Authorised NCIII List for Psychiatry / Neurosciences:

A Phase 1b multicenter, randomized, double-blind, placebo-controlled study, to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of intrathecally administered single ascending doses of ALN-HTT02 in adult patients with Huntington's disease

Department	Designated Lead	Nominated Pharmacy Lead(s)	Designated Contacts within On-Site Pharmacy Department	Nominated Nursing Lead(s)	NCIIIs Approved for Use	Indication	Licensed Status LM / ULM / LM but unlicensed indication	Aseptic Manipulation/ Preparation Required	Storage in Clinical Area Authorised	Approved Dose Range	Upper Limit	Folder/ Information Storage Location	Location of Intralipid for Potential LA Toxicity
GGC Psychiatry/ Huntington's Disease	Dr James Herron (also PI for Study) Deputy: Dr Maytal Wolfe Administ-ered by Dr Chris Hawthorne Deputy: Dr Louis Chalmers	Dr Samantha Carmichael Deputy: Elena Bichir Senior Pharmacist Clinical Trials	Scott Nicol	Karen Duffy Lead Research Nurse	ALN-HTT02, R&I GC24NE57, Sponsor: Alnylam, PI: James Herron Intrathecal placebo	Huntington's Disease Phase I Clinical Trial	Investigation-al Medicinal Product	Yes	No	Cohort 1: 25mg administered as a 20ml volume / bolus over 2-3 minutes, single dose (20ml CSF removed prior to administrat-ion) 20ml volume / bolus over 2-3 minutes, once every 6 months (20ml CSF removed prior to administrat-ion)	This is a single ascending dose trial – cohort 1 dose will be 25mg in 20ml bolus. Dose escalation will be determined by the study Safety Review Committee. The maximum dose administered will not exceed 1200 mg.	Edge manage-ment system, GCRF shared drive and paper documents – ISF Site file room GCRF @QEUH Pharmacy Shared Drive for NCIIIs	N/A

The study is ALN-HTT02-001, R&I GC24NE575, Sponsor: Alnylam, PI: James Herron, Administering physician: Chris Hawthorne. The IP ALN-HTT02 will be supplied as a sterile solution formulated in a vehicle composed of an isotonic aqueous buffer suitable for IT injection. See the Pharmacy Manual for further details of solution concentration and fill volume. The control drug for this study will be a placebo isotonic aqueous buffer suitable for IT injection. Placebo will be provided by the Sponsor; it will be packaged identically to ALN-HTT02.. The study will have a double-blinded period (placebo controlled) and an open-label period. The dose is determined by the study safety review committee and depends on the cohort to which the patient is recruited. The IP is administered as a 20ml volume once and it will be prepared in the aseptic. The IP is investigated for Huntington's Disease in a Phase I set-up. The Lead Research Nurse/study coordinator for this study is Helen Bannister.