Q32 Bio and Horizon Therapeutics plc Announce Dosing of First Patient in Phase 2 Trial of Bempikibart (formerly ADX-914) for Severe Alopecia Areata

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-- Alopecia areata is the second autoimmune indication being evaluated for bempikibart --

WALTHAM, Mass. and DUBLIN, Sept. 13, 2023 /PRNewswire/ -- Q32 Bio, a clinical stage biotechnology company developing biologic therapeutics to restore immune homeostasis, and Horizon Therapeutics plc (Nasdaq: HZNP), today announced that the first patient has been dosed in a randomized, double-blind, placebo-controlled, Phase 2 multicenter, proof-of-concept study to evaluate bempikibart in adult patients with severe alopecia areata. Q32 Bio and Horizon are collaborating to develop bempikibart, a fully human anti-IL-7Rα antibody that re-regulates adaptive immune function by blocking signaling mediated by both IL-7 and TSLP, two key immune pathways.

"Dosing of the first patient in the Phase 2 study in alopecia areata, in addition to our ongoing Phase 2 study in atopic dermatitis, demonstrate the breadth of potential applications of bempikibart and our deep commitment to transform the lives of patients with autoimmune and inflammatory diseases," said Jodie Morrison, board member and acting chief executive officer, Q32 Bio. "Q32 Bio and Horizon are mutually dedicated to advancing bempikibart expeditiously through both Phase 2 studies."

"Alopecia areata is a complex disease with a significant unmet medical need, where patients can experience patchy or total hair loss that can impact their quality of life," said Elizabeth H.Z. Thompson, Ph.D., executive vice president, research and development, Horizon. "Bempikibart represents a potential unique approach to correcting immune system dysregulation in this and other diseases."

"IL-7 is involved in the pathogenesis of alopecia areata and bempikibart has the potential to address this pathology by inhibiting IL-7Rα function and modulating T-cell responses. Bempikibart has the potential to provide long-term, durable improvement in hair loss, including the potential for disease remittance," added Jason Campagna, M.D., Ph.D., chief medical officer, Q32 Bio. "We are pleased to work alongside our partner, Horizon, to advance bempikibart to the next stage of development for patients enduring the debilitating physical and psychosocial effects of severe alopecia areata."

Alopecia areata is a common, acute onset, autoimmune disorder that affects hair follicles and is characterized by transient, non-scarring hair loss.[1] It is the second most common form of alopecia and is associated with comorbidities including depression, anxiety and autoimmune diseases such as lupus erythematosus and vitiligo.[2] This condition may develop at any age, disproportionately impacts women and African American populations in the U.S. and the incidence of this disease is estimated to be two percent of the population worldwide.[3-5]

About Bempikibart
Bempikibart (formerly ADX-914) is a fully human anti-IL-7Rα antibody that re-regulates adaptive immune function by blocking signaling mediated by both IL-7 and TSLP. Q32 Bio has completed a biomarker-enabled Phase 1 study characterizing the pharmacokinetics, pharmacodynamics and safety of bempikibart in healthy volunteers. Q32 has initiated Phase 2 trials in atopic dermatitis and alopecia areata.

About the Q32 Bio-Horizon Collaboration for Bempikibart in Autoimmune Diseases
In August 2022, Q32 Bio and Horizon announced that they had entered into a collaboration and option agreement to develop bempikibart for the treatment of autoimmune diseases. Under the terms of the agreement, Horizon is obligated to fund development through completion of two Phase 2 trials of bempikibart, with Q32 being operationally responsible for the conduct of all program-related activities. Horizon received an option to acquire the bempikibart program, exercisable through a period following completion of the Phase 2 trials and related development activities.

About Q32 Bio
Q32 Bio is a clinical stage biotechnology company developing biologic therapeutics targeting potent regulators of the innate and adaptive immune systems to re-balance immunity in autoimmune and inflammatory diseases. Q32 Bio's lead programs, focused on the IL-7 / TSLP receptor pathways and complement system, address immune dysregulation to help patients take back control of their lives.

The Company's most advanced program, bempikibart, is a fully human anti-IL-7Rα antibody that re-regulates adaptive immune function and is being developed in collaboration with Horizon Therapeutics for the treatment of autoimmune diseases, including Phase 2 trials in both atopic dermatitis and alopecia areata. The IL-7 and TSLP pathways have been genetically and biologically implicated in driving several T cell-mediated pathological processes in numerous autoimmune diseases.

Q32 Bio's lead program for innate immunity, ADX-097, is based on a novel platform enabling tissue-targeted regulation of the complement system without long-term systemic blockade – a key differentiator versus current complement therapeutics. Q32 Bio has recently completed a first-in-human, Phase 1, ascending dose (SAD/MAD) clinical study of ADX-097 in healthy volunteers. For more information, please visit www.Q32bio.com.

About Horizon
Horizon is a global biotechnology company focused on the discovery, development and commercialization of medicines that address critical needs for people impacted by rare, autoimmune and severe inflammatory diseases. Our pipeline is purposeful: We apply scientific expertise and courage to bring clinically meaningful therapies to patients. We believe science and compassion must work together to transform lives. For more information on how we go to incredible lengths to impact lives, visit www.horizontherapeutics.com and follow us on Twitter, LinkedIn, Instagram and Facebook.

Forward-Looking Statements
This press release contains forward-looking statements, including, but not limited to, statements related to future clinical development of bempikibart, activities and payments under the collaboration between Horizon and Q32 and the potential benefits of bempikibart. These forward-looking statements are based on Horizon's and Q32's current expectations and inherently involve significant risks and uncertainties. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of these risks and uncertainties, which include, without limitation, risks and uncertainties associated with development, regulatory approval and commercialization of novel therapeutic candidates, the timing of development activities under the collaboration; the fact that the collaboration is subject to early termination and the fact that Horizon has limited control over the development of the bempikibart program prior to exercising its acquisition option. For a further description of these and other risks facing Horizon, please see the risk factors described in Horizon's filings with the United States Securities and Exchange Commission, including those factors discussed under the caption "Risk Factors" in those filings. Forward-looking statements speak only as of the date of this press release and Horizon undertakes no obligation to update or revise these statements, except as may be required by law.

References

- Trueb RM, Dias M. Alopecia Areata: A Comprehensive Review of Pathogenesis and Management. *Clin Rev Allergy Immunol*. 2018;54(1):68-87.
- Chu SY, Chen YJ, Tseng WC, et al. Comorbidity profiles among patients with alopecia areata: the importance of onset age, a nationwide population-based study. *J Am Acad Dermatol*. 2011;65(5):949-956.
- Mostaghimi A, Gao W, Ray M, et al. Trends in Prevalence and Incidence of Alopecia Areata, Alopecia Totalis, and Alopecia Universalis Among Adults and Children in a US Employer-Sponsored Insured Population. *JAMA Dermatol*. 2023;159(4):411–418.
- Lee H, Jung SJ, Patel AB, Thompson JM, Qureshi A, Cho E. Racial characteristics of alopecia areata in the United States. *J Am Acad Dermatol*. 2020 Oct;83(4):1064-1070.
- Darwin, E., Hirt, P. A., Fertig, R., Doliner, B., Delcanto, G., & Jimenez, J. J. (2018). Alopecia Areata: Review of Epidemiology, Clinical Features, Pathogenesis, and New Treatment Options. *International journal of trichology*. 10(2), 51–60.

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