

Progranulin Clinical Trials – Recruiting Spring 2022

Trial Name	INFRONT-3	PROCLAIM	upliFT-D
Sponsor	Alector	Prevail Therapeutics	Passage Bio
Intervention	AL001	PR006	PBFT02
Mechanism	Anti-sortilin antibody optimized for less-frequent dosing	<i>GRN</i> replacement (AAV9)	<i>GRN</i> replacement (AAV1)
Phase	3	1/2	1/2
Trial Dates	2020-2023	2020-2027	2021-2027
Number of Participants	180	15	6
Number of Sites	46	4	9
Site Locations	US, Australia, Canada, Europe	US, Australia, Spain	US, Brazil, Canada, Europe
Need to Know <i>GRN</i> Mutation Status	Yes	Yes	Yes
Ages	25-85	30-80	35-75
Asymptomatic and/or Symptomatic	Either (If symptomatic, must have bvFTD, semantic subtype, or PPA)	Symptomatic	Symptomatic (CDR plus NACC FTLD global score of 0.5 or 1.0)
Interventions	IV infusion every 4 weeks	Single dose, ICM administration	Single dose, ICM administration
Placebo	Yes (saline)	No	No
Timeframe	48 or 96 weeks	12 months (efficacy) 5 years (safety)	2 years (efficacy) 5 years (safety)
Primary Outcomes	Evaluation of efficacy of AL001 as measured by the CDR plus NACC FTLD-SB	<ol style="list-style-type: none"> 1. Safety and tolerability 2. Blood <i>PGRN</i> immunogenicity 3. CSF <i>PGRN</i> immunogenicity 4. Blood AAV9 immunogenicity 5. CSF AAV9 immunogenicity 6. Blood [<i>PGRN</i>] 7. CSF [<i>PGRN</i>] 	<ol style="list-style-type: none"> 1. Safety and tolerability, 2. Change in nerve conduction velocity 3. Change in nerve conduction amplitude 4. Change in total neuropathy score-nurse 5. Change in neurological exam 6. Number of participants with clinically significant lab abnormalities 7. Assess humoral response against the vector and transgene in serum 8. Assess humoral response against the vector and transgene in CSF