

List of interventional PNH clinical trials marked "recruiting" or "not yet recruiting" as of 6 June 2022

company	Alexion	Alexion	Alexion	Apellis	Arrowhead	Novartis	Novartis	Regeneron	Regeneron	Regeneron	Roche	Roche
trial	ACH228-110	ALXN2040-PNH-301 ALPHA	ALXN2040-PNH-303 LTE	APL2-PNH-209	AROC3-1001	CLNP023C12301 APPOINT-PNH	CLNP023C12001B	R3918-PNH-20105	R3918-PNH-2022 ACCESS-2	R3918-PNH-2021 ACCESS-1	BO42161 COMMODORE 1	BO42162 COMMODORE 2
	NCT04170023	NCT04469465		NCT04901936	NCT05083364	NCT04820530	NCT04747613	NCT04888507	NCT05131204	NCT05133531	NCT04432584	NCT04434092
name	ALXN2050 Danicopan	ALXN2040 Danicopan	ALXN2040 Danicopan	APL-2 Pegcetacoplan	ARO-C3	LNP023 Iptacopan	LNP023 Iptacopan	Pozelimab and Cemdisiran	Pozelimab and Cemdisiran	Pozelimab and Cemdisiran	Crovalimab	Crovalimab
trade name				Empaveli®/Aspaveli®								
determination	Factor D	Factor D	Factor D	C3	C3	Factor B	Factor B	C5	C5	C5	C5	C5
substance	low molecular compound	low molecular compound	low molecular compound	Cyclopeptide		low molecular compound	low molecular compound	monoclonal antibody	monoclonal antibody	monoclonal antibody	monoclonal antibody	monoclonal antibody
application	oral	oral	oral	s.c.	s.c.	oral	oral	Pozelimab single i.v./then s.c. Cemdesiran s.c.	Pozelimab single i.v./then s.c. Cemdesiran s.c.	Pozelimab single i.v./then s.c. Cemdesiran s.c.	single i.v./then s.c.	single i.v./then s.c.
frequency	2 x daily	3 x daily	3 x daily	2 x weekly		2 x daily	2 x daily				every 4 weeks	every 4 weeks
phase of study	2	3	3	2	1/2a	3	3	2	3	3	3	3
research question	monotherapy, patients treatment naive or after C5 inhibitor therapy	add-on therapy to eculizumab or ravulizumab, ALXN2040 vs placebo	add-on therapy to eculizumab or ravulizumab, ALXN2040, LTE for patients who completed previous study with danicopan	pediatric	adult PNH patients and others	monotherapy, patients naive to inhibitor treatment	long-term evaluation	safety and tolerability after switch from eculizumab	versus eculizumab or ravulizumab	versus eculizumab or ravulizumab, treatment naive or not recently inhibitor treated	versus eculizumab, inhibitor treated	versus eculizumab, treatment-naive
status	recruiting	recruiting	not yet recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting
start	12/2019	12/2020	5/2022	02/2021	02/2022	07/2021	07/2021	7/2021	3/2022	7/2022	09/2020	10/2020
locations	Canada	Brazil	tbd	USA	New Zealand	China	Czechia	UK	USA	USA	Australia	Argentina
	Germany	Canada				Czechia	France				Belgium	Australia
	Italy	France				France	Germany				Brazil	Belgium
	New Zealand	Germany				Germany	Italy				Canada	Brazil
	South Korea	Italy				Italy	Japan				Czechia	Canada
	Spain	Japan				Japan	Lithuania				Estonia	Chile
	Turkey	Malaysia				Malaysia	Malaysia				France	China
	UK	Netherlands				Singapore	Netherlands				Germany	Colombia
		Poland				South Korea	Singapore				Greece	Estonia
		South Korea				UK	South Korea				Hong Kong	France
		Spain					Spain				Hungary	Germany
		Taiwan					Taiwan				Ireland	Greece
		Thailand									Italy	Hong Kong
		UK									Japan	Hungary
		USA									Netherlands	Ireland
											Poland	Italy
											Portugal	Japan
											Saudi Arabia	Lithuania
											Singapore	Malaysia
											South Korea	Mexico
											Spain	Netherlands
											Sweden	Philippines
											Taiwan	Poland
											Turkey	Portugal
											UK	Romania
											USA	Saudi Arabia
												Singapore
												South Africa
												South Korea
												Spain
												Sweden
												Taiwan
												Thailand
												Turkey
												Ukraine
												UK
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	06 June 2022											

List of interventional PNH clinical trials marked "active, not recruiting" or "enrolling by invitation" as of 6 June 2022

company	Akari	Alexion	Alexion	Amgen	Apellis	BioCryst	BioCryst	BioCryst	Novartis	Novartis	Regeneron	Roche	Roche
trial	AK585 CONSENT II	ACH471-103	ALXN1210-PNH-401	20150168 DAHLIA	APL2-307	BCX9930-201	BCX9930-203 REDEEM-1	BCX9930-203 REDEEM-2	CLNP023C12302 APPLY-PNH	CLFG316X2201	R3918-PNH-2092	BP39144 COMPOSER	YO42311 COMMODORE 3
ClinicalTrials ID	NCT03427060			NCT03818607	NCT03531255				NCT04558918		NCT04811716	NCT03157635	NCT04654468
EudraCT		NCT03181633	NCT04320602			NCT04702568	NCT05116774	NCT05116787	2019-004665-40	NCT02534909	2020-005005-17	2016-002128-10	
name	Nomacopan/ Coversin	ACH-0144471 Danicopan	Ravulizumab	ABP 959	APL-2 Pegcetacoplan	BCX9930	BCX9930	BCX9930	LNP023 Iptacopan	LFG316 Tesidulomab	Pozelimab and Cemdisiran	Crovalimab	Crovalimab
trade name			Ultomiris®		Empaveli®/Aspaveli®								
determination	C5	Factor D	C5	C5	C3	Factor D	Factor D	Factor D	Factor B	C5	C5	C5	C5
substance	tick saliva protein	low molecular compound	monoclonal antibody	Eculizumab Biosimilar	Cyclopeptide	low molecular compound	low molecular compound	low molecular compound	low molecular compound	monoclonal antibody	monoclonal antibody/siRNA	monoclonal anitbody	monoclonal anitbody
application	s.c.	oral	i.v.	i.v.	s.c.	oral	oral	oral	oral	i.v.	i.v. / s.c.	i.v. / s.c.	single i.v./then s.c.
frequency	2 x daily	3 x daily	every 8 weeks	every 2 weeks	2 x weekly	2 x daily	2 x daily	2 x daily	2 x daily	2-weekly /month.	every 2 or 4 weeks	every 4 weeks	every 4 weeks
phase of study	2	2	4	3	3	2	2	2	3	2	2	1/2	3
research question	patients with resistance to eculizumab	long-term evaluation for patients who had danicopan	patients in need of high dose eculizumab	versus eculizumab	long-term evaluation	long-term evaluation	monotherapy vs C5 inhibitor in pre-treated patients	monotherapy vs placebo in treatment naive patients	patients with residual anemia despite complement inhibitor treatment	determination whether LFG316 can induce a hematological response	Pozelimab and cemdisiran combined after pozelimab monotherapy		treatment naive
status	enolling by invitation	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	06/2017	03/2021	04/2019	08/2018	12/2020	12/2021	10/2021	01/2021	09/2015	07/2021	11/2016	03/2021
locations	USA	Italy	UK	Czechia	Australia	Austria	France	Malaysia	Brazil	Czechia	Hong Kong	France	China
		New Zealand		Finland	Belgium	South Africa	Hungary	South Africa	Czechia	Japan	Hungary	Germany	
		South Korea		France	Bulgaria	UK	Spain	Spain	France	Lithuania	Malaysia	Hungary	
				Ireland	Canada		Taiwan	Taiwan	Germany		South Korea	Italy	
				Italy	France		UK	USA	Italy		Taiwan	Japan	
				Netherlands	Germany		USA		Japan		UK	Netherlands	
				Norway	Hong Kong				Netherlands			South Korea	
				Portugal	Japan				South Korea				
				Slovenia	Malaysia				Spain				
				Spain	Russia				Taiwan				
				Sweden	Serbia				UK				
				Turkey	Singapore				USA				
				UK	South Korea								
				USA	Spain								
					Thailand								
					UK								
					USA								
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.												
Source	ClinicalTrials.gov (https://clinicaltrials.gov/ct2/home)												
Last updated	06 June 2022												