



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

Update on the Vtesse Clinical Trial of VTS-270:

Currently, we have enrolled 43 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.

21st Century Cures:

The passage of the 21st Century Cures Act in the United States was due in no small measure to the activities of hundreds of patient advocacy groups and assorted other stakeholders that worked in overdrive the past two years pushing the bill to the finish line. Below are some of the provisions of the new law:

New Funding

The act provides new funding over a 10-year period for initiatives that have the potential to significantly advance our understanding of disease and identify opportunities for new therapies.

- \$500 million total for the Food and Drug Administration (FDA)
- \$4.8 billion total for the National Institutes of Health (NIH):

Policy Reforms

The 21st Century Cures Act addresses the entire biomedical innovation system across discovery, development and delivery of new medical products. Here are examples of policy reforms in this act:

- Strengthens patient centricity in biomedical product development and regulatory approval.
- Bans information blocking between health data systems and provides the Department of Health and Human Services the authority to levy civil penalties against offenders.
- Reforms FDA hiring authorities to fill vacancies by enabling it to compete more effectively with industry to hire and retain the best and brightest experts to review medical product applications.
- Enhances NIH's ability to support innovative research.
- Catalyzes innovation in clinical trials and regulatory approval, without diminishing FDA's authority to determine what constitutes a safe and effective medical product by, for example:
 - Promoting FDA qualification of biomarkers and other drug development tools.
 - Having FDA study how best to use innovative clinical trial designs and real-world evidence generation during product development and regulatory approval.

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>.