Do you have patients or are you diagnosed with Multiple System Atrophy?

Multiple System Atrophy (MSA) is a rare, sporadic, progressive, neurodegenerative disorder of the central and autonomic nervous systems. Although the etiology of MSA is unknown, the generation of cytotoxic oxidants by the enzyme myeloperoxidase (MPO) may play an important role in the disease process. AstraZeneca plans to conduct a Phase 2 clinical trial with AZD3241, a potent, selective, brain-permeable MPO inhibitor. The study, entitled “A 12-Week, Multicenter, Randomized, Parallel-Group Study to Assess the Safety, Tolerability, Pharmacokinetics, Biomarker Effects, Efficacy, and Effect on Microglia Activation, as Measured by Positron Emission Tomography, of AZD3241 in Subjects with Multiple System Atrophy” is anticipated to start in the first half of this year. The study is double-blind and placebo-controlled, and will investigate two dosage levels of AZD3241. The study will be conducted at sites in the United States and Europe. Future studies are planned, including a study of longer duration focusing on safety and efficacy.

The primary objectives of this study are:

- To assess the safety and tolerability of AZD3241 in patients with MSA.
- To determine the effect of AZD3241 on microglia activation, as measured by PET imaging of $[^{11}C]PBR28$ binding at baseline and after 12 weeks of treatment (2 scans per patient), in patients with MSA.

A secondary objective is:

- To determine the biomarker effects of AZD3241 in patients with MSA.

Exploratory objectives are:

- To assess the pharmacokinetics of AZD3241 in patients with MSA.
- To assess the efficacy of AZD3241 in patients with MSA. Exploratory efficacy outcome measures include the Unified Multiple System Atrophy Rating Scale (UMSARS), the Composite Autonomic Symptom Scale (COMPASS) Select Change Scale (CCS), and the MSA–Quality of Life scale (MSA-QoL).

Patients may qualify for the study if they:

- Are 30-80 years old.
- Meet criteria for diagnosis of possible or probable MSA (parkinsonian- or cerebellar-subtype) according to the consensus criteria.
- Do not have significant neurological disease other than MSA that may affect motor or autonomic function.

Potential patient eligibility will be confirmed by an independent clinical expert. A Data and Safety Monitoring Board (DSMB) will monitor unblinded safety data on an ongoing basis to ensure the continuing safety of patients.

The study involves:

- A participation period of approximately five months for each patient
- Twelve weeks of treatment with study medication
- Approximately twelve study visits, including two visits to one of five global PET centers
- Imaging procedures, including the use of a radioligand
- Physical and neurological examinations
- Blood draws, ECGs, and vital signs assessments
- Administration of questionnaires

If you’d like more information on this study please visit clinicaltrials.gov, NCT # NCT02388295 or contact the center posting this information at 646-501-4367 or 212-263-7225