Updates on Vtesse Clinical Trial of VTS-270 (a unique formulation of 2-hydroxypropyl-β-cyclodextrin): *new sites added, inclusion/exclusion criteria updated, and new information added to 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:

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 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 or 301-233-2950 for more information.