

Step Pharma announces publication in *Haematologica* of key data supporting CTPS1 inhibition as a therapeutic target in blood cancer

St. Genis-Pouilly, France, 24 July 2024 - Step Pharma, the world leader in CTPS1 inhibition for the targeted treatment of cancer, today announces the publication in the August edition of [Haematologica](#) of preclinical data from the University of Nantes (CRCl²NA) further supporting the therapeutic activity of Step's highly selective CTPS1 inhibitors in the treatment of blood cancers.

The therapeutic activity was observed for mantle cell lymphoma (MCL), including difficult-to-treat *in vitro* and *in vivo* models. Furthermore, Step Pharma's CTPS1 inhibitors show significant synergy when combined with venetoclax, a selective BCL2 inhibitor that is commonly used to treat certain types of lymphoma and leukaemia.

CTPS1, an enzyme crucial in pyrimidine synthesis, plays a significant role in cancer cell proliferation. Step Pharma's compounds selectively inhibit the *de novo* pyrimidine synthesis pathway by targeting CTPS1, providing a novel approach to cancer treatment. The Company's lead asset, dencatistat (STP938), a first-in-class, highly selective, orally bioavailable CTPS1 inhibitor, is currently in phase 1 clinical development for T cell and B cell lymphoma (NCT05463263) with study sites open in France, the UK, and the USA.

MCL accounts for approximately 5% of B cell lymphoma. Despite recent advancements in the treatment of MCL, there is still an unmet clinical need for those who have not responded to Bruton's Tyrosine Kinase (BTK) inhibitor therapy.

Andrew Parker, Chief Executive Officer of Step Pharma, commented: "The publication in *Haematologica* of these preclinical data provides additional evidence of the importance of inhibiting CTPS1 for the treatment of blood malignancies, including T and B cell lymphoma. The findings further support our approach as we continue to progress our phase 1 trial to develop targeted and efficient treatment options for individuals with blood cancer."

David Chiron, CNRS researcher at CRCl²NA, Nantes University, added: "These data represent a significant advancement in our understanding of blood cancer biology, particularly regarding the role of CTPS1. Our research on inhibiting CTPS1 emphasises the potential of this approach to fill an important gap in current treatment methods. These findings support the targeted inhibition of CTPS1 as a promising therapeutic strategy and pave the way for further research to gain a deeper understanding of its role and broader applications in different types of blood cancers."

Article citation: Selective pharmacological targeting of CTPS1 shows single-agent activity and synergizes with BCL2 inhibition in aggressive mantle cell lymphoma. Romane Durand, Celine Bellanger, Benoit Tessoulin, Charlotte Kervoelen, Christelle Dousset, Emmanuelle Menoret, Helene Asnagli, Andrew Parker, Philip Beer, Catherine Pellat-Deceunynck, and David Chiron. *Haematologica*, 2024; 109, 2574 – 2584.

About Step Pharma

Step Pharma's goal is to bring about a step change in how cancer is treated with targeted therapies that kill cancer cells and leave healthy cells unharmed. The Company is the world leader in CTPS1 inhibition, a new approach with the potential to yield highly selective, safe and effective cancer treatments for both blood cancers and solid tumours.

The Company's lead product dencatistat (STP938) has received both IND and CTA clearance to proceed into first in human trials in the US and UK for the treatment of T cell and B cell



lymphomas. Clinical trials in lymphoma commenced in September 2022. Dencatostat has the potential to be the backbone of a multitude of cancer therapies as well as a potent monotherapy for hard-to-treat blood cancers.

Step Pharma was founded in 2014 by Kurma Partners, the Imagine Institute and Sygnature Discovery, based on the scientific discoveries of Prof. Alain Fischer and Dr Sylvain Latour. Step Pharma is based in Saint-Genis-Pouilly, France, and is supported by a strong syndicate of investors led by Kurma Partners and including Bpifrance (Fonds Biothérapies Innovantes et Maladies Rares and InnoBio2 Fund), Pontifax, Hadean Ventures, Sunstone Life Science Ventures, Inserm Transfert Initiative, Iinvest, Sygnature Discovery and the Imagine Institute. More information on the company can be found at www.step-ph.com.

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