Haematologica HAEMATOL/2018/198887 Version 3

Sotatercept, a novel transforming growth factor beta ligand trap, improves anemia in beta-thalassemia: a phase 2, open-label, dose-finding study

Maria Domenica Cappellini, John Porter, Raffaella Origa, Gian Luca Forni, Ersi Voskaridou, Frédéric Galactéros, Ali T. Taher, Jean-Benoît Arlet, Jean-Antoine Ribeil, Maciej Garbowski, Giovanna Graziadei, Chantal Brouzes, Michaela Semeraro, Abderrahmane Laadem, Dimana Miteva, Jun Zou, Victoria Sung, Tatiana Zinger, Kenneth M. Attie, and Olivier Hermine

Disclosures: M.D.C. has served on advisory boards at Celgene Corporation, Novartis, and Sanofi/Genzyme. J.P. has received research funding from Novartis, and has served on advisory boards at Agios Pharmaceuticals, Bluebird Bio, Celgene Corporation, and Novartis. R.O. has received honoraria from Apopharma and Novartis; and has served on advisory boards at Bluebird Bio. G.L.F. has received research funding from Novartis and Shire; has been a consultant for Roche; has received travel expenses from Novartis; and is on the data safety monitoring board of Apopharma. F.G. has received grants from Addmedica and Novartis. A.T.T. has received research funding from Celgene Corporation, La Jolla Pharmaceuticals, Novartis Pharmaceuticals, and Roche; and has received honoraria from Novartis Pharmaceuticals. J.A.R. has received grants from Addmedica and Vitalaire; and has been a consultant for Bluebird Bio, Cydan, and Novartis. A.L. and V.S. are employed by and have equity in Celgene Corporation. D.M. is an employee of Celgene Corporation and has received grants from Celgene Corporation. J.Z. and T.Z. are employees of Celgene Corporation. K.M.A is employed by and has equity in Acceleron Pharma. O.H. has received grants from AB Science, Celgene Corporation, Hybrigenics, Inatherys, and Novartis; and has received personal fees from AB Science. E.V., J.B.A., M.G., G.G., C.B., and M.S. have no conflict of interest to declare.

Contributions: A.L., J.A.R., J.B.A., O.H., M.D.C., and J.P. contributed to the study concept and design; all authors participated in the acquisition, analysis, or interpretation of data; M.D.C., O.H., A.L., and T.Z. drafted the manuscript; all authors critically revised the manuscript for important intellectual content; J.Z. performed the statistical analyses; A.L. and K.M.A. obtained funding; A.L. and T.Z. provided administrative, technical or material support; T.Z. and D.M. supervised the study; M.D.C. had full access to all of the data in the study and takes responsibility for the integrity of the data and the accuracy of the data analysis; all authors had full access to all of the data in the study and are fully responsible for content and editorial decisions for this manuscript.