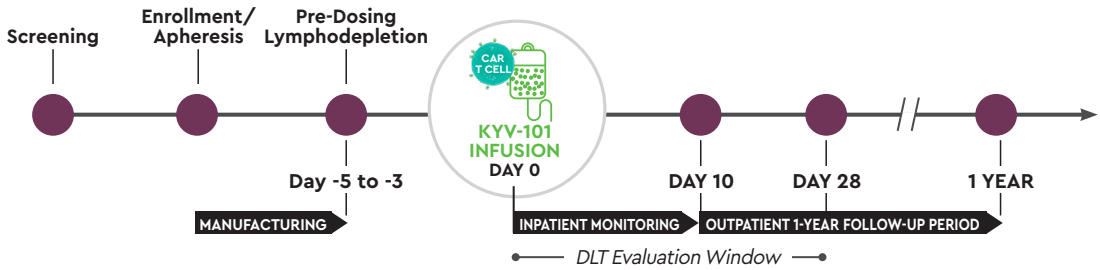
KYSA-8

Phase 2 Trial of KYV-101, a CAR T-Cell Therapy, in Refractory Stiff Person Syndrome

SPS is a rare progressive immune-mediated central nervous system disorder that can lead to permanent disability and increased mortality risk.[1–6] There is a need for new therapeutic paradigms for SPS that can halt disease progression and enable sustained treatment-free remission.[1,7]



Phase 2 (N=25)

Primary Objectives

- Efficacy of KYV-101
- Safety of KYV-101

Key Secondary Objectives

- Further evaluate efficacy of KYV-101
- PK/PD of KYV-101
- Immunogenicity of KYV-101

For more information
Please call **510-925-2484**
or email **ClinicalTrials@kyvernatx.com**

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CAR, chimeric antigen receptor; PD, pharmacodynamics; PK, pharmacokinetics; SPS, stiff person syndrome.

1. Baizabal-Carvallo JF, Jankovic J. *J Neurol Neurosurg Psychiatry*. 2015;86(8):840–848. 2. Dalakas MC. *Neurol Neuroimmunol Neuroinflamm*. 2023;10(3). 3. Muranova A, Shanina E. Stiff Person Syndrome. In: StatPearls. https://www.ncbi.nlm.nih.gov/books/NBK573078/. 4. Rizzi M, et al. *PLoS One*. 2010;5(5):e10838. 5. Levy LM, et al. *Ann Intern Med*. 1999;131(7):522–530. 6. Duddy ME, Baker MR. *Front Neurol Neurosci*. 2009;26:147–165. 7. Dalakas MC. *Neurotherapeutics*. 2022;19(3):832–847.

KYSA-8 Eligible Patients Must Have Treatment Refractory Stiff Person Syndrome

Key Inclusion Criteria

- ≥18 to ≤75 years of age
- Diagnosis of SPS per the following criteria:
  - Rigidity of limb and axial (trunk) muscles prominent in the abdominal and thoracolumbar paraspinal areas and making bending difficult
  - Clinical or electrophysiological evidence (accompanied by clinical evidence) of continuous contraction of agonist and antagonist muscles
  - Episodic spasms precipitated by unexpected noises, tactile stimuli, or emotional upset
  - Absence of any other neurologic disease that could explain the stiffness and rigidity
  - High titer serum anti-GAD65 antibodies at screening – OR – seropositive for anti-glycine receptor antibodies. If anti-GAD65 antibodies are lower than the high titer threshold peripherally but positive in the CSF, the subject can be included. A prior documented high titer anti-GAD65 antibody level may be acceptable with sponsor review
- Active symptoms with inadequate response to at least one immunomodulatory therapy (IVIg, rituximab, or plasmapheresis)
- Stiffness index ≥2

Key Exclusion Criteria

- Bedridden for more than 3 months
- History of CNS or spinal cord tumor, metabolic or infectious cause of myelopathy, genetically inherited progressive CNS disorder, sarcoidosis, non-SPS progressive neurologic condition or progressive multifocal leukoencephalopathy (PML)
- Seizure disorder even if well controlled on antiepileptics
- History of stroke, dementia, Parkinson's disease, cerebellar diseases, psychosis, aphasia, and any other neurologic disorder that is of a nature and severity that the investigator considers would increase the risk for the subject
- Impaired cardiac function or clinically significant cardiac disease
- History of allogeneic or autologous stem cell transplant

CNS, central nervous system; CSF, cerebrospinal fluid; GAD, glutamic acid decarboxylase; IVIg, intravenous immunoglobulin; SPS, stiff person syndrome.

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