

Wave Life Sciences: PRECISION-HD1 and PRECISION-HD2

- PRECISION-HD is a global clinical program consisting of the PRECISION-HD1 trial evaluating investigational WVE-120101 targeting SNP1 and the PRECISION-HD2 trial evaluating investigational WVE-120102 targeting SNP2
 - WVE-120101 and WVE-120102 both use the allele-selective approach to selectively lower mHTT while leaving wtHTT relatively intact
 - Two parallel, multicenter, double-blind, randomized, placebo-controlled Phase 1b/2a clinical trials for WVE-120101 and WVE-120102, administered intrathecally, with single-ascending dose and multiple-ascending dose portions
 - Primary objective: Assess safety and tolerability of intrathecal doses in early manifest HD patients
 - Key additional objectives: Measurement of mHTT and total HTT; exploratory pharmacokinetic (PK), pharmacodynamic (PD), clinical and MRI endpoints
 - Key inclusion criteria: early symptomatic HD who have screened positively for the presence of SNP1 or SNP2
- Open-label extension (OLE) study to allow for assessment of longer term safety and tolerability as well as clinical outcomes

