

# List of interventional PNH clinical trials marked "recruiting" or "not yet recruiting" as of 10 January 2023

company	Alexion	Alexion	Alexion	Apellis	Arrowhead	Care Pharma	Kira Pharmaceuticals	Novartis	Novartis	NovelMed	NovelMed	Regeneron	Roche
trial	ACH228-110	ALXN2050	ALXN2040-PNH-303	APL2-PNH-209	AROC3-1001	CAN 106-PNH-102/201	KP104-201	CLNP023C12001B	CLNP023C12303	NM8074	NM8074	R3918-PNH-2021	BO42161
ClinicalTrials ID	NCT04170023	NCT04170023	NCT05389449	NCT04901936	NCT05083364	NCT05539248	NCT05476887	NCT04747613	NCT05630001	NCT05646524	NCT05646563	ACCESS-1	COMMODORE 1
name	ALXN2050 Vemircopan	ALXN2050 Danicopan	ALXN2040, ACH-0144471, Danicopan	APL-2 Pegcetacoplan Empaveli® Aspaveli®	ARO-C3	CAN106	KP104	LNP023 Iptacopan	Iptacopan			Pozelimab and Cemdisiran	Crovalimab
trade name													
determination	Factor D	Factor D	Factor D	C3	C3	C5	alt. + term. pathway	Factor B	C5	anti-Factor Bb	anti-Factor Bb	C5	C5
substance	low molecular compound	low molecular compound	low molecular compound	Cyclopeptide		monoclonal antibody	biologic	low molecular compound	low molecular compound	monoclonal antibody	monoclonal antibody	monoclonal antibody	monoclonal anitbody
application	oral	oral	oral	s.c.	s.c.	i.v.	i.v.	oral	oral		i.v.	Pozelimab 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		every 4 or every 8	weekly/biweekly	2 x daily	2 x daily	every 2 weeks	every 4 weeks/2		every 4 weeks
phase of study	2	2	3	2	1/2a	1/2	2	3	3	2	2	3	3
research question	monotherapy, patients treatment naive or after C5 inhibitor therapy	monotherapy for untreated and pretreated patients and for patients who completed previous study with danicopan	LTE add on to C5i treatment in patients previously treated with danicopan	pediatric recruiting	adult PNH patients and others recruiting	assessment of safety, tolerability, efficacy, pc and pc in complement-inhibitor treatment-naive and pretreated adults recruiting	inhibitor-naive patients, dose and efficacy assessment not yet recruiting	long-term evaluation recruiting	patients on stable eculizumab or ravulizumab treatment and Hb≥10 g/dl not yet recruiting	treatment-naive patients, assessment of safety, efficacy and immunogenicity in 2 dosing schemes not yet recruiting	mono vs eculizumab addon therapy to assess safety, efficacy and immunogenicity not yet recruiting	versus ravulizumab, treatment naive or not recently inhibitor treated recruiting	versus eculizumab, inhibitor treated recruiting
status	recruiting	recruiting	not yet recruiting	recruiting	recruiting	recruiting	not yet recruiting	recruiting	not yet recruiting	not yet recruiting	not yet recruiting	recruiting	recruiting
start	12/2019	12/2019	11/2022	02/2021	02/2022	03/2022	02/2023	07/2021	04/2023	11/2023	9/2023	8/2022	09/2020
locations	Canada Germany Italy New Zealand South Korea Spain Turkey UK	Canada Germany Italy	UK USA	Czechia France Malaysia Netherlands	Australia New Zealand South Korea Thailand	China	China Brazil Czechia France	France Germany Italy Japan Lithuania Malaysia Netherlands Singapore South Korea Spain Taiwan UK USA	France Germany Italy Japan Lithuania Malaysia Netherlands Singapore South Korea Spain Taiwan UK USA	France Germany Italy Japan Lithuania Malaysia Netherlands Singapore South Korea Spain Taiwan UK USA	France Germany Italy Japan Lithuania Malaysia Netherlands Singapore South Korea Spain Taiwan UK USA	Singapore South Korea Taiwan USA	Australia Belgium Brazil Canada Czechia Estonia France Germany Greece Hong Kong Hungary Ireland Italy Japan Netherlands Poland Portugal Saudi Arabia Singapore South Korea Spain Sweden Taiwan Turkey UK USA
Remarks	This table contains clinical trials listed under ClinicalTrials.gov that are marked recruiting or not yet recruiting.												
Source	ClinicalTrials.gov ( <a href="https://clinicaltrials.gov/ct2/home">https://clinicaltrials.gov/ct2/home</a> )												
Last updated	10 January 2023												

List of interventional PNH clinical trials marked "active, not recruiting" or "enrolling by invitation" as of 10 January 2023

company	Akari	Alexion	Alexion	Apellis	BioCryst	BioCryst	BioCryst	Kira Pharmaceuticals	Novartis	Novartis	Regeneron	Regeneron	Regeneron	Roche	Roche	Roche	Wuhan Createrna
<b>trial</b>	AK585	ALXN1210-PNH-401	ALXN2040-PNH-301	APL2-307	BCX9930-201	BCX9930-203	BCX9930-203	KP104-101	CLNP023C12302	CLNP023C12301	R3918-PNH-2092	R3918-PNH-20105	R3918-PNH-2022	BP39144	BO42162	YO42311	MY008211-1-01
<b>ClinicalTrials ID</b>	NCT03427060	NCT04320602	NCT04469465	NCT03531255	NCT04702568	REDEEM-1	REDEEM-2	NCT05490017	APPLY-PNH	APPOINT-PNH	NCT04811716	NCT04888507	NCT05131204	COMPOSER	COMMODORE 2	COMMODORE 3	NCT05642585
<b>name</b>	Nomacopan/ Coversin	Ravulizumab	Danicopan	APL-2 Pegcetacoplan Empaveli*	BCX9930	BCX9930	BCX9930	KP104	LNP023 Iptacopan	LNP023 Iptacopan	Pozelimab and Cemdisiran	Pozelimab and Cemdisiran	Pozelimab and Cemdisiran	Crovalimab	Crovalimab	Crovalimab	MY008211A
<b>trade name</b>		Ultomiris*		Aspaveli*													
<b>determination</b>	C5	C5	Factor D	C3	Factor D	Factor D	Factor D	alt. + term. pathway	Factor B	Factor B	C5	C5	C5	C5	C5	C5	C5
<b>substance</b>	tick saliva protein	monoclonal antibody	low molecular compound	Cyclopeptide	low molecular compound	low molecular compound	low molecular compound	biologic	low molecular compound	low molecular compound	monoclonal antibody/siRNA	monoclonal antibody	monoclonal antibody	monoclonal antibody	monoclonal antibody	monoclonal antibody	monoclonal antibody
<b>application</b>	s.c.	i.v.	oral	s.c.	oral	oral	oral	i.v. or s.c.	oral	oral	i.v. / s.c.	Cemdesiran s.c.	Cemdesiran s.c.	i.v. / s.c.	single i.v./then s.c.	single i.v./then s.c.	oral
<b>frequency</b>	2 x daily	every 8 weeks	3 x daily	2 x weekly	2 x daily	2 x daily	2 x daily	weekly/biweekly	2 x daily	2 x daily	every 2 or 4 weeks	every 2 or 4 weeks	every 2 or 4 weeks	every 4 weeks	every 4 weeks	every 4 weeks	daily
<b>phase of study</b>	2	4	3	3	2	2	2	2	3	3	2	2	3	1/2	3	3	1
<b>research question</b>	patients with resistance to eculizumab enrolling by invitation	patients in need of high dose eculizumab active, not recruiting	add-on therapy to ravulizumab or placebo active, not recruiting	long-term evaluation active, not recruiting	long-term evaluation active, not recruiting	monotherapy vs C5 inhibitor in pre-treated patients who are still anemic active, not recruiting	monotherapy vs placebo in treatment-naive patients active, not recruiting	vs placebo active, not recruiting	patients with residual anemia despite complement inhibitor treatment active, not recruiting	monotherapy, patients naive to inhibitor treatment active, not recruiting	monotherapy active, not recruiting	Pozelimab and cemdisiran combined after eculizumab active, not recruiting	safety and tolerability after switch from eculizumab active, not recruiting	versus eculizumab or ravulizumab active, not recruiting	versus eculizumab, treatment-naive active, not recruiting	treatment naive active, not recruiting	single ascending dose vs placebo active, not recruiting
<b>start</b>	05/2018	03/2021	12/2020	08/2018	12/2020	12/2021	10/2021	12/2020	01/2021	07/2021	07/2021	07/2021	7/2021	11/2016	10/2020	03/2021	05/2022
<b>locations</b>	USA	UK	Brazil Canada Czechia France Germany Greece Israel Italy Japan Malaysia Netherlands Poland South Korea Spain Taiwan Thailand UK USA	Australia Austria Belgium Bulgaria Canada France Germany Greece Hong Kong Japan Malaysia Russia Serbia Singapore Spain Thailand UK USA	Austria South Africa UK	France Hungary Italy Spain UK	Malaysia South Africa	Australia	Brazil Czechia France Germany Italy Japan Netherlands South Korea Spain Taiwan UK USA	China France Germany Italy Malaysia South Korea Singapore South Korea UK	Hong Kong Hungary Malaysia South Korea Taiwan UK	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	
<b>Remarks</b>	This table contains clinical trials listed under ClinicalTrials.gov that are marked active/not recruiting or enrolling by invitation and have no results published.																
<b>Source</b>	ClinicalTrials.gov ( <a href="https://clinicaltrials.gov/ct2/home">https://clinicaltrials.gov/ct2/home</a> )																
<b>Last updated</b>	10 January 2023																