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ORAL ABSTRACTS

508.BONE MARROW FAILURE: ACQUIRED

Factor B Inhibition with Oral Iptacopan Monotherapy Demonstrates Sustained Long-Term Efficacy and Safety in Anti-C5-Treated Patients (pts) with Paroxysmal Nocturnal Hemoglobinuria (PNH) and Persistent Anemia: Final 48-Week Results from the Multicenter, Phase III APPLY-PNH Trial

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Background: Iptacopan is the first oral complement inhibitor that acts proximally in the complement system to target factor B in the alternative pathway. Iptacopan has shown efficacy/safety in PNH pts with persistent anemia despite anti-C5 therapy and complement inhibitor-naive pts. In the Phase III APPLY-PNH trial (NCT04558918), iptacopan monotherapy led to clinically meaningful hemoglobin (Hb) increases and normal/near-normal Hb levels in a majority of pts, transfusion avoidance and improved pt-reported fatigue, showing superiority vs C5 inhibitors at Week (Wk) 24.

Aim: We report the final APPLY-PNH data after a 24-wk extension period in which all pts received iptacopan monotherapy (study completion: 6 March 2023).

Methods: Adult PNH pts (mean Hb <10 g/dL, receiving anti-C5 therapy for ≥6 months) were randomized to receive iptacopan 200 mg twice daily or continue their anti-C5 regimen for 24 wks. Pts could then opt to enter an extension period; pts in the iptacopan arm received iptacopan for another 24 wks and pts who had been receiving anti-C5 switched to iptacopan monotherapy.

Results: In the extension period, 95 pts received iptacopan: 61/62 in the iptacopan arm (1 discontinued iptacopan in the randomized period because of pregnancy) and 34/35 in the anti-C5-to-iptacopan arm (1 did not enter the extension period [investigator's decision]). In the iptacopan arm, the improvements at 24 wks were sustained at 48 wks, with maintenance of increased Hb, normal/near-normal mean Hb levels (Figure), improved Functional Assessment of Chronic Illness Therapy - Fatigue (FACIT-F) scores, decreased absolute reticulocyte counts (ARCs) and transfusion avoidance (Table). Pts who switched from anti-C5 to iptacopan had rapid changes in Hb, FACIT-F and ARC, achieving comparable improvements to the iptacopan arm. Mean Hb levels at Wk 48 were 12.2 and 12.1 g/dL in the iptacopan and anti-C5-to-iptacopan arms, respectively (standard deviations 1.6 and 1.4). At Wk 48, the adjusted mean change from baseline in the iptacopan arm was +3.35 g/dL for Hb, +9.80 FACIT-F points and -106.26×10^9/L for ARC. In the anti-C5-to-iptacopan arm, the adjusted mean change from baseline at Wk 48 was +3.36 g/dL for Hb, +10.96 FACIT-F points and -107.95×10^9/L for ARC (adjusted mean difference in change from baseline at Wk 48 vs Wk 24: +3.02 g/dL, +10.79 points and -102.29×10^9/L, respectively). Transfusion avoidance was achieved by 93.5% of pts in the iptacopan arm (Wks 2 to 48) and 94.1% in the anti-C5-to-iptacopan arm (Wks 26 to 48). Mean lactate dehydrogenase levels were generally maintained <1.5 × upper limit of normal in both arms.

In the trial, 6/62 pts in the iptacopan arm had clinical breakthrough hemolysis (BTH). One pt in the anti-C5-to-iptacopan arm had clinical BTH after switching to iptacopan. BTH resolved without changing iptacopan dosing. Three pts had major adverse vascular events (MAVEs; randomized period: 1 serious transient ischemic attack [TIA]; extension period: 1 non-serious TIA, 1 serious portal vein thrombosis [PVT]). The pt with PVT had a history of PVT and discontinued heparin prior to the MAVE. All MAVEs were considered unrelated to iptacopan and resolved without changing iptacopan dosing. After 48 wks in the iptacopan arm, the most frequently reported treatment-emergent adverse events (TEAEs) were COVID-19 (29.0% of pts), headache (19.4%), diarrhea (16.1%) and nasopharyngitis (14.5%). There were no deaths, no serious hemolysis TEAEs on iptacopan, no serious infections caused by *N. meningitidis, S. pneumoniae* or *H. influenzae* and no pts discontinued treatment because of TEAEs.

Conclusions: Long-term data from the Phase III APPLY-PNH trial show a durable response to iptacopan monotherapy in anti-C5-treated PNH pts with persistent anemia. Pts who received iptacopan for 48 wks had sustained improvements in multiple hematological and clinical outcomes, including maintenance of increased Hb, mean normal/near-normal Hb levels, transfusion avoidance and decreased pt-reported fatigue; these benefits quickly emerged in the anti-C5-to-iptacopan arm, supporting the benefit of switching from C5 inhibitors to iptacopan monotherapy. The data indicate good control of hemolysis by iptacopan and a similar safety profile at Wk 48 vs Wk 24. Our findings continue to support oral iptacopan monotherapy as a potentially practice-changing treatment for hemolytic PNH.

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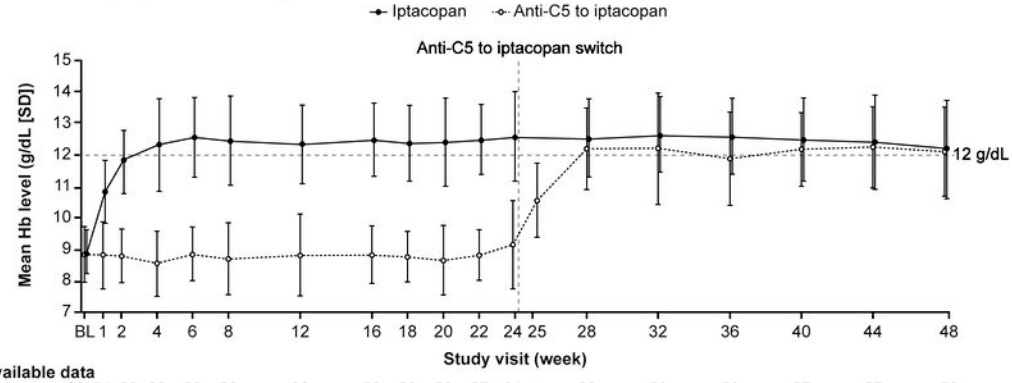
Figure. Mean Hb level (SD) over time during the entire 48-week treatment period of APPLY-PNH



Pts with available data																		
Iptacopan	62	61	59	60	59	59	60	59	59	59	57	61	–	56	59	59	57	57	59
Anti-C5 to iptacopan	35	29	27	32	32	31	33	28	34	32	32	34	27	28	32	31	29	30	30

Includes post-transfusion data. At Week 25, Hb data were only available for 1 pt in the iptacopan arm (Hb level: 13.9 g/dL); this was not a scheduled visit in the protocol for the iptacopan arm but was for the anti-C5-to-iptacopan arm. The value in the iptacopan arm is not plotted on the graph as 1 pt cannot be representative of the whole treatment group
BL, baseline; Hb, hemoglobin; pt, patient; SD, standard deviation

Table. Summary of efficacy parameters after the entire 48-week treatment period of APPLY-PNH, including comparison of data at Week 48 vs Week 24

Parameter	Arm			
	Iptacopan N=62 *Anti-C5 to iptacopan N=35*		*Adjusted mean change from baseline (95% CI) at Week 48*	*Adjusted mean difference in change from baseline (95% CI): Week 48 vs Week 24*
Change from baseline* in Hb level (g/dL)†	Iptacopan		**+3.35** (3.03, 3.66)	**–0.41** (–0.80, –0.01)
	Anti-C5 to iptacopan		**+3.36** (2.93, 3.79)	**+3.02** (2.48, 3.56)
Change from baseline‡ in FACIT-F score	Iptacopan		**+9.80** (8.04, 11.56)	**+0.73** (–1.14, 2.60)
	Anti-C5 to iptacopan		**+10.96** (8.58, 13.34)	**+10.79** (8.12, 13.47)
Change from baseline§ in ARC (10^9/L)	Iptacopan		**–106.26** (–117.57, –94.96)	**+9.92** (–4.40, 24.25)
	Anti-C5 to iptacopan		**–107.95** (–123.18, –92.73)	**–102.29** (–121.57, –83.02)
			Geometric adjusted mean ratio to baseline (95% CI) at Week 48	*Geometric adjusted mean ratio (95% CI): Week 48 vs Week 24*
Ratio to baseline¶ in log-transformed LDH (U/L)	Iptacopan		**1.11** (1.02, 1.22)	**1.12** (1.00, 1.25)
	Anti-C5 to iptacopan		**0.99** (0.88, 1.11)	**0.99** (0.85, 1.15)
		Time period	*Pts not requiring an RBC transfusion since 2 weeks after initiation of iptacopan monotherapy (n [%])*	
Transfusion avoidance#	Iptacopan	Week 2 to Week 48	58 (**93.5**)	
	Anti-C5 to iptacopan	Week 26 to Week 48 (iptacopan)	32 (**94.1**)**	
		Time period	*n/N‡‡*	*Overall adjusted annualized rate of events since initiation of iptacopan monotherapy, including both treatment arms (95% CI)*
Rate of clinical BTH††	Iptacopan	Baseline to Week 48	6/62	**0.11** (0.05, 0.23)
	Anti-C5 to iptacopan	Week 24 to Week 48 (iptacopan)	1/34	
Rate of MAVEs	Iptacopan	Baseline to Week 48	2/62	**0.04** (0.01, 0.13)
	Anti-C5 to iptacopan	Week 24 to Week 48 (iptacopan)	1/34	

*Mean (SD) baseline Hb levels were 8.93 (0.70) and 8.85 (0.90) g/dL in the iptacopan and anti-C5-to-iptacopan arms, respectively; †Analysis includes all central lab Hb data, including post-transfusion data; ‡Mean (SD) baseline FACIT-F scores were 34.7 (9.8) and 30.8 (11.5) in the iptacopan and anti-C5-to-iptacopan arms, respectively; §Mean (SD) baseline ARCs were 193.2 (83.6) and 190.6 (80.9) × 10^9/L in the iptacopan and anti-C5-to-iptacopan arms, respectively; ¶Mean (SD) baseline LDH levels were 269.1 (70.1) and 272.7 (84.8) U/L in the iptacopan and anti-C5-to-iptacopan arms, respectively; #Defined as neither receiving nor meeting the criteria to receive an RBC transfusion; **34 of 35 patients in the anti-C5-to-iptacopan arm received iptacopan in the extension period; ††Events that met the protocol-specified criteria for clinical BTH; ‡‡n=number of pts with event, N=number of pts treated with iptacopan
ARC, absolute reticulocyte count; BTH, breakthrough hemolysis; CI, confidence interval; FACIT-F, Functional Assessment of Chronic Illness Therapy – Fatigue; Hb, hemoglobin; LDH, lactate dehydrogenase; MAVE, major adverse vascular event; pt, patient; RBC, red blood cell; SD, standard deviation

Figure 1

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