Building on recently published peer reviewed papers in high impact journals, Drs. Scherbakov and Akbergenov at the University of Zurich and the University of Basel have proposed a novel strategy for developing new medicines for a broad array of neurodegenerative disorders. Their hypothesis, that agents able to increase translational fidelity could have therapeutic potential, is supported by experimental results from transgenic mice engineered to have a higher-than-normal error rate in protein translation. Deficits seen in these animals, but not genetically intact control mice, included neuronal hyperexcitability, perturbations in cerebral glucose metabolism, abnormal epileptiform discharges, and behavioral deficits in which memory, sleep, and emotional regulation are compromised.

This research, while very interesting, should be seen from a commercial perspective as an academic project still in the ideation phase. Included among still unresolved issues are i) clarifying the relevance of laboratory experiments to mechanisms underlying neurodegeneration in patients, ii) understanding the likelihood that a drug modulating translational fidelity would show benefit in already symptomatic individuals, iii) identifying a lead indication for which to develop new chemical matter, and iv) ensuring that what is developed is compatible with current and future standard of care. This view that the program is early is consistent with discussion with the team regarding the workplan and their suggestion that “there won’t be improved chemical matter in 12 months, but there should be greater understanding of the target”.

That said, because amyloid and tau-based strategies alone currently account for almost 30% of clinical programs for Alzheimer’s Disease, investing in other potential strategies is critical (see 10.1002/trc2.12465). Moreover, the potential here is not limited to Alzheimer’s Disease alone but could be applicable to numerous other conditions including Parkinson’s Disease and even healthy aging. As such, a modest amount of capital from CerebrumDAO at this pre-IP stage, if paired with drug development expertise from community members, could result in something attractive for follow-on funding. This view is consistent with the observation that numerous high-profile entities including Alphabet-backed Calico Life Sciences and Westlake Village BioPartners’ Neuron23 have expressed enthusiasm for translational-focused approaches for the development of neurodegeneration.