Complement C1s inhibition with sutimlimab results in durable response in cold agglutinin disease: CARDINAL study 1-year interim follow-up results

Alexander Röth,¹ Wilma Barcellini,² Shirley D'Sa,³ Yoshitaka Miyakawa,⁴ Catherine M. Broome,⁵ Marc Michel,⁶ David J. Kuter,ⁿ Bernd Jilma,⁶ Tor Henrik Anderson Tvedt,⁶ Ilene C. Weitz,¹⁰ Parija Patel,¹¹ Xiaoyu Jiang,¹¹ Caroline Reuter,¹¹ Jun Su,¹¹ Frank Shafer,¹¹ Michelle Lee¹¹ and Sigbjørn Berentsen¹²

¹Department of Hematology and Stem Cell Transplantation, West German Cancer Center, University Hospital Essen, University of Duisburg-Essen, Essen, Germany; ²Fondazione IRCCS Ca' Granda Ospedale Maggiore Policlinico, Milan, Italy; ³UCLH Centre for Waldenström's Macroglobulinemia and Related Conditions, University College London Hospitals NHS Foundation Trust, London, UK; ⁴Thrombosis and Hemostasis Center, Saitama Medical University Hospital, Saitama, Japan; ⁵Division of Hematology, MedStar Georgetown University Hospital, Washington, DC, USA; ⁶Henri-Mondor University Hospital, Assistance Publique-Hôpitaux de Paris, UPEC, Créteil, France; ⁷Division of Hematology, Massachusetts General Hospital, Harvard Medical School, Boston, MA, USA; ⁸Department of Clinical Pharmacology, Medical University of Vienna, Vienna, Austria; 9Section for Hematology, Department of Medicine, Haukeland University Hospital, Bergen, Norway; ¹⁰Keck School of Medicine of USC, Los Angeles, CA, USA; ¹¹Sanofi, Cambridge, MA, USA and ¹²Department of Research and Innovation, Haugesund Hospital, Haugesund, Norway

Correspondence: ALEXANDER RÖTH - alexander.roeth@uk-essen.de https://doi.org/10.3324/haematol.2021.279812

Supplementary data

Supplemental table 1. Number of transfusions per patient and total units transfused, up to Week 53 (full analysis set)

	Part A (N = 24)		Part B (N = 22)
	Baseline to Week 5	Week 5 to Week 26	Week 27 to Week 53
	(n = 24)	(n = 23)	(n = 22)
Transfusions per patient,	0 (0–2)	0 (0–13)	0 (0–5)
median (range), n			
Patients requiring ≥1	5 (20.8)	6 (25.1)	3 (13.6)
transfusion, n (%)			
Patients requiring transfusion, r	1 (%)		
0 transfusions	19 (79.2)	17 (70.8)	19 (86.4)
1–2 transfusions	5 (20.8)	4 (16.7)	1 (4.5)
3–4 transfusions	0 (0.0)	1 (4.2)	1 (4.5)
5–6 transfusions	0 (0.0)	0 (0.0)	1 (4.5)
7–8 transfusions	0 (0.0)	0 (0.0)	0 (0.0)
9–10 transfusions	0 (0.0)	0 (0.0)	0 (0.0)
>10 transfusions	0 (0.0)	1 (4.2)	0 (0.0)
Total units transfused,* n	5	7	3
Median (range)	3 (2-4)	4 (2-23)†	8 (2–9)

Data cutoff date was 16 January 2020.

All patients were required to have received ≥ 1 blood transfusions in the 6 months prior to enrollment; at baseline the mean (\pm SD) number of transfusions during the previous year was 4.8 ± 6.2 with a median number of 2 (range, 1–23).

[†]One patient who received 23 units of blood had 13 red blood cell transfusions between Week 5 and end of treatment (in Part A), based on protocol criteria of transfusion for hemoglobin <9 g/dL without symptoms. Bilirubin levels in this patient remained above the ULN, consistent with continued extravascular hemolysis; reported adverse events did not indicate blood loss that required transfusion. This patient had been heavily transfused before study entry: 23 red blood cell transfusions were recorded within 12 months prior to study entry.

^{*}Included all patients who had ≥1 transfusion.