

List of interventional PNH clinical trials marked "recruiting" or "not yet recruiting" as of January 19th 2024

Company	ADARx	Alexion	Apellis	Care Pharma	Chengdu	Chengdu	Chengdu	Fred Hutchinson Cancer Center	Haisco	Haisco	Longbo	Kira Pharmaceuticals	Nanjing	JHLBI	Novartis	NovelMed	NovelMed	NovelMed	Omeros	Omeros	Regeneron	Regeneron	Wuhan Createrna	Wuhan Createrna	Wuhan Createrna
trial	ADX-038-101	D7332C00006	APL-2-PNH-209	CAR-120-PNH-102/201	NCT06238544	HRS-5965-301	HRS-5965-302	BMTCTN1904	HSK39297-201	HSK39297-203	PLD-1-P005-01	KP104-201	NTD5082-201	180090	CLMPO23C120018	NM8074	NM8074	NM8074-PNH-101	OMS906-PNH-002	OMS906-PNH-003	R3918-PNH-1021	2020	MYO08211A-PNH-2-01-F	MYO08211A-PNH-2-01-F	NCT06543459
ClinicalTrials ID	NCT05876312	NCT06449001	NCT04901936	NCT05539248	NCT06238544	NCT06593938	NCT06715943	NCT04965597	NCT06745622	NCT06745622	NCT06294301	NCT05476887	NCT0582-201	NCT03520647	NCT047471613	NCT05646524	NCT05646563	NCT05731050	NCT05889299	NCT06298955	NCT05133531	NCT05744921	NCT06050226	NCT06134414	NCT06543459
name	ADX-038	Danicocan	APL-2 Pezacetacolan Empaveli® Aspaveli® C3	CAN106		HRS-5965	HRS-5965	treosulfan, fludarabine and rATG	HSK39297	HSK39297	LP-005	KP104	NTD5082	Cyclophosphamide	lotacocan			NM8074	OMS906	OMS906	Pozelimab and Cemdisiran	Pozelimab and Cemdisiran	MYO08211A	MYO08211A	
trade name																									
determination	genes	Faktor D		CS								alt. + term. pathway	CFB factor		Factor B	anti-Factor Bb	anti-Factor Bb				CS	CS	hemolysis	hemolysis	
substance	siRNA duplex oligonucleotide	monoclonal antibody	Cyclopeptide	monoclonal antibody				cytostatics, cytotoxines and antibodies				biologic	small molecule	cytostatic	low molecular compound	monoclonal antibody	monoclonal antibody				monoclonal antibody i.v./hem.s.c.	monoclonal antibody and RNAi	oral	oral	
application		oral	s.c.	i.v.	oral	oral	oral	i.v.	o.o.	o.o.	i.v.	oral	oral	i.v.	oral	i.v.	i.v.	s.c.	i.v.			s.c.	oral	oral	oral
frequency		daily	2 x weekly	every 4 or every 8 weeks	daily dosage	probably daily dose	probably daily dose	6 days daily until transplantation, following 180 days of prophylaxis	probably daily for 24 weeks	probably daily	single dosage	weekly/biweekly	daily dosage	once, hospital stay over weeks	2 x daily	every 2 weeks	every 4 weeks/2 weeks	every two weeks, total of 6 doses	every four weeks	every eight weeks		every four weeks	2x daily	2 x daily	single dose
phase of study	1	3	2	2	2	3	3	2	2	2	1	2	2	2	3	2	2	2	1b	2	2	3	3	2	2
research question			evaluate efficacy as a add-on treatment	assessment of safety, tolerability, efficacy, pc and pc in complement-inhibitor treatment	assessment of safety and tolerability	evaluation of efficacy and safety	evaluation of efficacy and safety	assessment of therapeutic potential in patients naive to complement inhibitor therapy	assessment of safety, tolerability, pharmacokinetics, and Pharmacodynamics of Escalating Single and Multiple Doses	long-term safety, naive and efficacy	tolerability and efficacy	inhibitor-naive patients, dose and efficacy assessment	evaluation of efficacy, safety and PK/PD characteristics	haplo-identical transplantation and post-transplant cyclophosphamide	long-term evaluation	immuno-generacy in 2	immuno-generacy in 2	immuno-generacy in 2	immuno-generacy in 2	immuno-generacy in 2	immuno-generacy in 2	immuno-generacy in 2	immuno-generacy in 2	immuno-generacy in 2	immuno-generacy in 2
start	07/2023	01/2025	02/2023	03/2022	02/2024	10/2024	12/2024	04/2022	02/2024	01/2025	11/2023	11/2022	01/2025	02/2019	07/2021	11/2023	9/2023	06/2025	12/2022	02/2024	8/2022	09/2023	07/2023	12/2024	08/2024
locations	Australia		Czechia	France	Malaysia	Netherlands	Serbia	Spain	Thailand	UK	USA														
Remarks	This table contains clinical trials listed under ClinicalTrials.gov that are marked recruiting or not yet recruiting.																								
Source	ClinicalTrials.gov (https://clinicaltrials.gov/ct2/home)																								
Last updated	05 January 2024																								

