

## **Preparing for Gene Therapy for PLAN**

There are currently no disease-modifying treatments for PLA2G6-associated neurodegeneration (PLAN). Development of precision therapies is therefore an unmet clinical need and constitutes research priority. Whilst our efforts are underway to develop a gene therapy approach for PLAN, in tandem we are seeking to be 'trial ready' in order to have meaningful outcome measures to gauge both safety and efficacy. Here we will present our work in undertaking a retrospective natural history study for PLAN, as well as the development of radiological and blood/CSF biomarkers and a PLAN focused disease-rating scale. Robust and meaningful clinical outcome measures will be of the utmost importance when clinical trials are planned and designed, as well as for regulatory approval in the longer term.