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Joint Symposium

Patients supported drug discovery and development for neurological incurable disease termed HTLV-1-associated myelopathy (HAM)

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Some infected with human T-lymphotropic virus type 1 (HTLV-1), which causes adult T cell leukemia/lymphoma (ATL), develop the neuroinflammatory disease HTLV-1 associated myelopathy (HAM). Suffering from progressive spinal cord paralysis, HAM patients experience a low quality of life with high unmet needs. Since the prognosis for HAM patients is extremely poor, there is a strong demand for a novel therapeutic strategy. Therefore, we established a national patient registration system (HAM-net) in collaboration with patient groups to gather data from and distribute information to patients on a nation-wide scale. By establishing a registration system for HAM patients, we have learned more about the variation in the rate of HAM progression that enables to divide patients into groups based on progression speed and compare attributes between groups. In addition, we recently showed that HTLV-1 mainly infects CCR4+ T-cells and causes functional abnormalities that are believed to drive HAM pathogenesis. We next demonstrated that anti-CCR4 antibody is effective at reducing the proviral load and inflammatory response in PBMCs from patients with HAM. Given the overwhelming evidence to support the idea that anti-CCR4 antibody could benefit HAM patients, we began an Investigator-led clinical trial. The phase 1/2a trial of the anti-CCR4 antibodies proceeded smoothly; in January 2016, the clinical studies were completed, and a proof of concept of the safety and efficacy of the treatment was obtained (*N Engl J Med*, 2018).