

List of interventional PNH clinical trials marked "recruiting" or "not yet recruiting" as of July 31st 2023

Company	ADARx	Alexion	Alexion	Apellis	BioCryst	Care Pharma	Fred Hutchinson Cancer Center	Kira Pharmaceuticals	NHLBI	NHLBI	Novartis	Novartis	NovelMed	NovelMed	NovelMed	Omeros	Regeneron	Regeneron	Roche						
trial	ADX-038-101	ALXN2040-PNH-303	D7414C00001	APL2-PNH-209	BCX9930-205 2021-006776-17	CAN 106-PNH-102/201	BMTCTN1904	KP104-201	100154 10-H-0154	180090 18-h-0090	CLNP023C12001B	CLNP023C12303	NM8074	NM8074	NM8074-PNH-101	OMS906-PNH-002	R3918-PNH-2021 ACCESS-1	2050 004931-10	2021- B042161 COMMODORE 1						
ClinicalTrials ID	NCT05876312	NCT05389449	NCT05886244	NCT04901936	NCT05741346	NCT05539248	NCT04965597	NCT05476887	NCT01174108	NCT03520647	NCT04747613	NCT05630001	NCT05646524	NCT05646563	NCT05731050	NCT05889299	NCT05133531	NCT05744921	NCT04432584						
name	ADX-038	ALXN2040, ACH-0144471, Danicopan	Eculizumab	APL-2 Pegcetacoplan Empaveli® Aspaveli®	BCX9930	CAN106	treosulfan, fludarabine and rATG	KP104		Cyclophosphamide	LNP023 Iptacopan	Iptacopan			NM8074	OMS906	Pozelimab and Cemdisiran	Pozelimab and Cemdisiran	Crovalimab						
trade name determination	genes	Factor D	C5	C3	Factor D	C5		alt. + term. pathway			Factor B	C5	anti-Factor Bb	anti-Factor Bb	anti-Factor Bb	MASP-3	C5	C5	C5						
substance	siRNA duplex oligonucleotide	low molecular compound	monoclonal antibody	Cyclopeptide		monoclonal antibody	cytostatics, cytotoxines and antibodies	biologic	allogenic stem cell therapy	cytostatic	low molecular compound	low molecular compound	monoclonal antibody	monoclonal antibody	monoclonal antibody	monoclonal antibody	monoclonal antibody	monoclonal antibody and RNAi	monoclonal antibody						
application		oral	i.v. once a week for 22 days, followed by once every two weeks	s.c. 2 x weekly	oral 2	i.v. 2	i.v. 2	i.v. 2	i.v. single infusion combined with various medicaments	i.v. once, hospital stay over weeks	oral 2 x daily	oral 2 x daily		i.v. every 2 weeks	i.v. every 4 weeks/2 weeks	s.c. every two weeks, total of 6 doses	s.c. every four weeks	s.c. i.v./then s.c. Cemdisiran s.c.	s.c. every four weeks	single i.v./then s.c. every 4 weeks					
frequency phase of study	1	3	3	2	2	2	2	2	2	2	3	3	2	2	2	1b	3	3	3	3					
research question	safety, tolerability, pk and pd of adx-038 in healthy volunteers and pnh-patients	LTE add on to CSI treatment in patients previously treated with danicopan	safety of eculizumab	pediatric recruiting	continued treatment for patients already receiving this drug	inhibitor treatment-naive and pretreated adults	assessment of safety, tolerability, efficacy, pc and pc in complement-	inhibitor-naive patients, dose and efficacy assessment	variation of allogenic stem cell transplantation	haplo-identical transplantation and cyclophosphamide in SAA and PNH	long-term evaluation	patients on stable eculizumab or ravulizumab treatment and Hb<10 g/dl	treatment-naive patients, assessment of safety, efficacy and immunogenicity in 2 dosing schemes	mono vs eculizumab addon therapy to assess safety, efficacy and immunogenicity	safety and efficacy of NM8074 in Soliris-treated pnh patients	versus ravulizumab, treatment naive or recently inhibitor treated	safety, efficacy and tolerability of combination therapy	versus eculizumab, inhibitor treated							
status	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	not yet recruiting	not yet recruiting	not yet recruiting	recruiting	recruiting	recruiting	recruiting						
start	07/2023	10/2022	07/2023	02/2021	01/2023	03/2022	04/2022	11/2022	12/2010	02/2019	07/2021	04/2023	11/2023	9/2023	06/2025	12/2022	8/2022	03/2023	09/2020						
locations	Australia	USA	China	Czechia	France	China	USA	China	USA	USA	Brazil	China	France	Germany	Italy	Japan	Lithuania	Malaysia	Netherlands	Singapore	South Korea	Spain	Taiwan	UK	USA
Remarks	This table contains clinical trials listed under ClinicalTrials.gov that are marked recruiting or not yet recruiting.																								
Source	<a href="https://clinicaltrials.gov/">https://clinicaltrials.gov/</a>																								
Last updated	July 31st 2023																								

List of interventional PNH clinical trials marked "active, not recruiting" or "enrolling by invitation" as of July 31st 2023

company	Akari	Alexion	Alexion	Alexion	Apellis	BioCryst	BioCryst	BioCryst	Regeneron	Regeneron	Roche	Roche	Roche	Roswell	Wuhan Createrna	Wuhan Creterna
trial	AK585 CONSENT II	ALXN1210-PNH-303	ACH228-110	ALXN2040-PNH-301	ALPHA	BCX9930-201	BCX9930-203	BCX9930-203	R3918-PNH-2092	ACCESS-2	BP39144	BO42162	YO42311	I 40916	MY008211-1-01	MY008211-1-02
ClinicalTrials ID	NCT03427060	NCT03748823	NCT04170023	NCT04469465	NCT03531255	NCT04702568	NCT05116774	NCT05116787	NCT04811716	NCT05131204	NCT03157635	NCT04434092	NCT04654468	NCT03333486	NCT05642585	NCT05828472
name	Nomacopan/ Coversin	Ravulizumab	ALXN2050 (ACH-0145	Danicopan	Pegcetacoplan	BCX9930	BCX9930	BCX9930	Pozelimab and Cemdisiran	Pozelimab and Cemdisiran	Crovalimab	Crovalimab	Crovalimab		MY008211A	MY008211A
trade name					Empaveli® Aspaveli®											
determination	C5	C5	Factor D	Factor D	C3	Factor D	Factor D	Factor D	C5	C5	C5	C5	C5		Factor B	Factor B
substance	tick saliva protein	monoclonal antibody		low molecular compound	Cyclopeptide	low molecular compound	low molecular compound	low molecular compound	monoclonal antibody/siRNA	monoclonal antibody	monoclonal antibody	monoclonal antibody	monoclonal antibody		small-molecule compound	small-molecule compound
application	s.c.	i.v. and s.c.	oral	oral	s.c.	oral	oral	oral	i.v. / s.c.	Cemdesiran s.c.	i.v. / s.c.	single i.v./then s.c.	single i.v./then s.c.	i.v.	oral	oral
frequency	2 x daily	weekly		3 x daily	2 x weekly	2 x daily	2 x daily	2 x daily	every 2 or 4 weeks	every 2 or 4 weeks	every 4 weeks	every 4 weeks	every 4 weeks	at least 6 days	daily	for 7 days
phase of study	2	3	2	3	3	2	2	2	2	3	1/2	3	3	2	1	1
research question	patients with resistance to eculizumab enrolling by invitation	ravulizumab i.v. vs s.c. in pnh patients treated with eculizumab	safety and efficacy of ALXN2050 as a monotherapy	add-on therapy to eculizumab or ravulizumab, ALXN2040 vs placebo	long-term evaluation	long-term evaluation	monotherapy vs C5 inhibitor in pre-treated patients who are still anemic	monotherapy vs placebo in treatment-naive patients	Pozelimab and cemdisiran combined after pozelimab monotherapy	versus eculizumab or ravulizumab	versus eculizumab, treatment-naive	treatment naive	combination of different therapies to treat blood cancer and other conditions incl. PNH	single ascending dose vs placebo	active, not recruiting	safety, tolerability, PK and PD of MY008211A in healthy subjects
status		active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting
start	05/2018	03/2019	12/2019	12/2020	08/2018	12/2020	12/2021	10/2021	07/2021	10/2022	11/2016	10/2020	03/2021	12/2017	05/2022	01/2023
locations	USA		Canada Italy Korea New Zealand Spain Turkey UK	Brazil Canada Czechia France Germany Greece Israel Japan Japan Malaysia Netherlands Poland South Korea Spain Taiwan Thailand UK USA	Australia Belgium Bulgaria Canada France France Germany Hong Kong Japan Malaysia Russia Serbia Singapore South Korea Spain Thailand UK USA	Austria South Africa UK	France Hungary Italy Spain UK	Malaysia South Africa Taiwan UK	Hong Kong Hungary Malaysia South Korea Taiwan UK	USA	France Germany Hungary Italy Japan Netherlands South Korea	Argentina Australia Brazil China France Germany Greece Hong Kong Japan Lithuania Malaysia Mexico Netherlands Philippines Poland Portugal Romania Singapore South Africa South Korea Spain Sweden Taiwan Thailand Turkey Ukraine UK	China USA	China	China	
Remarks	This table contains clinical trials listed under ClinicalTrials.gov that are marked active/not recruiting or enrolling by invitation and have no results published.															
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Last updated	July 31st 2023															