

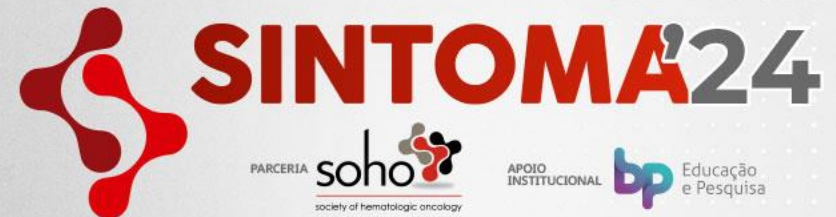
Inibidores de complemento para HPN: quanto mais próximo melhor?

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São Paulo, Brasil



Declaração de Conflito de Interesse

De acordo com a Norma 1595/2000 do Conselho Federal de Medicina e com a RDC 96/2008 da ANVISA, declaro que:

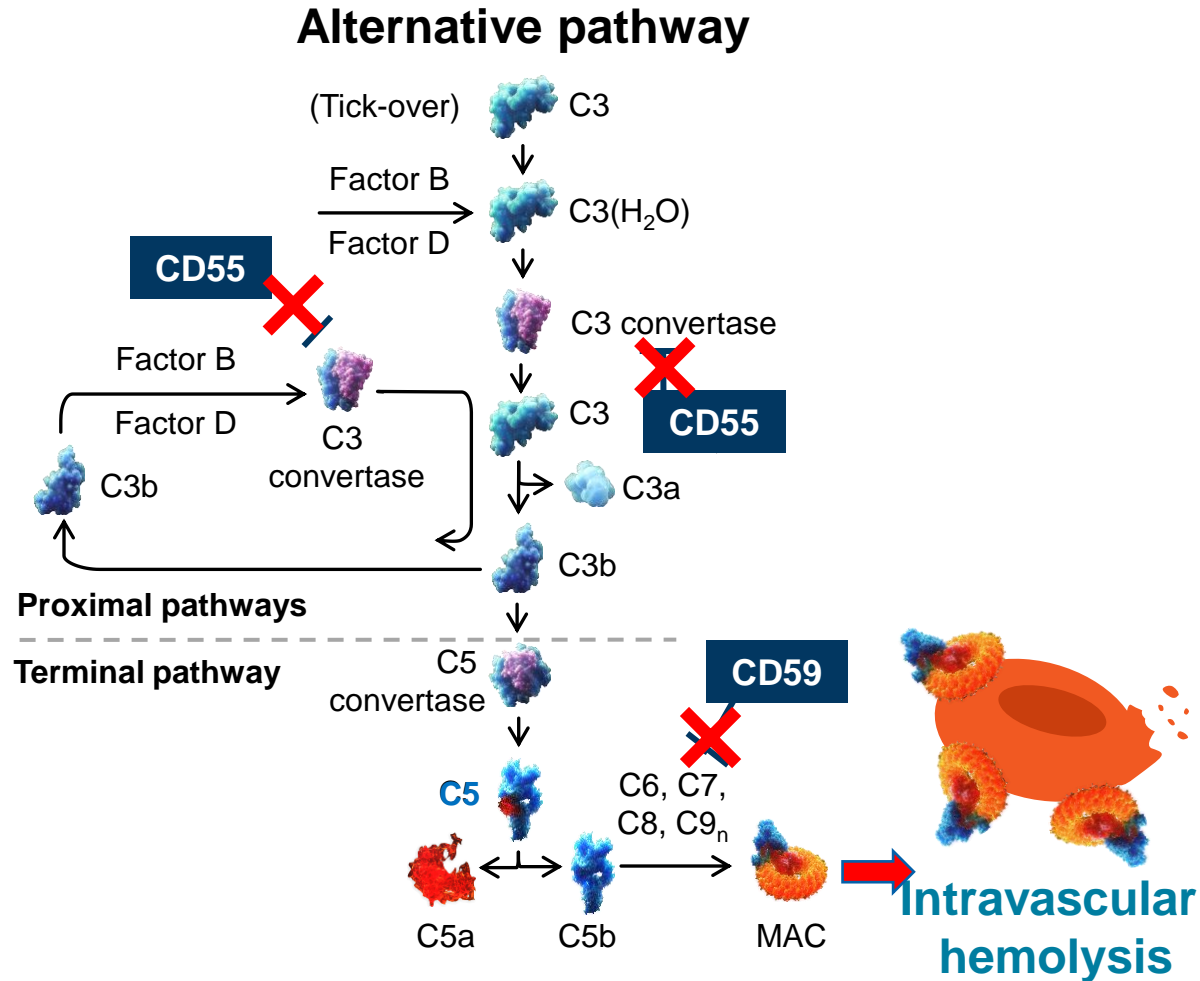
- Pesquisa Clínica – como investigador: Roche, Novartis, BioCryst, AstraZeneca
- Apresentações Científicas – Novartis, Roche, Alexion, Janssen, AstraZeneca
- Atividades de Consultoria – Roche, Alexion, Janssen, Pfizer, BioCryst, AstraZeneca
- Speaker - Novartis, Pfizer, Alexion, AstraZeneca, Amgen, BMS

Declaro não ter ações, opções, patentes ou royalties das empresas supracitadas.

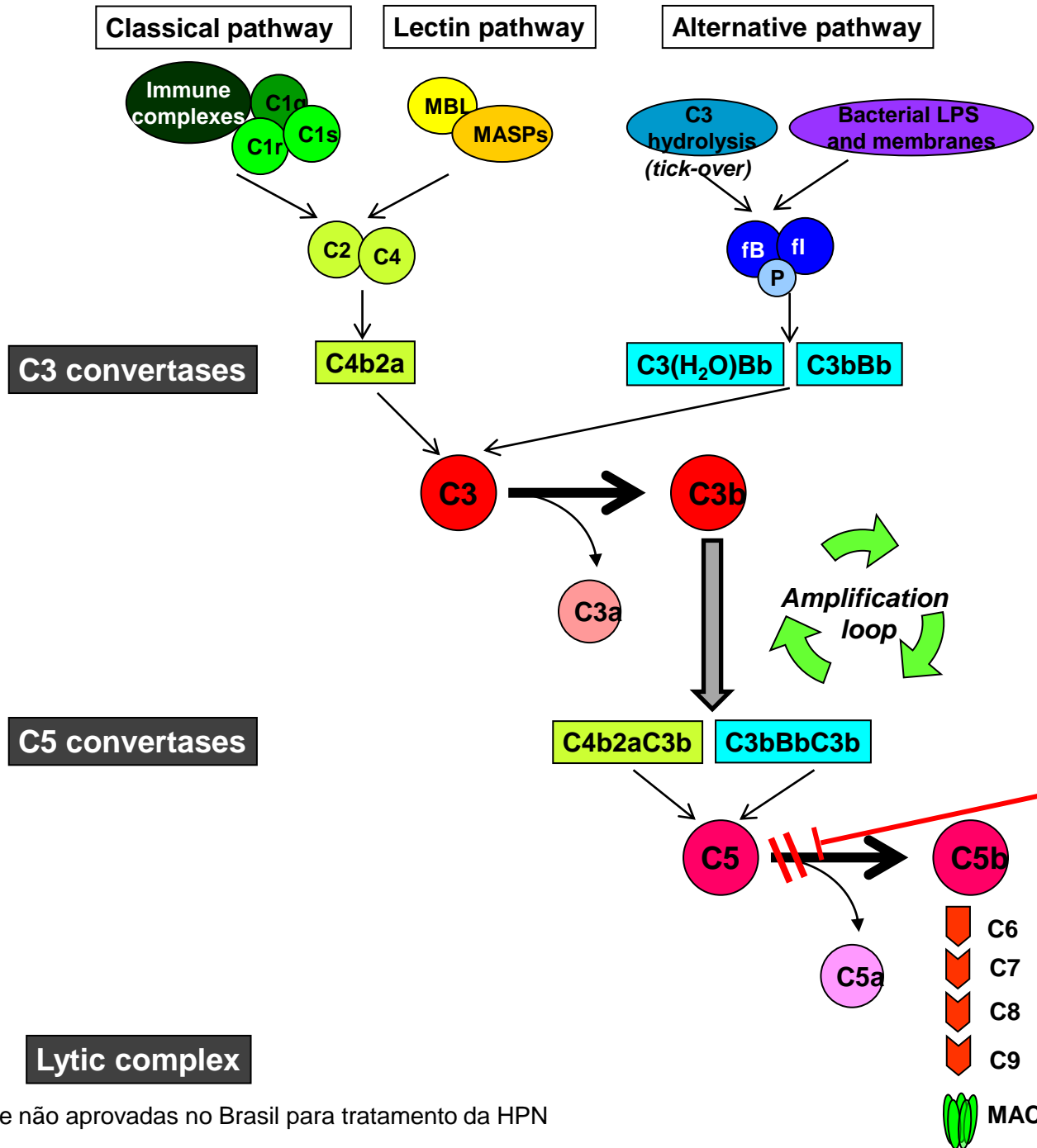
Meus pré-requisitos para participar destas atividades são o intercâmbio científico, a autonomia do pensamento científico, a independência de opinião e a liberdade de expressão, aspectos estes respeitados pela Novartis.

Esta apresentação reflete a minha opinião pessoal e não a do meu empregador ou universidade; é para efeitos de discussão científica independente. Por favor, consulte a informação de prescrição nas bulas aprovadas no Brasil.

Complement Pathway



- PNH is a rare, chronic hematological disorder characterized by complement-mediated intravascular haemolysis, thrombophilia and bone marrow failure
 - Caused by a somatic mutation in the *PIGA* gene, resulting in a lack of the GPI-anchored complement-regulating proteins CD55 and CD59, leading to intravascular haemolysis



*Terapias atualmente não aprovadas no Brasil para tratamento da HPN

Cenário no Brasil

2019 - Protocolos Clínicos e Diretrizes Terapêuticas (PCTD)

Critérios de Inclusão

- Clone > 10%, com atividade hemolítica DHL $\geq 1,5$
- Histórico de evento tromboembólico
- Anemia crônica Hb $\leq 7,0$ g/dL ou ≤ 10 g/dL com sintomas
- Hipertensão arterial pulmonar por eco PSAP > 35
- História de insuficiência renal – clearance ≤ 60 ml/min
- Gestaçã

Cenário no Brasil

2019 - Protocolos Clínicos e Diretrizes Terapêuticas (PCTD)

Critérios de Exclusão

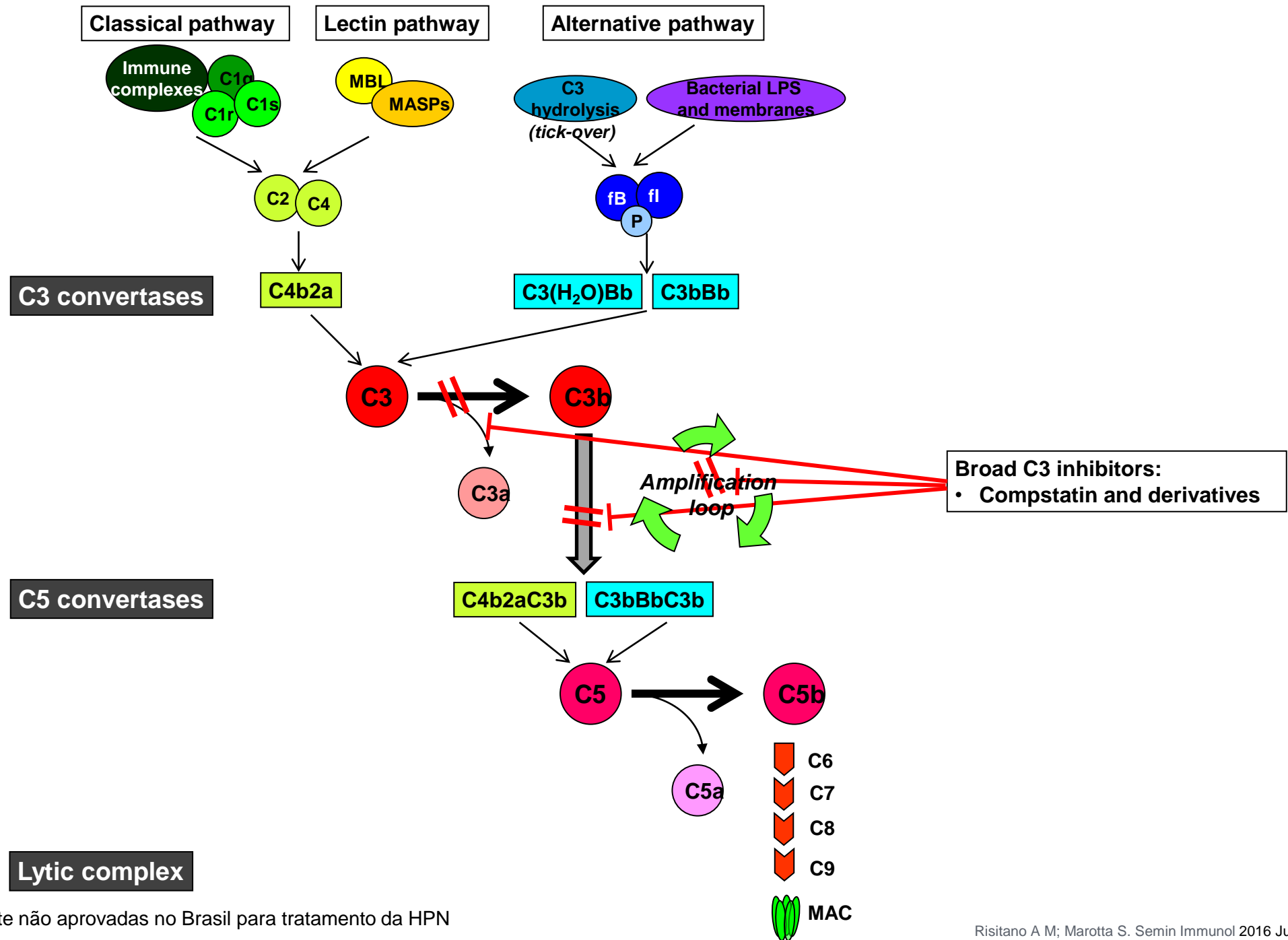
- HPN subclínica
- Concomitância com síndrome de falência medular grave ativa
- neutrófilos $< 500 / \mu\text{L}$, plaquetas $< 20.000 / \mu\text{L}$, reticulócitos $< 25.000 / \mu\text{L}$
- Clone $< 10\%$, sem atividade hemolítica DHL $\geq 1,5$

High Unmet Medical Need Despite

Multicenter, real-life, retrospective study of 160 patients with PNH treated with eculizumab¹

	Response category	Response criteria	Main goal of new therapy
21.3%	Complete response 11% BTH, 17% EVH	Hb ≥ 12 g/dL, transfusion independent	Decrease hospital visits
40.2%	Good response 8% BTH, 43% EVH	Hb 10 – 12 g/dL, transfusion independent	Normalization of Hb
26.8%	Partial response 21% BTH, 55% EVH	Hb 8 – 10 g/dL, occasional transfusions	Transfusion independency
11.8%	Minor response 27% BTH, 66% EVH	Hb < 8 g/dL, regular transfusions	Transfusion independency, limit hemachromatosis

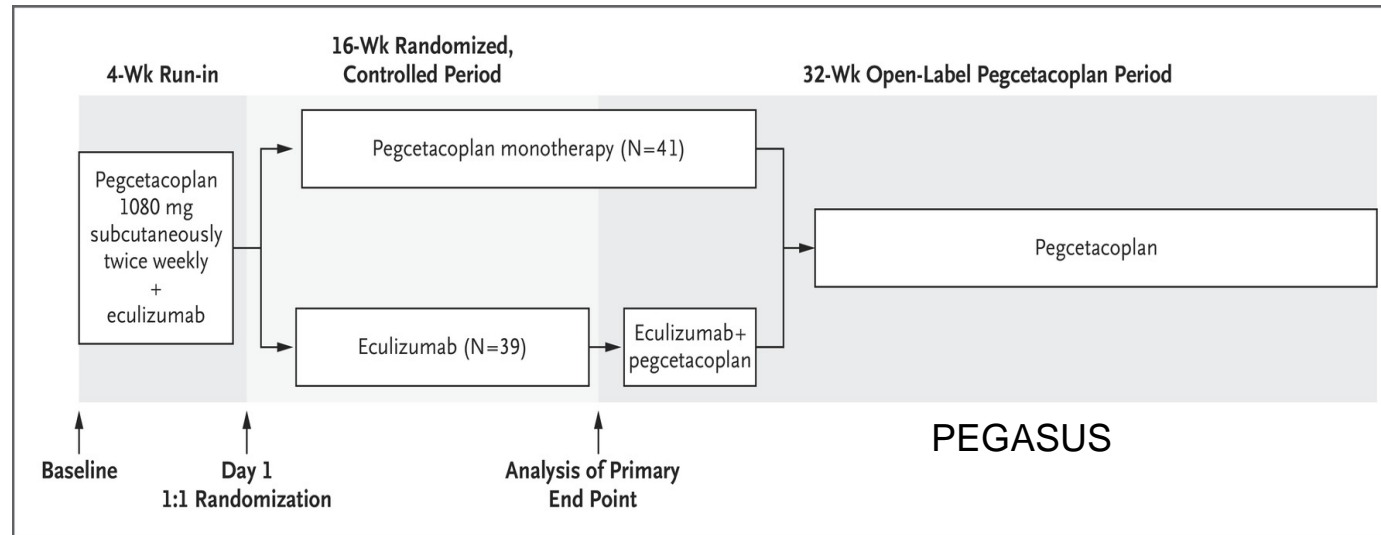
Hb, hemoglobin; RBCT, red blood cell transfusion; SoC, standard of care; BTH, breakthrough hemolysis; EVH, extravascular hemolysis



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Pegcetacoplan

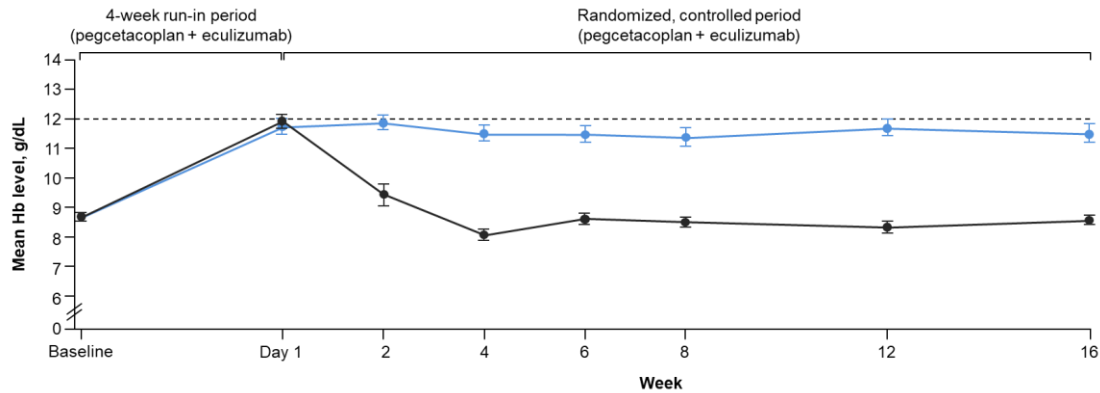
- Pegcetacoplan is a targeted C3 inhibitor.
- Pegcetacoplan targets the proximal complement pathway, binding to C3 and inhibiting its activation¹
- Pegcetacoplan is administered SC twice weekly¹⁻⁴



Pegcetacoplan

Pegcetacoplan (Phase III; PEGASUS)¹
 (80 patients who were anemic despite receiving eculizumab)

Difference at Week 16 = **+3.84 g/dL**
 (95% CI 2.33, 5.34; $P < 0.001$) between patients receiving
pegcetacoplan and **eculizumab***

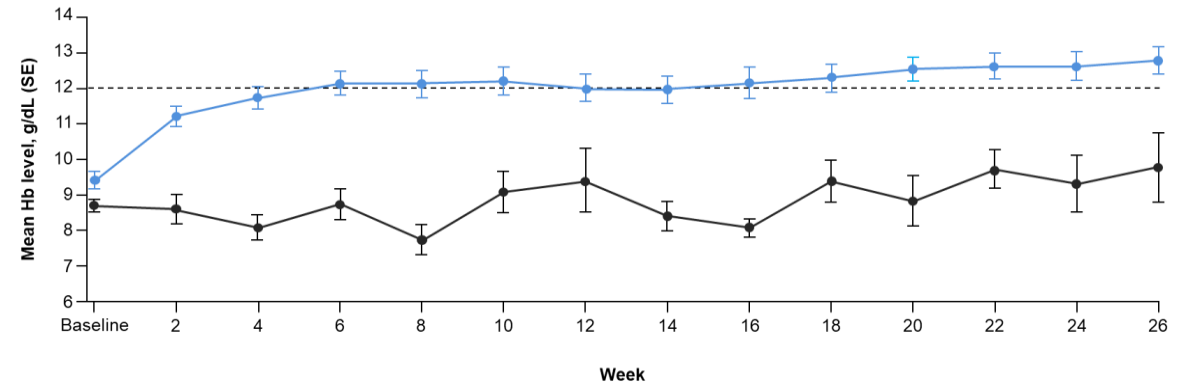


Patients with available data

Pegcetacoplan	41	40	40	40	39	37	38	37
Eculizumab	39	37	38	39	36	39	39	38

Pegcetacoplan (Phase III; PRINCE)²
 (53 complement inhibitor-naïve patients)

Difference at Week 26 = **+2.7 g/dL**
 (95% CI 1.0, 4.4; $P = 0.019$) between patients
 receiving **pegcetacoplan** and **supportive care only†**



Patients with available data

Pegcetacoplan	35	33	33	33	32	34	34	34	34	33	33	34	30
Control	18	17	17	16	13	9	8	8	8	8	7	6	7

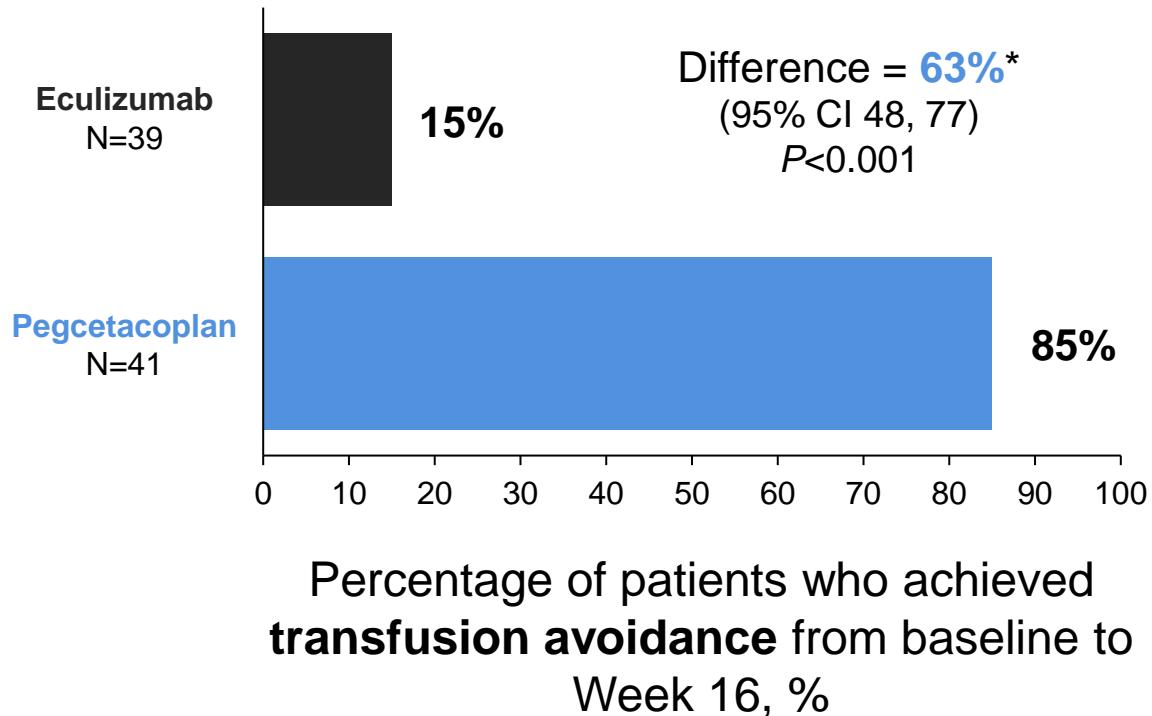
*Graph includes all available data for all patients, regardless of transfusion events. Week 8 data includes three patients in the pegcetacoplan group who had discontinued the trial and one patient with missing data; †Control group patients received supportive care (eg RBCTs, corticosteroids and supplements [iron, folate, vitamin B12])

CI, confidence interval; RBC, red blood cell transfusion; SE, standard error

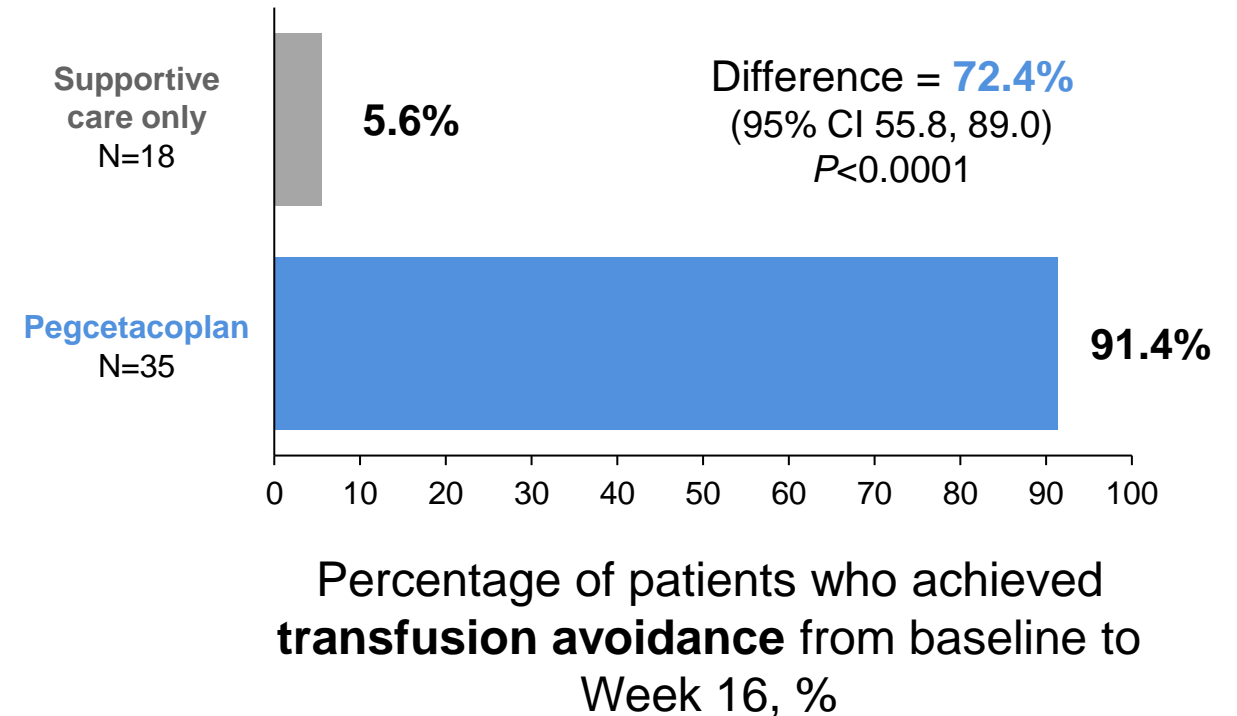
1. Hillmen P *et al.* *N Engl J Med* 2021;384:1028–37; 2. Wong RS *et al.* *Blood Adv* 2023 doi: <https://doi.org/10.1182/bloodadvances.2022009129>

Pegcetacoplan

Pegcetacoplan (Phase III; PEGASUS)¹
(80 patients who were anemic despite receiving
eculizumab)

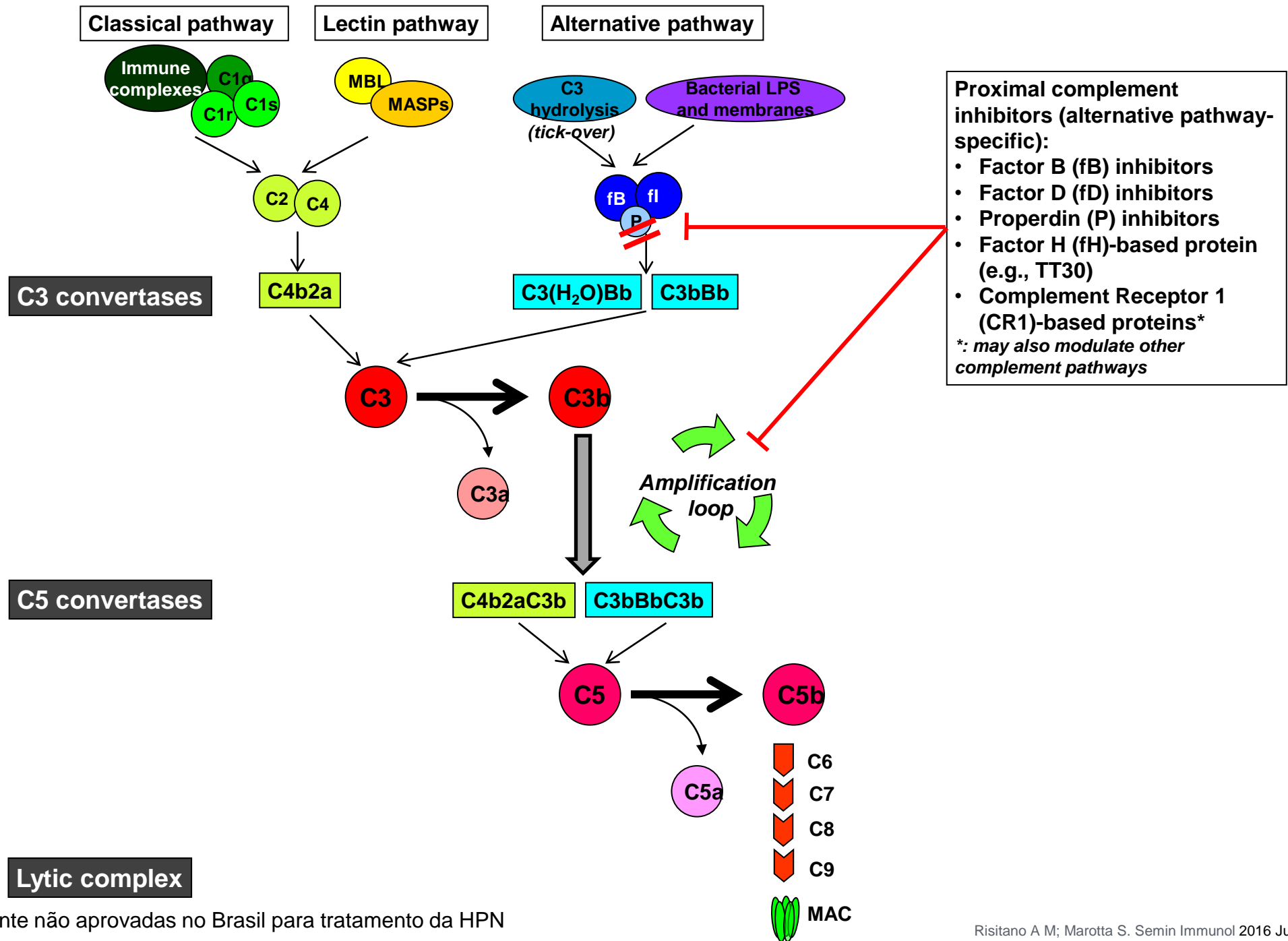


Pegcetacoplan (Phase III; PRINCE)²
(53 complement inhibitor-naïve patients)



*Pegcetacoplan was non-inferior to eculizumab. To control for a type 1 error, the proportion of patients who did not require a transfusion during the randomized controlled period were tested for non-inferiority if superiority was declared for the primary endpoint

1. Hillmen P *et al.* *N Engl J Med* 2021;384:1028–37; 2. Wong RS *et al.* *Blood Adv* 2023 doi: <https://doi.org/10.1182/bloodadvances.2022009129>



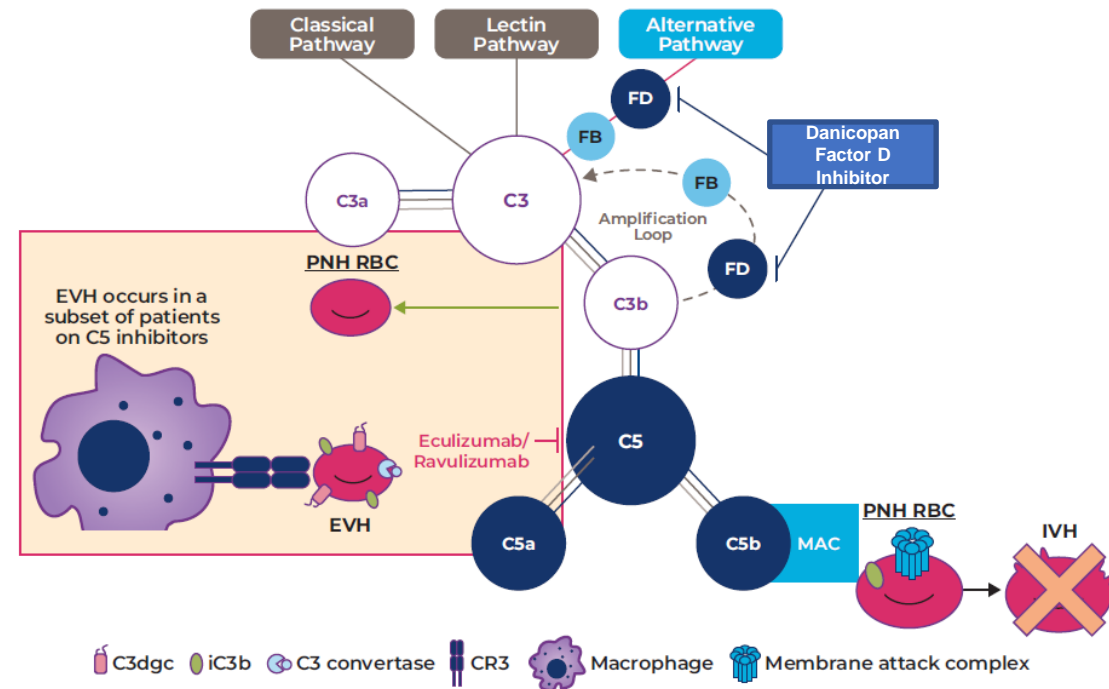
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Danicopan*

Introduction

- Danicopan (ALXN2040) is an investigational, first-in-class, oral factor D inhibitor targeting complement AP activity
 - The AP, unlike the classical and lectin pathways, turns over continuously at a low level in the blood via C3 hydrolysis¹
- Danicopan demonstrated predictable pharmacokinetics and a favorable benefit-risk profile in phase 2 trials in patients with PNH^{2,3}

IVH and EVH Associated Complement System in PNH



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1. Wiles JA, et al. *Curr Med Chem*. 2020. 2 Kulasekararaj AG, et al. *Blood*. 2021. 3. Risitano AM, et al. *Haematologica* 2021.

AP, alternative pathway; cs-EVH, clinically significant EVH; EVH, extravascular hemolysis; FB, factor B; FD, factor D; IVH, intravascular hemolysis; MAC, membrane attack complex; PNH, paroxysmal nocturnal hemoglobinuria; RBC, red blood cell.

Methods

Study Design

- Ongoing, phase 3, randomized, double-blind, placebo-controlled clinical trial

Dosing

- Initial danicopan dose of 150 mg TID
 - Escalation to 200 mg TID permitted based on clinical response at the investigator's discretion

Endpoints

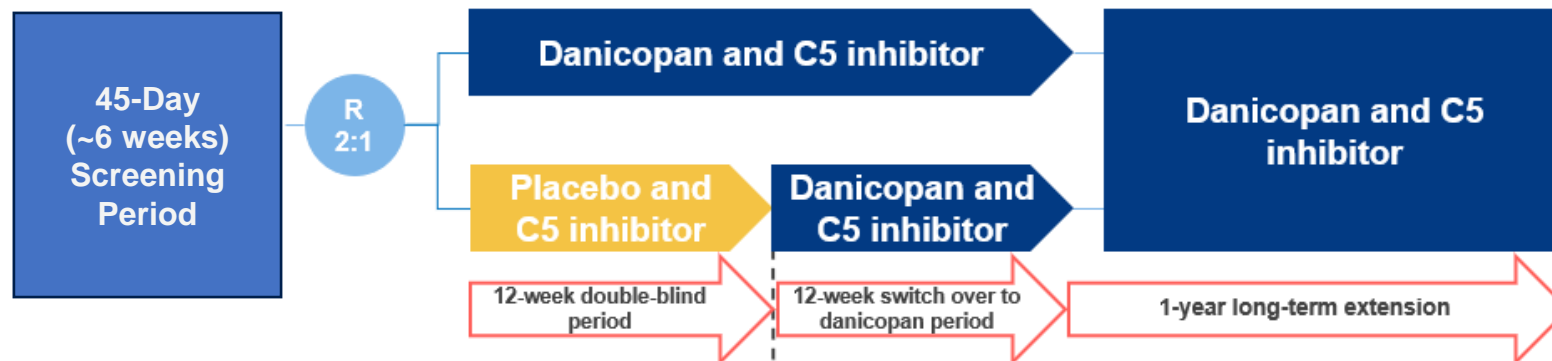
- **Primary endpoint:** Change in Hgb from baseline at week 12
- **Key secondary endpoints at week 12:**
 - Proportion of participants with Hgb increase of ≥ 2 g/dL without transfusion
 - Proportion of participants with transfusion avoidance
 - Change from baseline in FACIT-F score and ARC
- **Safety:** TEAEs and laboratory abnormalities

Key Inclusion Criteria

- Age ≥ 18 years with PNH and cs-EVH, despite receiving stable eculizumab or ravulizumab treatment for ≥ 6 months

Data Analysis

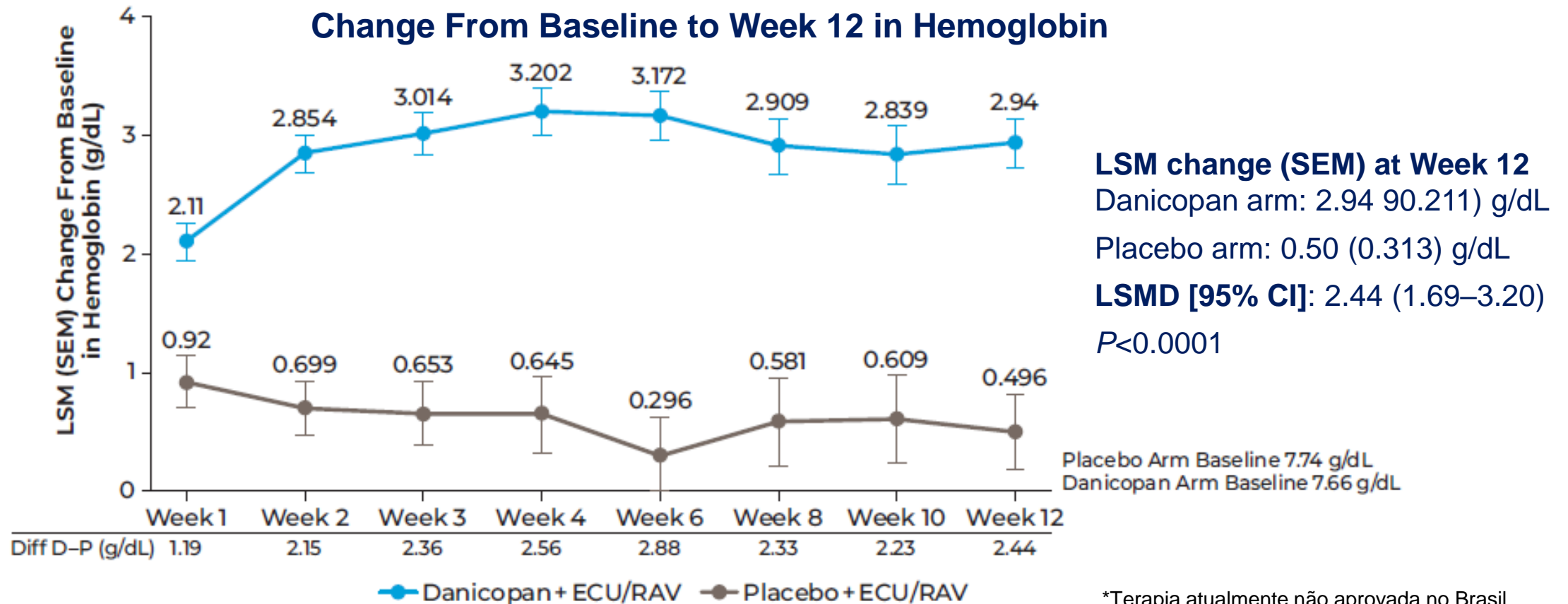
- Statistical significance:
 - 2-sided 0.018 for primary endpoint
 - 2-sided 0.042 for key secondary endpoints
- Safety analysis performed using data from all participants who took ≥ 1 dose of the study drug
 - Data analyzed descriptively



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Methods

- Danicopan* as an add-on treatment to eculizumab or ravulizumab resulted in a statistically significant increase in Hb from baseline to week 12 vs placebo as add-on to eculizumab or ravulizumab

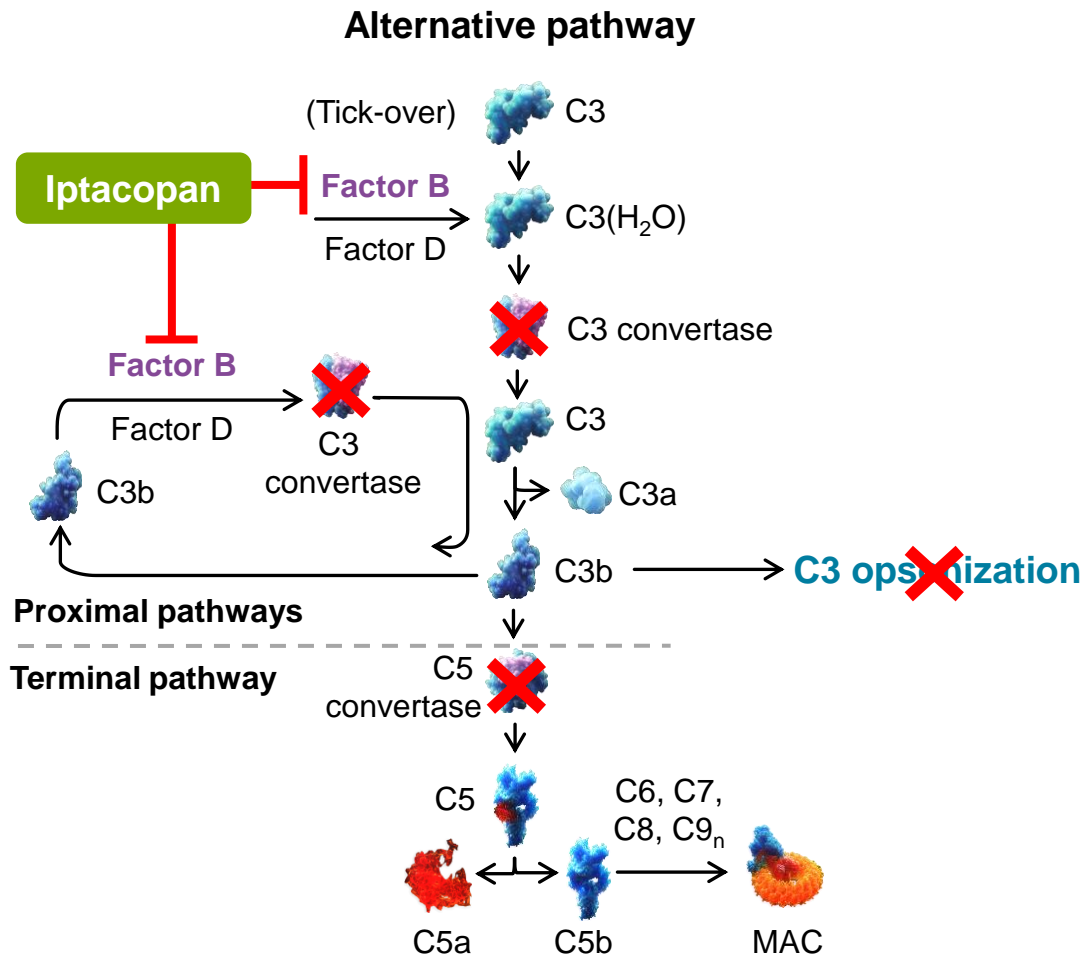


P<0.0001 for all data points.
 D, danicopan; Diff, difference; ECU, eculizumab; Hgb, hemoglobin; LSM, least squares mean; P, placebo; RAV, ravulizumab; SE, standard error.

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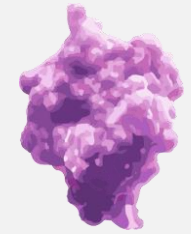
Iptacopan*

First-in-class, oral, selective factor B¹



Iptacopan binds to the **active site** of factor B, **inhibiting** the activity of **C3 convertase**¹

Iptacopan



Factor B

Iptacopan **controlled intra- and extravascular hemolysis** in 10 patients with a suboptimal response to eculizumab, leading to **transfusion independence** and an **improved quality of life**²

THE LANCET
Haematology

ARTICLES | VOLUME 8, ISSUE 5, E344-E354, MAY 2021

Addition of iptacopan, an oral factor B inhibitor, to eculizumab in patients with paroxysmal nocturnal haemoglobinuria and active haemolysis: an open-label, single-arm, phase 2, proof-of-concept trial

Prof Antonio M Risitano, MD, Prof Alexander Röth, MD, Juliette Soret, MD, Camilla Frieri, MD, Flore Sicre de Fontbrune, MD, Luana Marano, MD, Ferras Alashkar, MD, Lina Benajiba, MD, Serena Marotta, MD, Izabela Rozenberg, PhD, Julie Milojević, PhD, Peter End, PhD, Prasanna K Nidamarthy, MSc, Guido Junge, MD, Prof Régis Peffault de Latour, MD • Show less

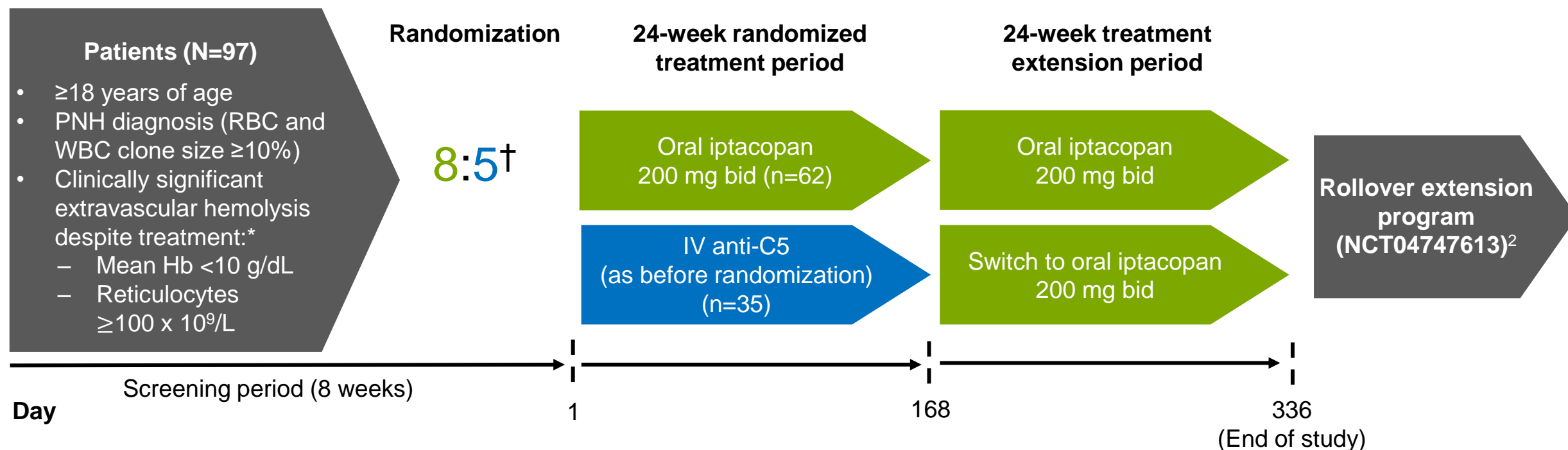
Published: March 22, 2021 • DOI: [https://doi.org/10.1016/S2352-3026\(21\)00028-4](https://doi.org/10.1016/S2352-3026(21)00028-4) • Check for updates

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1. Schubart A et al. Proc Natl Acad Sci USA 2019;116:7926–31; 2. Risitano AM et al. Lancet Haematol 2021;8:e344–54

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APPLY-PNH is an open-label, randomized, multicenter, Phase III trial investigating iptacopan monotherapy in PNH patients with residual anemia despite anti-C5 therapy (NCT04558918)¹



*With a stable regimen of eculizumab or ravulizumab for at least 6 months preceding randomization; [†]Stratified by prior anti-C5 treatment and RBC transfusions in the preceding 6 months

bid, twice daily; Hb, hemoglobin; IV, intravenous; RBC, red blood cell; WBC, white blood cell

1. ClinicalTrials.gov. NCT04558918. Available at: <https://clinicaltrials.gov/ct2/show/NCT04558918> (accessed April 2023);

2. ClinicalTrials.gov. NCT04747613. Available at: <https://clinicaltrials.gov/ct2/show/NCT04747613> (accessed April 2023)

N Engl J Med . 2024 Mar 14;390(11):994-1008

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APPLY-PNH is a superiority trial with two primary endpoints

Primary

- Hematological response defined as an **increase from baseline in Hb of ≥ 2 g/dL*** **in the absence of RBC transfusions[†]**
- Hematological response defined as **Hb ≥ 12 g/dL*** **in the absence of RBC transfusions[†]**

Secondary

- **Transfusion avoidance[†]**
- **Change from baseline:***
 - Hb levels[‡]
 - FACIT-Fatigue scores
 - Absolute reticulocyte count
 - LDH levels
- Occurrences of **clinical breakthrough hemolysis and MAVEs[§]**
- **Safety[§]**

- A multiple testing procedure adjusting for multiplicity was used to determine superiority^{1,2}

*Assessed between Days 126 and 168; [†]Between Days 14 and 168 and neither meeting the criteria for administration of an RBC transfusion nor receiving an RBC transfusion between Days 14 and 168; [‡]Excluding values within 30 days of RBC transfusion; [§]Throughout the study

The overall study Type I error rate was controlled at the one-sided 2.5% level. All presented *P* values are two-sided and unadjusted

FACIT, Functional Assessment of Chronic Illness Therapy; LDH, lactate dehydrogenase; MAVE, major adverse vascular event

1. Bretz F *et al. Stat Med* 2009;28:586–604; 2. Bretz F *et al. Stat Med* 2011;30:1489–501

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Demographics and disease characteristics at baseline were generally balanced between arms

	Iptacopan* 200 mg bid N=62	Anti-C5 N=35	Overall N=97
Mean age, years (SD)	51.7 (16.9)	49.8 (16.7)	51.0 (16.8)
Female, n (%)	43 (69.4)	24 (68.6)	67 (69.1)
Time since diagnosis, years (SD)	11.9 (9.8)	13.6 (10.9)	12.5 (10.2)
Anti-C5 therapy			
Eculizumab,* n (%)	40 (64.5)	23 (65.7)	63 (64.9)
Ravulizumab,* n (%)	22 (35.5)	12 (34.3)	34 (35.1)
Mean duration, years (SD)	3.8 (3.5)	4.2 (3.9)	4.0 (3.6)
Received RBC transfusions,* n (%)	35 (56.5)	21 (60.0)	56 (57.7)
Mean baseline Hb, g/dL (SD) [range]	8.9 (0.7) [6.8–10.0]	8.9 (0.9) [6.2–9.9]	8.9 (0.8) [6.2–10.0]
Mean baseline LDH, U/L (SD) [range]	269.1 (70.1) [150–539]	272.7 (84.8) [133–562]	270.4 (75.3) [133–562]
Baseline LDH >1.5 x ULN, n (%)	4 (6.5)	3 (8.6)	7 (7.2)
Mean baseline absolute reticulocyte count, 10 ⁹ /L (SD) [range]	193.2 (83.6) [51–563]	190.6 (80.9) [90–412]	192.3 (82.3) [51–563]

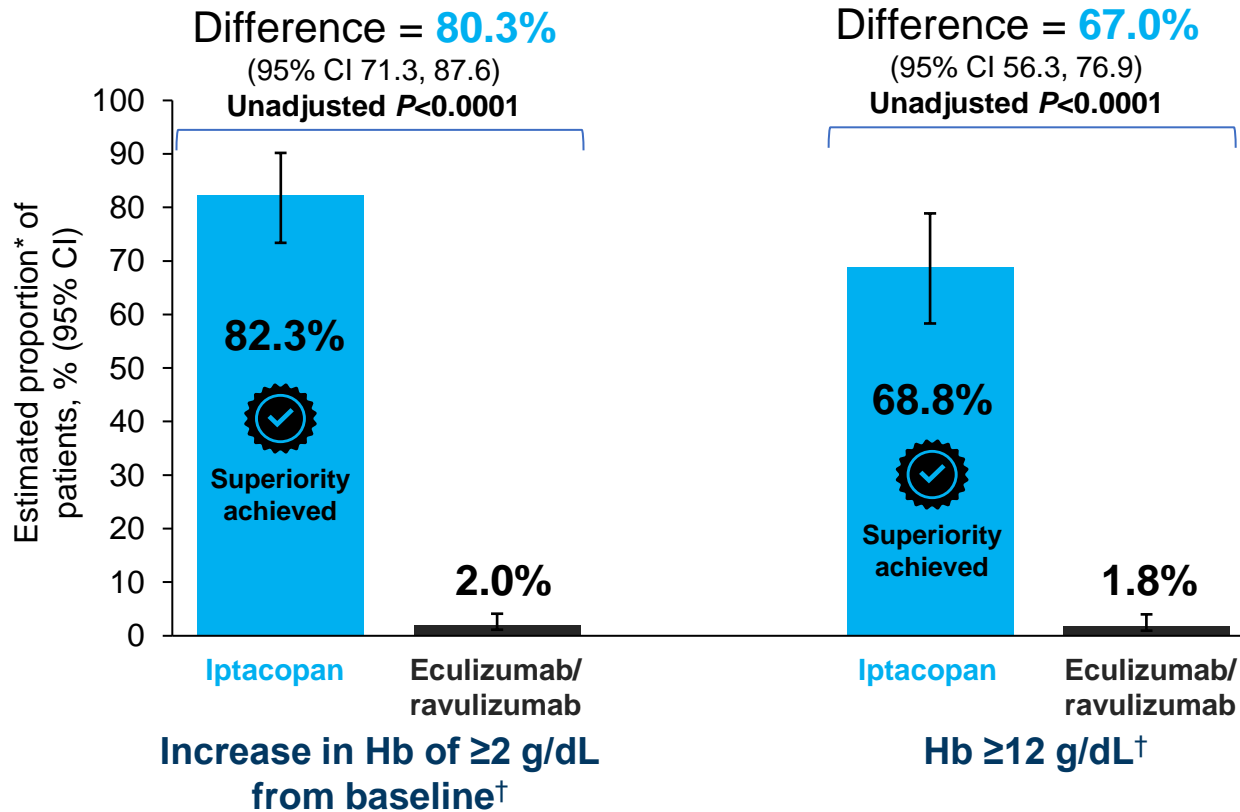
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*In the 6 months prior to randomization
SD, standard deviation; ULN, upper limit of normal

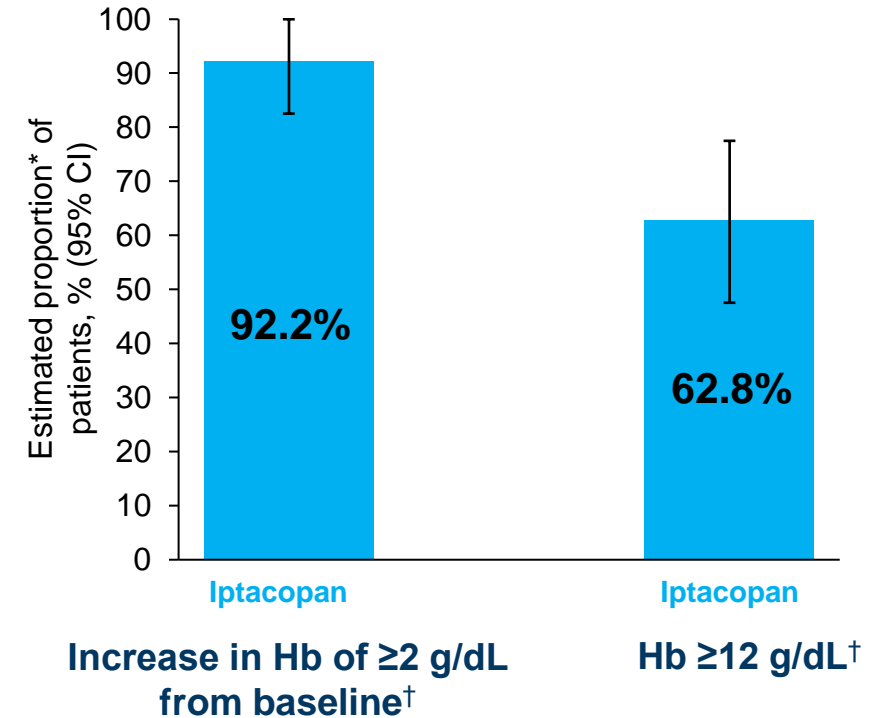
In Phase III trials, inhibition of factor B with oral iptacopan* monotherapy led to clinically meaningful Hb improvements in the absence of RBCTs



Iptacopan (Phase III; APPLY-PNH)¹
(97 patients who were anemic despite receiving eculizumab/ravulizumab)



Iptacopan (Phase III; APPOINT-PNH)²
(40 complement inhibitor-naïve patients)



*Terapia atualmente não aprovada no Brasil para tratamento da HPN

^{*}Estimated proportions were calculated to reflect the population average probability of a patient meeting the endpoint criteria; [†]Assessed between Days 126 and 168 in the absence of (or meeting the criteria for) RBCTs between Days 14 and 168

1. Peffault de Latour R *et al. Blood* 2022;140(Suppl 2):LBA-2; 2. Risitano AM *et al. European Society for Blood and Marrow Transplantation (EBMT)*. Paris, France, 23–26 April 2023; abstract OS12-06

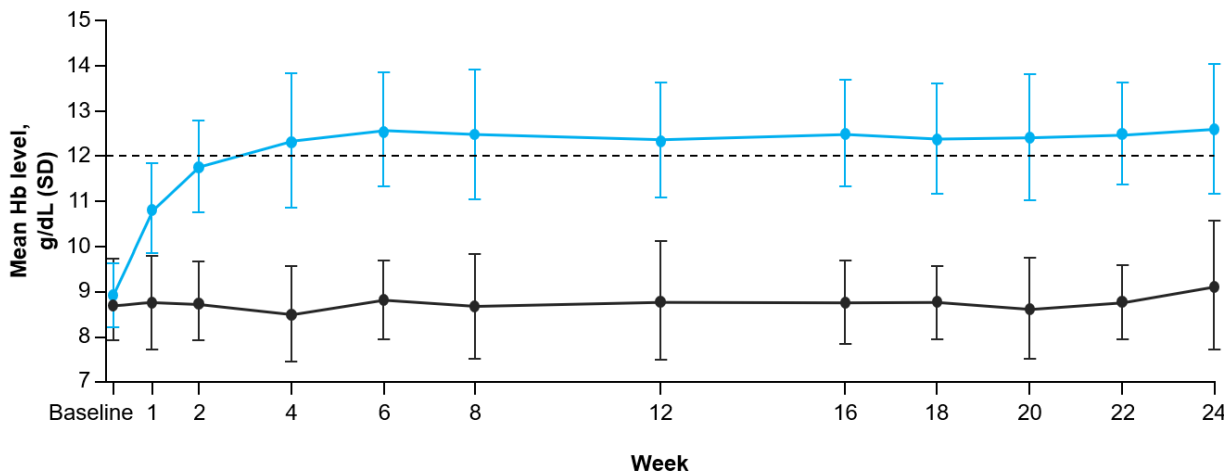


In Phase III trials, inhibition of factor B with oral iptacopan monotherapy led to clinically meaningful Hb improvements in the absence of RBCTs

Superiority achieved



Iptacopan (Phase III; APPLY-PNH)¹
(97 patients who were anemic despite receiving eculizumab/ravulizumab)

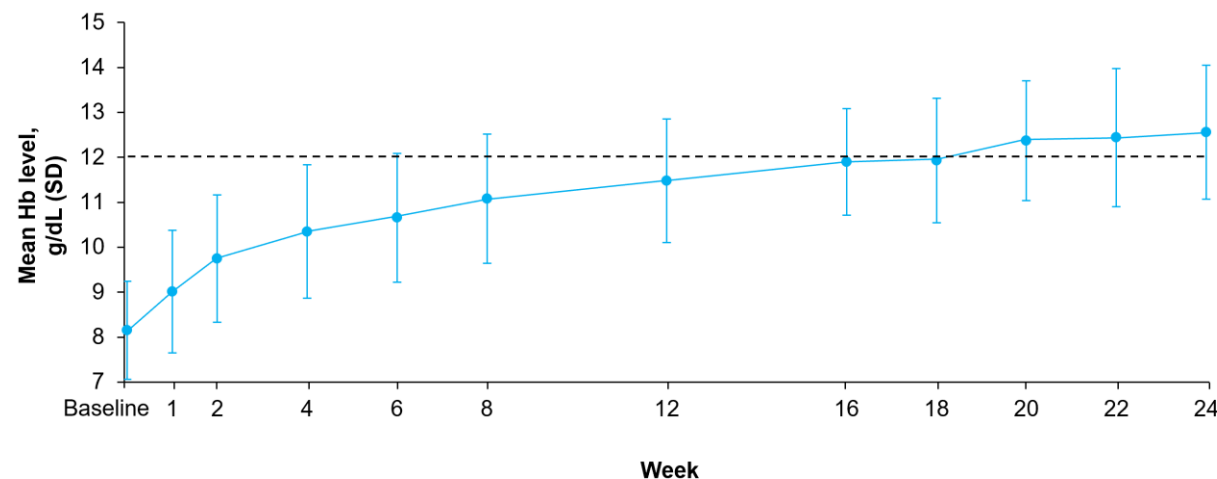


Patients with available data*†

Iptacopan	62	61	59	60	59	59	60	59	59	59	57	61
Ecu/ravu	35	29	27	32	32	31	33	28	34	32	32	34

Iptacopan was superior to eculizumab/ravulizumab for **adjusted mean change in Hb from baseline[§]**, with a difference of **+3.63 g/dL** (95% CI +3.18, +4.08; **unadjusted $P < 0.0001$**) between treatment arms

Iptacopan (Phase III; APPOINT-PNH)²
(40 complement inhibitor-naïve patients)



Patients with available data*‡

Iptacopan	40	39	39	36	35	37	36	32	30	34	32	39
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Iptacopan led to an **adjusted mean change in Hb from baseline[§]** of **+4.28 g/dL** (95% CI +3.87, +4.70)

*Terapia atualmente não aprovada no Brasil para tratamento da HPN

*Includes post-transfusion data; †2/62 patients in the iptacopan arm and 21/35 patients in the eculizumab/ravulizumab arm had RBCTs between Days 14 and 168; ‡RBCTs were received by 5/40 patients between Days 1 and 13 and by 0/40 patients between Days 14 and 168; §Assessed between Days 126 and 168 in the absence of (or meeting the criteria for) RBCTs between Days 14 and 168. Ecu, eculizumab; ravu, ravulizumab; SD, standard deviation

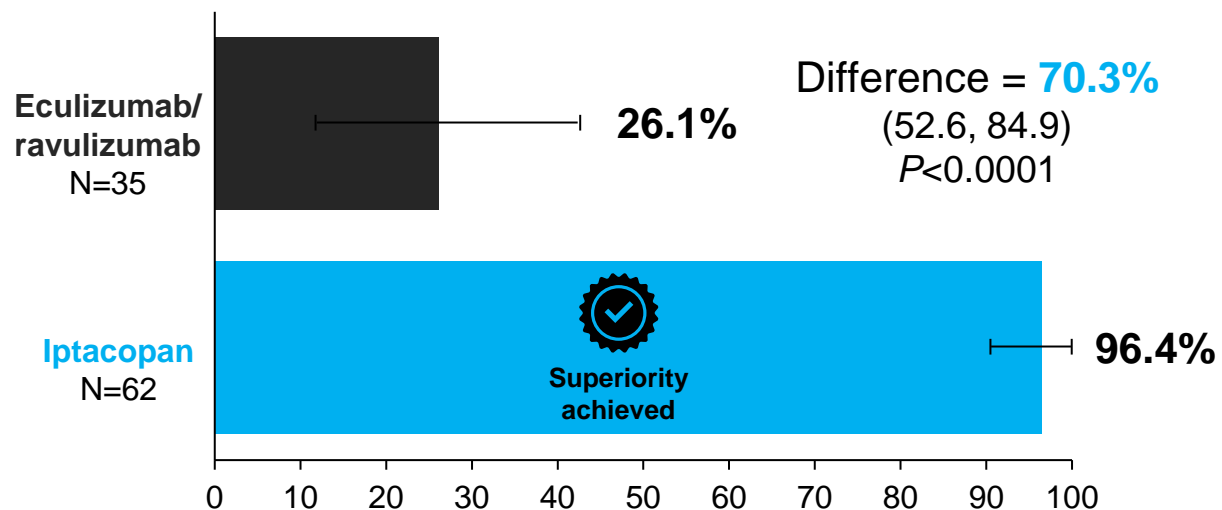
1. Peffault de Latour R et al. Blood 2022;140(Suppl 2):LBA-2; 2. Risitano AM et al. European Society for Blood and Marrow Transplantation (EBMT). Paris, France, 23–26 April 2023; abstract OS12-06

The factor B inhibitor iptacopan* led to transfusion independence in nearly all patients treated in Phase III trials



Iptacopan (Phase III; APPLY-PNH)¹

(97 patients who were anemic despite receiving eculizumab/ravulizumab)

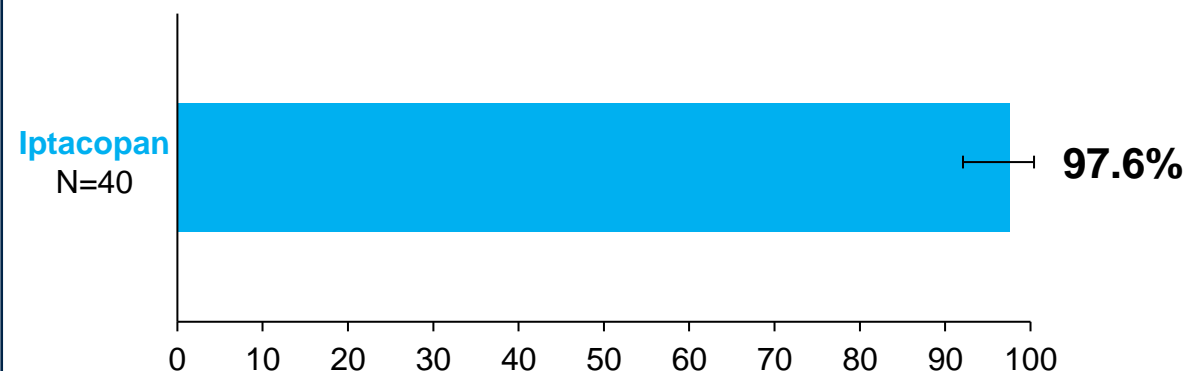


Estimated proportion* of patients who achieved **transfusion avoidance**^{†,‡} between Days 14 and 168, % (95% CI)

Iptacopan (Phase III; APPOINT-PNH)²

(40 complement inhibitor-naïve patients)

None of the 40 patients enrolled in APPOINT-PNH required a RBCT



Estimated proportion* of patients who achieved **transfusion avoidance**[†] between Days 14 and 168, % (95% CI)

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*Estimated proportions were calculated to reflect the population average probability of a patient meeting the endpoint criteria; †Defined as the proportion of patients who remained free from (and did not meet the criteria for) transfusions between Days 14 and 168; ‡The prespecified methodology for handling of missing data may have underestimated transfusion avoidance in the eculizumab/ravulizumab arm, so a *post hoc* sensitivity analysis was conducted using a different approach. In this analysis, estimated proportions (95% CI) were 96.7% (91.3, 100.0) versus 38.9% (23.1, 55.8) for iptacopan and eculizumab/ravulizumab, respectively (P<0.0001)

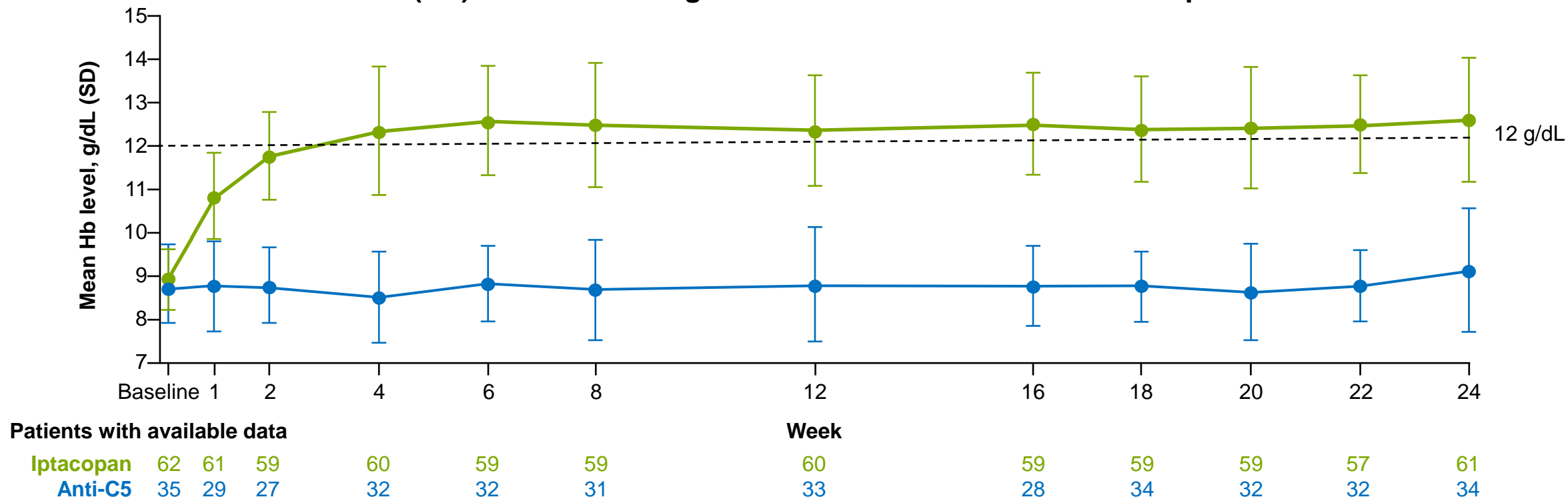
1. Peffault de Latour R *et al. Blood* 2022;140(Suppl 2):LBA-2; 2. Risitano AM *et al. European Society for Blood and Marrow Transplantation (EBMT). Paris, France, 23–26 April 2023; abstract OS12-06*



Iptacopan* monotherapy was superior to anti-C5 at increasing Hb level from baseline

Adjusted mean Hb change from baseline* (95% CI) was **+3.59 (3.32, 3.86) g/dL for iptacopan** vs **-0.04 (-0.42, 0.35) g/dL for anti-C5**, with a **difference of +3.63 (3.18, 4.08) g/dL ($P < 0.0001$)[†]**

Mean Hb (SD) over time during the 24-week randomized treatment period[‡]



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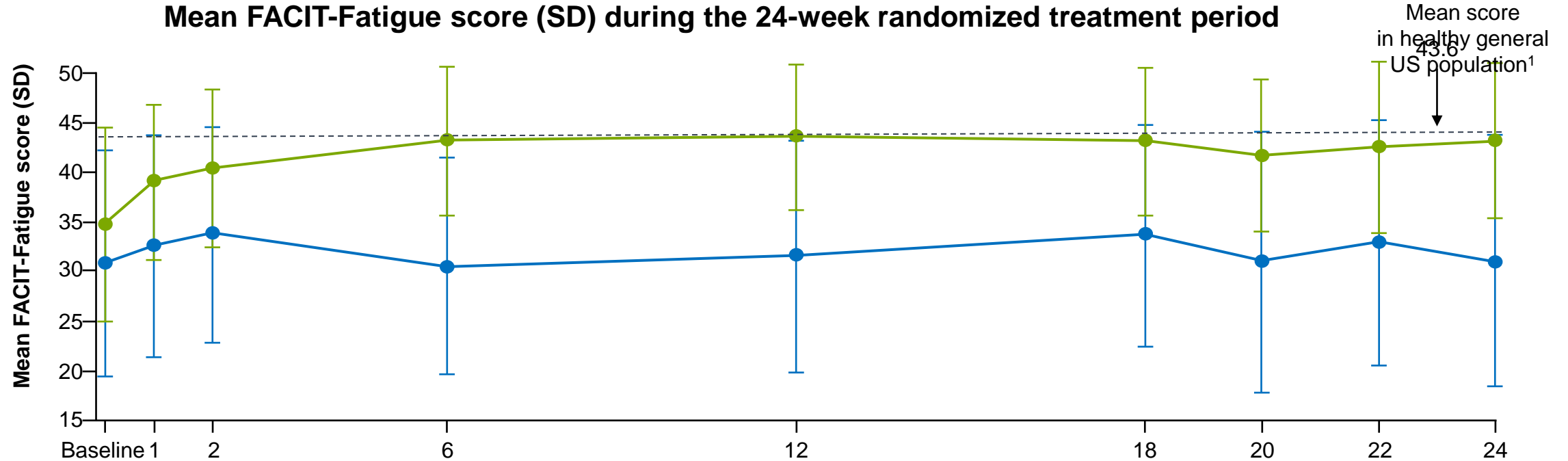
N Engl J Med . 2024 Mar 14;390(11):994-1008

^{*}Between Days 126 and 168 (excluding values within 30 days of RBC transfusion); [†]A repeated measures model, adjusting for covariates including baseline Hb, was used for comparisons between the treatment arms. *P* value is two-sided and unadjusted; [‡]Includes post-transfusion data. 2/62 patients in the iptacopan arm and 21/35 patients in the anti-C5 arm had RBC transfusions between Days 14 and 168. BL, baseline; Wk, week

Iptacopan* monotherapy was superior to anti-C5 at reducing patient-reported fatigue from baseline

Adjusted mean change from baseline* in FACIT-Fatigue score (95% CI) was **+8.59 (6.72, 10.47)** for **iptacopan** vs **+0.31 (-2.20, 2.81)** for **anti-C5**, with a **difference of +8.29 (5.28, 11.29) (P<0.0001†)**

Mean FACIT-Fatigue score (SD) during the 24-week randomized treatment period



Patients with available data

	Baseline	1	2	6	12	18	20	22	24
Iptacopan	62	60	57	61	57	58	59	56	60
Anti-C5	33	27	28	32	29	29	28	28	30

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N Engl J Med . 2024 Mar 14;390(11):994-1008

*Between Days 126 and 168; †A repeated measures model, adjusting for covariates including baseline FACIT-Fatigue score, was used for comparisons between the treatment arms. P value is two-sided and unadjusted

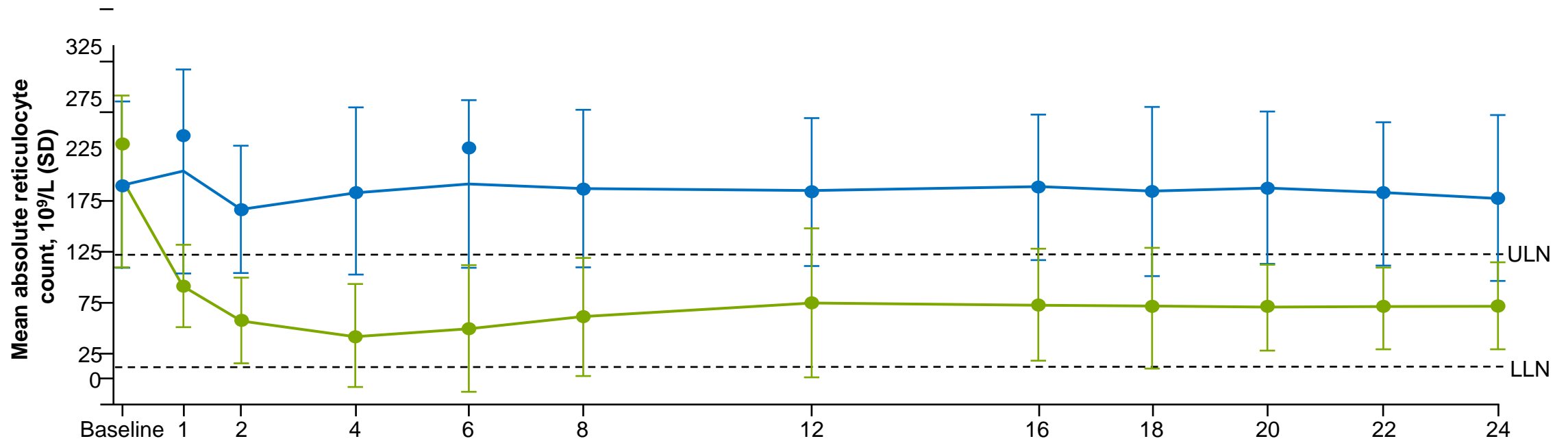
1. Cella D *et al. Cancer* 2002;94:528–38

Iptacopan* monotherapy was superior to anti-C5 at reducing absolute reticulocyte count from baseline

Adjusted mean change from baseline* in absolute reticulocyte count (95% CI) was

-115.89 (-126.49, -105.30) x 10⁹/L for iptacopan vs **+0.37 (-13.03, 13.77) x 10⁹/L for anti-C5**, with a **difference of -116.26 (-132.17, -100.36) x 10⁹/L (P<0.0001[†])**

Mean absolute reticulocyte count (SD) during the 24-week randomized treatment period



Patients with available data

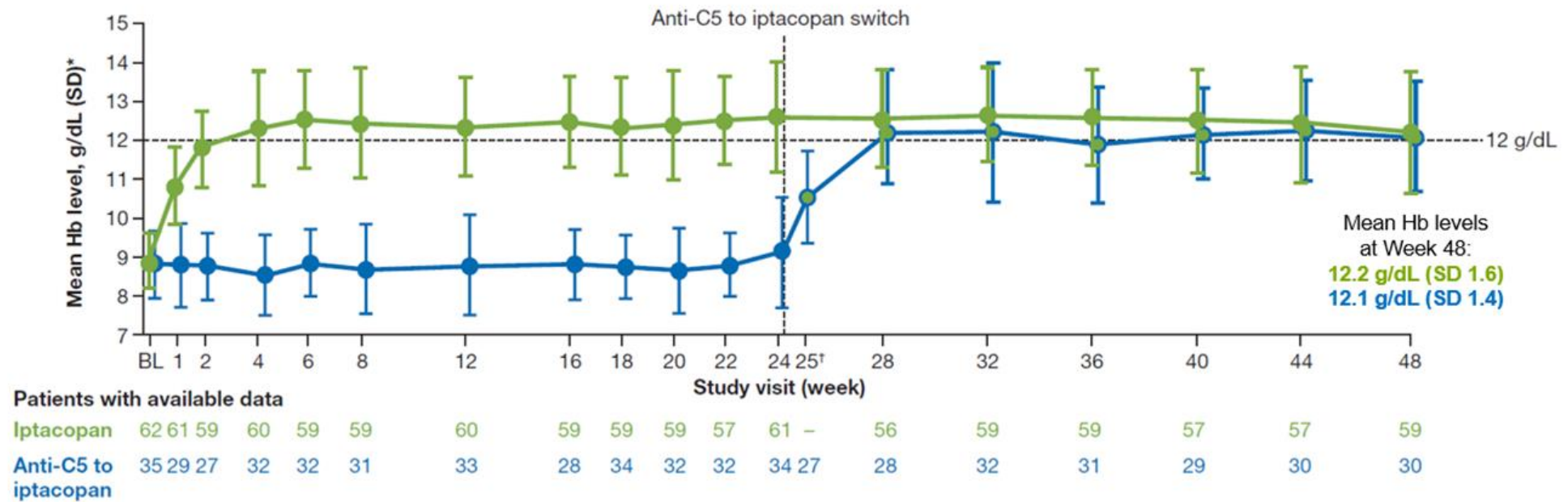
	Baseline	1	2	4	6	8	12	16	18	20	22	24
Iptacopan	62	61	58	60	59	59	60	56	59	56	56	58
Anti-C5	35	28	27	32	31	31	33	27	34	32	32	34

*Terapia atualmente não aprovada no Brasil para tratamento da HPN

N Engl J Med . 2024 Mar 14;390(11):994-1008

[†]Between Days 126 and 168; [†]A repeated measures model, adjusting for covariates including baseline absolute reticulocyte count, was used for comparisons between the treatment arms. P value is two-sided and unadjusted. LLN, lower limit of normal

Cross over to iptacopan from C5i



Mean Hb level over time during the entire 48-week treatment period of APPLY-PNH

There was no significant difference between iptacopan monotherapy and anti-C5 for the annualized rate of MAVEs

	Arm	n/N*	Adjusted annual rate, % (95% CI)	Rate ratio (95% CI)†	P value†
Rate of MAVEs	Iptacopan	1/62	0.03 (0.00, 0.25)	Not estimable	0.3173
	Anti-C5	0/35	0		

- Serious TEAE of **transient ischemic attack**, considered by the investigator to be **unrelated to iptacopan**
 - The patient had a concomitant serious TEAE of sick sinus syndrome and is **continuing** to receive **iptacopan** treatment

*Terapia atualmente não aprovada no Brasil para tratamento da HPN

Iptacopan* monotherapy was well tolerated and had a favorable safety profile

Most common TEAEs (≥4 patients in either arm)*

n (%)	Iptacopan 200 mg bid N=62	Anti-C5 N=35
Any TEAE	51 (82.3)	28 (80.0)
Mild / Moderate / Severe, %	32.3 / 45.2 / 4.8	37.1 / 34.3 / 8.6
Headache	10 (16.1)	1 (2.9)
Diarrhea	9 (14.5)	2 (5.7)
Nasopharyngitis	7 (11.3)	2 (5.7)
Nausea	6 (9.7)	1 (2.9)
COVID-19	5 (8.1)	9 (25.7)
Urinary tract infection	5 (8.1)	1 (2.9)
Arthralgia	5 (8.1)	1 (2.9)
Abdominal pain	4 (6.5)	1 (2.9)
Increased blood LDH	4 (6.5)	3 (8.6)
Dizziness	4 (6.5)	0
Breakthrough hemolysis	2 (3.2)	6 (17.1)

Serious TEAEs: 9.7% vs 14.3%

Hemolysis serious TEAEs:

- Anti-C5: breakthrough hemolysis (n=1) and extravascular hemolysis (n=1)
- Iptacopan: none

No serious infections caused by *Neisseria meningitidis*, *Streptococcus pneumoniae* or *Haemophilus influenzae*

No patients discontinued study treatment, except one because of pregnancy (iptacopan)

No deaths

*Organized by descending frequency in the iptacopan arm

*Terapia atualmente não aprovada no Brasil para tratamento da HPN

Conclusions

- Proximal Complement Inhibitors are finally here.
- Oral iptacopan monotherapy led to a significant majority of patients achieving clinically meaningful Hb increases and Hb ≥ 12 g/dL, associated with a higher rate of transfusion independence and reduced patient-reported fatigue, compared with anti-C5
- Iptacopan monotherapy achieved resolution of extravascular hemolysis and maintenance of intravascular hemolysis control
- The therapy was well tolerated, with a favorable safety profile and no serious breakthrough hemolysis
- Iptacopan monotherapy may represent a practice-changing, oral, outpatient treatment, which may become a preferred treatment option for patients with hemolytic PNH who have an inadequate response to IV anti-C5 therapy

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