgeBio is important to both NeuroVive and our innovative NVP015 program. The agreement validates the scientific quality of the NVP015 program, at the same time as it is a commercial confirmation that our business development model works as it is intended. We will work closely with BridgeBio to further develop this chemistry subset and make the LHON program successful. The agreement with BridgeBio is a positive expansion of the NVP015 program. It is, however, important to note that our intentions for the NVP015 program are unchanged. The next step in the pre-clinical development is an experimental proof-of-principle that is planned to be conducted during the second half of 2018.

In April, NeuroVive conducted a rights issue with the intention of raising capital for our forthcoming clinical trials. Despite the relatively weak interest in new share issues recently, our issue was oversubscribed – a show of strength for both our research and our business model. The proceeds now generated will mainly be used to finance development in our currently perhaps most exciting project: KL1333.

NeuroVive's KL1333 was granted orphan drug designation in the EU towards the end of last year, and in early 2018 in the US. In addition, our partner Yungjin Pharm's initial Phase I trial with KL1333 in South Korea was completed, with positive pharmacokinetic and safety results. This will enable NeuroVive to quickly proceed with planning for the next trial in the KL1333 project, and with preparations for our upcoming Phase I MAD (multiple ascending dose) trial with KL1333 on healthy volunteers and patients with mitochondrial genetic disorders. NeuroVive's selected CRO has begun working and the trial is expected to commence in the second half of 2018. This trial has attracted strong interest from researchers, patient organizations and treating physicians.

In other words, we are looking forward to an equally exciting second half of 2018.

NeuroVive would like to wish you a happy summer!

CEO, Erik Kinnman
Lund, June 21, 2018

NeuroVive Pharmaceutical AB (publ)
Medicon Village • SE 223 81 Lund, Sweden

info@neurovive.com
+46 46 275 62 20

Breakthrough agreement confirms NeuroVive's commercial potential

NeuroVive has signed a landmark licensing agreement with the US firm BridgeBio Pharma. The agreement pertains to the development of drugs for the treatment of the mitochondrial eye disorder LHON. The agreement validates the scientific quality of the NVP015 program and, in commercial terms, confirms the practical functionality of NeuroVive's business model. The potential future revenue for NeuroVive amounts to USD 60 million.

"The agreement is a major recognition of our advanced research," states Erik Kinnman. "This success will have a ripple effect and I am convinced that other areas of the NVP015 project and our other research programs will now be viewed in a new light. Opportunities to license other parts of the NVP015 project have been boosted and we have initiated discussions with a number of interested parties regarding the parts of NVP015 for treatment of other mitochondrial disorders."

USD 20 million in initial research resources

BridgeBio intends to make an initial investment of USD 20 million in the project to prepare a drug candidate for LHON (Leber's hereditary optic neuropathy). The project will be run by BridgeBio's subsidiary, Fortify Therapeutics. NeuroVive will collaborate closely with Fortify to ensure the project is initiated as quickly and as successfully as possible.

Potential value of USD 60 million

The potential value of the agreement amounts to USD 60 million. The initial payments are limited, but revenue will increase as the project advances and be actuated in the form of milestone payments and royalties on any future sales.

Highly reputed partner

"BridgeBio represents a highly reputed partner with extensive experience of similar contracts. The agreement will generate future revenue and confirms NeuroVive's commercial potential as well as our capability to create value for our shareholders, and expands the opportunities within the NVP015 program," says Erik Kinnman.

Hereditary disorder with no curative treatment

LHON is a hereditary disorder limited to the retina. Generally, the disorder debuts in men aged 20–40 with a decrease in visual acuity that progressively leads to blindness — first in one eye and then in the other. The prevalence in Europe is estimated at between 1:30,000 and 1:50,000. There is only one treatment with limited effect — *Raxone*, which was developed by Santhera Pharmaceuticals. This treatment has only received approval in the EU, and is reimbursed in 8 countries. Raxone achieved sales of SEK 200 million 2017.

"The lack of any curative treatments for LHON has been a critical driver for NeuroVive. This is exactly what our expertise within mitochondrial medicine and frontline research aims at, namely: to develop drugs for mitochondrial disorders that currently lack effective and adequate treatments," concludes Erik Kinnman.

Denise Goode, new member of the Board

Denise Goode is NeuroVive's newest Director of the Board, joining Chair David Laskow-Pooley and Directors David Bejker and Jan Törnell. Denise has extensive experience in Life Science, including serving 20 years with AstraZeneca in senior finance and business development roles.

Denise Goode

Denise, welcome to NeuroVive! Could you tell us a little about yourself?

... Well, I am European, I am a company director and an advisor to a number of Biotechs. I am married to a Dutch immunologist and I have adult sons. I have worked globally through most of my career, initially in finance at PwC (PriceWaterhouseCoopers) and then in a variety of roles in AstraZeneca. I now provide strategic advice. I am passionate about supporting the development of people through mentoring : at an individual level to be their 'best selves', and as team members playing their part in delivering the success of high performing teams. This includes "STEM" ambassadorship, encouraging young people, in particular young women, to consider Science, Technology, Engineering and Maths as interesting and important career choices.

What piqued your interest in NeuroVive?

... It has been a pleasure working through my career in Life Sciences with Swedish academics, scientists and business leaders. I enjoy the way business is done and the excellent level of communication and decision making within Swedish companies.

My interest in NeuroVive is a combination of the focus on unmet need for patients in the areas of research and development the company is pursuing, together with the quality of the people in the company – both scientists and the management team - that I met. I saw strong ability, high commitment and teamwork in working to deliver NeuroVive's strategy.

You have extensive experience in Life Science, particularly in business development. How will you put this to use as Director of the Board at NeuroVive?

... I will leave Erik and his team to deliver on the NeuroVive strategy which, as part of generating shareholder value, includes active management of the pipeline. They are on the front-line addressing business development initiatives, talking to many companies about collaboration, and driving the business development agenda. However, yes, I have experience in this area – and the

bruises to show for it – I intend to support the team by contributing ideas and access to my network and by challenging the team in areas where I believe there could be additional value to be gained.

Looking at NeuroVive's different projects, is there a certain area you find particularly interesting or exciting?

... As with all pharmaceutical companies, each of NeuroVive's compounds and projects has its own merits and challenges. That is where I see NeuroVive has strength – it is not a one product company, instead it has a rich pipeline to drive forward. If I was to pull out just one area then finding solutions which address mitochondrial disease in children is both an intellectual challenge and one I find personally meaningful, with potential for huge patient and societal benefit.

What are some of the challenges and opportunities that you have identified so far, both for the industry in general and NeuroVive in particular?

... For the Industry, where do I start? A few areas that particularly exercise my mind and which could provide opportunity for the right approaches include: Changing demographics, combined with the knowledge of patients and patient advocacy groups and the high expectations that go with that, and the impact on Government budgets for healthcare. Where should bets be placed in a changing World? Will new therapies have to be further rationed? – and if so, how? Will there be fundamental changes, for example, in the need for payment for new drugs? How do we improve patient adherence? The importance of Real World Evidence for supporting the health economics of new therapies is already important for formulary access and reimbursement and, with that in mind, it is essential that new discoveries developed by the industry show appreciable benefit over existing standard of care. As science evolves, I am curious about the impact of artificial intelligence (combined with Big Data) in helping identify further opportunity – possibly in what may already exist, for example, new indications for existing mechanisms of action.

NeuroVive is well placed in its innovative approaches in areas of unmet need. Being able to optimize its activities with the finance the company has means there is a need for precision and focus in delivering data and positive news where novel science doesn't always deliver to plan! This means top class innovative thinking, combined with smart application of those ideas, through the breadth of company activity. It means proactively engaging with shareholders and securing other sources of funding and investment to ensure appropriate resources are available to support the continued drive for success of the company.

You have experience working with Swedish Life Science companies, both in management and as a Director of the Board. What stands out in comparison to Life Science companies in for instance the UK?

... UK Universities and Life Sciences companies have discovered, developed and brought to market great new therapies for the benefit of patients. I spent over 20 years of my career with one of the best UK life science companies and feel privileged to have been part of that process in action.

Is there a contrast with Sweden? – My main observation is for a country with a population the size of Sweden, throughout my time in Pharma I have been astonished by the level of successful innovation that has been delivered and continues to be delivered from the Swedish Life Science Industry. I have not noted too much dissimilarity in approach, and I guess I was fortunate to have enjoyed first-hand the complementarity when a UK company in Zeneca joined the Swedish Astra, combining respective strengths and different ideas and - over the passage of time – that delivered great success.

Off to new adventures

Sarah Piel

In the newsletter from June 2017 we introduced NeuroVive's researcher Sarah Piel who had just presented her research concerning the NVP015 program and paracetamol intoxication at the international conference EUROMIT in Cologne, Germany.

Sarah defended her thesis entitled *Mitochondrial medicine - New strategies to evaluate drug toxicity and develop pharmacological protection of the cell's powerhouse*, on 6 April this year. She will now start a new research position in the lab of NeuroVive's collaborator Assoc. Prof. **Todd Kilbaugh** at The Children's Hospital of Philadelphia (CHOP), USA, where she will continue working with the prodrugs of succinate, developed within NeuroVive's NVP015 program. In our newsletter from October 2017 there was an interview with Dr. Kilbaugh who, together with his team and researchers affiliated with NeuroVive, last fall received a substantial research grant from NIH to evaluate these prodrugs within the CounterACT program. This program aims at finding strategies to mitigate health effects of chemical threats, such as the one in Bhopal, India, in 1984 when thousands of people died after a release of 40 tons of methylisocitrate. Research suggests that this highly toxic compound inhibits complex I of the mitochondrial respiratory chain. NeuroVive's succinate prodrugs strengthen complex II and can thereby bypass such inhibition and restore mitochondrial energy production.

We wish Sarah good luck in her new position and are looking forward to our continued collaboration!

ABOUT NEUROVIVE

NeuroVive Pharmaceutical AB is a leader in mitochondrial medicine, with one project in clinical phase II development for the prevention of moderate to severe traumatic brain injury (NeuroSTAT®) and one project in clinical phase I (KL1333) for genetic mitochondrial diseases. The R&D portfolio consists of several late stage research programs in areas ranging from genetic mitochondrial disorders to cancer and metabolic diseases such as NASH. The company's strategy is to advance drugs for rare diseases through clinical development and into the market. The strategy for projects within larger indications outside the core focus area is out-licensing in the preclinical phase. NeuroVive is listed on Nasdaq Stockholm, Sweden (ticker: NVP). The share is also traded on the OTCQX Best Market in the US (OTC: NEVPF).