

**Updates on Vtesse Clinical Trial of VTS-270 (a unique formulation of 2-hydroxypropyl- $\beta$ -cyclodextrin):** *new sites added, inclusion/exclusion criteria updated, and new information added to [www.theNPCstudy.com](http://www.theNPCstudy.com).*

**New sites:**

We are pleased to announce the opening of University Hospital Muenster in Germany and Great Ormond Street Hospital in the UK. Also, in the US, we have opened Children’s Hospital of Orange County in California and Lehigh Valley Health Network in Pennsylvania. These newly opened sites join Rush University Medical Center in Illinois and NIH in Maryland in recruiting for the trial of VTS-270. We have many more sites in the process of opening (outlined below), and we will keep you updated on their status.

Recruiting Sites	Planned Sites in US	Planned Sites OUS
Rush University Medical Center, Chicago, IL, USA	Tennessee	France
NIH, Bethesda, MD, USA	Florida	Spain
Lehigh Valley Medical Center, PA	New York	Turkey (not yet submitted)
CHOC, Orange County, CA, USA	California (second location)	Australia (not yet submitted)
Muenster, Germany	Utah	Italy (investigating)
London, UK	Minnesota	
	Cincinnati	
	Pennsylvania Massachusetts	

### **Inclusion/Exclusion Criteria:**

After receiving feedback from the entire NPC community, we have made some updates to the entry criteria for the trial of VTS-270. The updates are outlined below:

Inclusion / Exclusion	Rationale
Age range expanded to include 4-5 year olds	No evidence that dosing patients in this range poses greater risk
Seizures	Existing criteria too restrictive. Revise to “adequately controlled”
Anti-psychotic meds for non-psychotic conditions	Patients on anti-psychotics for non-psychotic conditions potential candidates for screening.
Allow G-tube for reasons other than total nutrition	Providing the patient is not getting ALL feedings through G-tube (i.e., meds delivery), they could qualify.
NIH Patient Part C Dosing	Revise to incorporate/address patients who have eclipsed current 900mg dose
Miglustat use	Require three month washout or three months on stable dose before entry

### **New Information Added to [www.theNPC.com](http://www.theNPC.com):**

There have been several updates to the clinical trial website. Additional scientific background information has been added to the site. The information sheets for the trial have been added in German, French, European Spanish, Italian, Turkish, and Spanish. In addition, the new criteria for participation in the trial have been updated and new sites have

been added. Please visit [www.theNPCstudy.com](http://www.theNPCstudy.com) for more information. Below is a snapshot of the website. Also, please feel free to reach out to Carrie Burke, head of advocacy for Vtesse, at [carrie@vtessepharma.com](mailto:carrie@vtessepharma.com) or 301-233-2950 for more information.

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# The NPC Study

Why VTS-270 About The NPC Study Trial Enrollment Study Locations About Clinical Trials For Physicians Contact Us

## Why VTS-270

Information about VTS-270 is available in English, French, German, Italian, Spanish (Spain), Spanish (Latin America) and Turkish.

### VTS-270, THE ONLY INVESTIGATIONAL DRUG

- TO DEMONSTRATE STRONG SURVIVAL BENEFIT IN MULTIPLE ANIMAL MODELS...**
  - ... when treatment is started early
  - ... when treatment is started after the progression of symptoms
- TO DEMONSTRATE PRESERVATION OF NEURONS...**
  - ... in naturally occurring animal models of the disease
- TO HAVE BEEN CLINICALLY TESTED IN NPC PATIENTS...**
  - ... demonstrating emerging positive effects on clinically-relevant function, based on initial analyses from the Phase 1 trial and from compassionate use/iIND studies
- TO BE SAFE AND WELL-TOLERATED IN STUDIES THUS FAR...**
  - ... in NPC patients. High frequency hearing loss reported as a side effect with minimal and manageable impact on quality of life that can be corrected to date with hearing aids
- IN A SINGLE, GLOBAL PIVOTAL TRIAL...**
  - ... with a protocol that is approved by both FDA and EMA.
  - THE TRIAL IS CURRENTLY DOSING PATIENTS.**
  - (Up to 20 sites in the US and EU)*

## AND...

- STUDY DESIGN ENSURES** that the controlled portion of the study is limited to 12 months for any single subject
- COMPREHENSIVE BATTERY OF PRECLINICAL STUDIES** have not only demonstrated safety and positive effects but also predicted dose levels, frequency and route of administration for the pivotal clinical trial
- CLINICAL TRIAL DOSES** VTS-270 will be tested in ranges that are predicted to be safe and efficacious from the animal model studies and Phase 1 clinical trial
- INTRATHECAL ADMINISTRATION** Essential for positive effects in the brain with strong dose dependent response seen for this route of administration *(device under development)*

### VTS-270 Information Sheets

- English PDF
- French PDF
- German PDF
- Italian PDF
- Spanish (Spain) PDF
- Spanish (Latin America) PDF
- Turkish PDF

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# The NPC Study