Q&A with Santhera Pharmaceuticals

1. I understand there was a phase II clinical trial with DMD patients from 8 to 16 years of age. It appears idebenone is safe and tolerable. It looks like the clinical trial for DMD phase III is in the process? I went to www.santhera.com and was trying to locate specific DMD information and timelines.
   In collaboration with Prof. Gunnar Buyse at the University in Leuven (Belgium) Santhera has recently completed this Phase II trial with SNT-MC17/idebenone in DMD patients which showed positive efficacy. Idebenone was well tolerated by participating DMD boys and the good safety profile of the compound was confirmed again. The planning of a Phase III clinical trial program is now underway, but at the current time, we are not yet able to provide the details and firm timelines for the start of these trials. We are currently working on this in close contacts with clinical experts and regulatory authorities.

2. Will the clinical trials for DMD all be outside of the United States? When do you expect Phase III to start and end?
   We plan two Phase III studies, one to be conducted in the US and the other in Europe. As mentioned above it is not yet possible for us to provide timelines or details on the study protocol, as this is still under discussion with clinical experts from the US and Europe. The duration of a clinical trial will depend on the speed with which patients can be included in the trial. In this context we very much appreciate good contacts to patient organizations and physicians as they can greatly assist in trial recruitment.

3. What is the expected time frame for Phase III? Do you expect to start in Europe around September (I am sure enrollment will help determine the start date)? Then after Europe completes, start in the United States? What is the expected time line?
   We would like to start both trials as soon as possible and it is our plan to perform both studies roughly in parallel.

4. If all results go well and once Phase III completes, how long does it usually take to fill and get a drug like Idebenone approved and available to the DMD population? All information on idebenone for DMD sounds very favorable that I have read. I am hoping as you and your company hopes to move forward to getting idebenone on the market to help our families.
   It is difficult to speculate on the time taken for new medicines approval as there are many factors involved, most of them beyond the control of a company. As mentioned above, recruitment speed is one time-critical factor and therefore we are very grateful to all patients and families who can and would like to participate in these trials. Please refer to Santhera’s website (www.santhera.com) for updates on SNT-MC17/idebenone and up-to-date information on our clinical studies (timelines, participating clinical centers). We also have a company policy to register all clinical trials under www.clinicaltrials.gov, where more and up-to-date information can be found.

5. Idebenone if I remember correctly is an already approved drug and as a result the filing should go quickly if all the clinical trials are successful, right?
   In some countries outside of the US, idebenone was previously approved for use in cognitive disorders, and is still available in few selected countries. We are currently developing SNT-MC17/idebenone at a different (higher) dose for use in Friedreich’s Ataxia, Duchenne Muscular Dystrophy and Leber’s Hereditary Optic Neuropathy. For DMD we need to undertake a full clinical program and approval process with the FDA and other regulatory authorities.