Aptahems forskningschef: »Vi ser fram emot att presentera unika resultat under sommaren«

Publicerad 30 juni 2017

Bioteknikföretaget Aptahem har, efter en något turbulent vår, uppdaterat sin kliniska utvecklingsstrategi för läkemedelskandidaten Apta-1 och befinner sig nu enligt uppgift i en intensiv utvecklingsfas. För att försöka få en bättre förståelse kring bolagets nya utvecklingsinriktning och ta reda på mer om vad som sker bakom dörrarna på det Malmöbaserade bolaget kontaktade Biostock Luiza Jedlina, doktor i molekylär parasitologi och immunologi samt Aptahems forskningschef (CSO) för en intervju.
(Intervjun gjordes på engelska)

Luiza Jedlina, could you please share a little about your background and tell BioStock's readers about your role as CSO at Aptahem?

– Certainly! I have a Ph.D. in molecular parasitology and immunology and have worked with cell biology and biochemistry in over 15 years. Science fascinates me and I have the pleasure to work as coordinator in several EU-funded research projects where we are developing new methods for experimental and analytical biochemistry. My responsibility as CSO for Aptahem is to lead our scientific efforts and to coordinate development activities between our in-house expertise and network of associated labs. Like most other biotech companies, we outsource the conduct of our studies to contract research organizations. My role in this, in addition to develop the scientific rationale, is to ensure that we procure the right collaboration partner that complies with regulatory standards and maintains quality that can be used for both data submission and protection of intellectual property.

What specifically attracted you to Aptahem?

– Thanks to my previous academic experience as a parasitologist and immunologist I had the opportunity to observe the complex capabilities for which parasites could module the body's immune system. Building on the Aptahem's platform technology, which is protected under intellectual property rights, we identified an interesting relation between the malarias parasites unique mechanism of survival and modulation of the coagulation cascade and stages of inflammation. This process eventually evolved into the due diligence process that led us to Apta-1 being selected as a therapeutic candidate, a process which I headed. In basic terms, the aptamer blocks the adherence between affected red blood

cells and other cells which can stop events leading to serious diseases and potential death. This process for which parasites can create these "anti-molecules" is incredibly fascinating with many promising advantages compared to traditional targeted therapies such as monoclonal antibodies. In general, the project is incredibly exciting and scientifically attractive.

In contrast to many companies working with targeted candidates, such as monoclonal antibodies, Aptahem is developing a novel concept by applying DNA/RNA molecules, called aptamers. Could you perhaps explain the rationale behind choosing this novel class of molecules?

– Traditionally, monoclonal antibodies have been widely used as therapeutics for different indications due to their abilities to target specific molecules with high affinity and specificity. While great products have been created with monoclonal antibodies, a key disadvantage with these is that they are more prone to create immune responses and give toxic reactions. This means that they do not only bind to the desired target but could also affect other receptors. Aptamers, in contrast, are strands of DNA/RNA molecules with higher selectivity and specificity.

– In addition, aptamers have good thermostability, low immunogenicity, low toxicity and reach their target faster. A historical reason why monoclonal antibodies are used in clinical practice to a higher degree than aptamers, despite the huge potential advantages, was due to a patent situation that obstructed research in this space. That situation is no longer an issue but the situation discouraged some of the early attempts. In addition to the unique properties of Apta- 1, the candidate has a set of favorable characteristic due to belonging to this novel class of molecules that really sets it apart from competing products in the evaluated indications.

– It is also my belief that we will in the future see aptamers replace the role of antibodies in academic, research and clinical applications due to the development of novel high-throughput selection processes that will rapid aptamer selection of varied types. The ease of selection, by syntactical chemical reactions, in stark contrast to the ethical and high cost issues of animal produced-antibodies will drive this paradigm shift.

So, if we get down to brass tax: what kind of work is currently being conducted at the company and what type of results would you say can be expected from Aptahem this year?

– As has been previously communicated, we have recently learnt more about the combined effects of Apta-1. To capture these insights in the best way, my team is currently evaluating Apta-1 in several animal models that develop diseases were coagulation and inflammation are key elements. These conditions are typically fatal and we are considering several endpoints to investigate whether Apta-1 can treat some of these events or improve factors that we believe are indicative for quality of life parameters. Animals and humans are naturally different but share certain characteristics. Much of our current translational attempts are to distinguish if the improvements we see in mice are likely to be seen in the clinic. Our plan is to present the results from our studies as soon as technically feasible, something we are hopeful to accomplish later this summer.

While aptamers are more recognized in the US and Germany by companies such as Noxxon Pharmaceuticals, this type of molecules are more widely used as research candidates in academia. What has been the general response to Aptahem's portfolio of aptamers by fellow scientists and potential partners?

– Most are quite intrigued as Apta-1 offers a new approach through a set of unique mechanism of actions in indications where most conventional drugs have failed to provide sufficient treatment. The unique characteristics of aptamers has attracted commercial collaborations of significant value in the past. For example, it was not too long ago that Ophthotech Corp entered into a $1 billion licensing and commercialization agreement with Novartis Pharmaceuticals for their aptamer-containing drug Fovista. While I'm to some extent is involved in the partnering process with my expertise in the scientific development, I'm concentrating my efforts to make sure that we have a portfolio based on sound science and validated data that enables those types of collaborations.

Speaking of partnerships, Aptahem has a number of ongoing collaborations. Could you elaborate on some of these partners?

– Yes, we are very proud to work with some of the top institutions in our field to support our efforts to investigate the unique properties of apta-1. For example, we are collaborating with a top institution in the form of Nencki Institute of Experimental Biology, where we are evaluating the fascinating biological effects of Apta-1. To make sure that all our efforts are in line with external expectations, I talk to our scientific advisory board regularly, particularly in regard to the new challenges and opportunities to be taken into consideration with the new clinical direction.

– On the regulatory side, we are collaborating with a number of CROs to cover the different activities that are required to enter clinical development. Formulation and stability work is carried out by Swedish and international experts, and toxicology and safety is done by well-known top European CRO's and consultants. Finally, in order for Aptahem to follow the right path in the nest of regulations, we work with individual consultants in each region of interest, which currently is Europe and USA. All our current efforts are done to bring Apta-1 to the next phase and we currently working on the clinical phase 1/2 synopsis with Sourcia, our scientific board and project management for this purpose.

Thank you for speaking to BioStock – do you have final comments regarding the research at Aptahem?

– Well, these are certainly exciting times for the team and I look forward with great excitement to be sharing, as we see it, new and unique results soon.