p-044

Thursday

Early Results From the ROCKIT-1 Trial: A Phase 2a trial of Fasudil in Patients with Progressive Supranuclear Palsy or Corticobasal Syndrome.

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State of the art:
Rho-kinases (ROCKs) are aberrantly upregulated in neurons from patients with 4-repeat tauopathies, including progressive supranuclear palsy (PSP) and corticobasal degeneration (CBD). ROCK inhibitors increase tau autophagy, decrease tau phosphorylation, and decrease overall tau levels in murine cortical neurons and a drosophila model of tauopathy. ROCKIT-1 (NCT04734379) is the first clinical trial of an oral ROCK inhibitor, fasudil, in patients with clinical syndromes predictive of tauopathy, PSP and corticobasal syndrome (CBS).

Methodology:
This is a phase 2a, open-label, safety, tolerability, and biomarker study of oral fasudil (60mg TID) treatment given over 12 months in patients with PSP Richardson syndrome (N=10) and CBS (N=5). Clinical measures of disease progression, cerebrospinal fluid, and MR imaging data were collected at baseline, Month 6, and Month 12 of treatment.

Results:
N=10 patients with PSP and N=6 patients with CBS were recruited and enrolled at UC San Francisco in 2021. One patient with CBS and one patient with PSP discontinued treatment due to progression in their diseases, and the remaining cohort is predicted to conclude 12 months of treatment in November 2022. Exploratory analysis of clinical measures, CSF biomarkers (including NfL and tau phospho-proteomics), and imaging measures is ongoing.

Conclusion: Oral fasudil appears safe and well tolerated in patients with PSP and CBS. The results of interim analyses of exploratory clinical, fluid biomarker, and imaging endpoints will be discussed during our presentation.

Conflicts of interest

Fasudil is owned by Woolsey Pharmaceuticals, of which Doctors Mian and Tunstal are employees