



## **Update on the clinical trial of VTS-270: The NPC Study** *August 2016 Newsletter*

### **Update on the Vtesse Clinical Trial of VTS-270:**

August was a very busy month here at Vtesse. We continue to open up new sites and enroll patients. We currently have over 30 patients who have been screened in or dosed. The patients who have screened in are awaiting scheduling for their dose "Day 0" which signifies the first visit in the trial.

There continues to be positive momentum in enrollment, but we still need the help of the NPC community if we are going to meet the enrollment target of 51. Vtesse would like to sincerely thank all of the families who have supported this trial and we ask for your ongoing support.

In July, two families from China have relocated to the United States for the clinical trial. This month, a family from India has relocated to the United States and we are working to assist two families from two other countries who may also be relocating. It is a huge sacrifice to uproot their families for a year to come for the trial, and for that, we sincerely thank them.

### **Clinical Trial Site Expansion Update:**

Our sites in Paris, France and Mainz, Germany are actively recruiting and have screened patients. The sites in Turkey have pushed through the political turmoil in the country to enroll patients in spite of the hardship in the country. Their resilience is remarkable! So we have now enrolled and screened patients at our sites in the US, UK, Spain, France, Germany, and Turkey! Vtesse is also on track to open sites in Australia in the near term.

We would like to thank all of our clinical trial site teams for all the work they have done to get their site active and enrolling patients. We recognize it has been a lot of work and we are sincerely appreciative.

### **Port-Device Update:**

The necessary work to get the port device ready for entry into Part C in the EU is on track. We are in the process of doing the necessary drug-device interaction studies that are needed to support the use of the device with VTS-270. We will keep you posted about the developments related to the implementation of the device.

### **NNPDF Family Meeting:**

Vtesse was invited to speak at the NNPDF family meeting in Boston from August 12<sup>th</sup>-14<sup>th</sup>, 2016. We presented our company values, information on product differentiation, and an update on the 18 month data from the Phase 1 trial at NIH. This was a great opportunity for us not only to present our data, but it was also an opportunity for us to receive feedback from the patient community. There is a lot of excitement in the NPC community regarding potential treatments, and it is our hope that the level of excitement and motivation needed to see the studies through to completion remains high. We are all working toward a common goal of finding answers for the children.

To find out more about the trial and to find a clinical trial site please visit [www.theNPCstudy.com](http://www.theNPCstudy.com) or <https://clinicaltrials.gov/ct2/show/NCT02534844?term=vtesse&rank=1>.