



Update on the clinical trial of VTS-270: The NPC Study

January 2017 Newsletter

Update on the Vtesse Clinical Trial of VTS-270:

Currently, we have enrolled 47 patients into the trial across 20 sites worldwide. We continue to screen and enroll patients. As a reminder, we need 51 patients to be enrolled in the trial. We thank you for your help and support so far, and we ask for your continued help and support in getting this trial fully enrolled.

If you know of anyone who is interested in participating in the trial, please contact Carrie Burke at carrie@vtessepharma.com or refer them to the nearest clinical trial site. A full list of clinical trial sites can be found at www.theNPCstudy.com.

Rare Disease Day 2017 'With research, possibilities are limitless':

To find out ways you can be involved on Rare Disease Day 2017, please visit <http://www.rarediseaseday.org/article/get-involved>.

On 28 February 2017, the tenth edition of Rare Disease Day will see thousands of people from all over the world come together to advocate for more research on rare diseases. Over the last few decades, funds dedicated to rare disease research have increased. But it can't stop there.

Rare Disease Day 2017 is therefore an opportunity to call upon researchers, universities, students, companies, policy makers and clinicians to do more research and to make them aware of the importance of research for the rare disease community.

Rare disease patients and families, patient organizations, politicians, carers, medical professionals, researchers and industry will come together to raise awareness of rare diseases through thousands of events all over the world.

Rare Disease Day 2017 is also an opportunity to recognize the crucial role that patients play in research.

Patient involvement in research has resulted in more research, which is better targeted to the needs of patients. Patients no longer solely reap the benefits of research; they are empowered and valued partners from the beginning to the end of the research process.

- Patients and their families advocate for research on a specific disease or across diseases.
- They know where research is needed and work to influence research bodies and companies to prioritize these areas in their research.
- Individuals or patient organizations often raise money for clinical trials or research projects, on their own or in partnership with private funding initiatives.
- They partner in research projects and are included in the governance of research.
- They participate as subjects in clinical trials and also aid in the design of clinical trials.
- Patients, their families, and patient organizations therefore help to ensure that the development of a medicine takes into account their real needs, so that the patient perspective is not overlooked.

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