Q32 Bio Initiates First-in-Human Phase 1 Clinical Study of ADX-097 for the Treatment of Complement Disorders

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- *First clinical-stage program from Q32 Bio's tissue targeted complement inhibitor platform*
- *Clinical-stage pipeline now consists of two programs in development to restore immune homeostasis*

WALTHAM, Mass., May 26, 2022 /PRNewswire/ -- Q32 Bio, a clinical stage biotechnology company developing biologic therapeutics to restore immune homeostasis, today announced that dosing has been initiated in the healthy volunteer portion of its Phase 1, single ascending dose and multiple ascending dose (SAD/MAD) clinical study evaluating ADX-097, a first-in-class fusion protein, for the treatment of complement disorders. ADX-097 is a tissue-targeted inhibitor of complement activation that minimizes systemic complement blockade and is being investigated for the treatment of patients with skin and renal diseases associated with increased complement activation.

"The initiation of the Phase 1 study of ADX-097 is an important milestone for Q32 Bio. We believe that ADX-097's unique mechanism and potent tissue-targeted complement inhibition have the potential to provide benefit for patients living with diseases associated with dysregulated complement activity," said Michael Broxson, Chief Executive Officer of Q32 Bio. "The Phase 1 ADX-097 study, Q32 Bio's first clinical program designed to restore regulation of complement activity in diseased tissue, marks the second clinical stage program in our portfolio, exemplifying Q32 Bio's commitment to rapidly develop biologic therapeutics targeting powerful regulators of the innate and adaptive immune systems and re-balancing immunity in severe autoimmune and inflammatory diseases."

"We are pleased to initiate this first-in-human study of ADX-097," said Shelia Violette, Ph.D., Founder, Chief Scientific Officer and President of Research of Q32 Bio. "ADX-097 is a promising development candidate because of its unique potential to restore control of the complement system at tissue-specific sites where there is ongoing complement activation. Tissue-targeting could provide therapeutic benefit in a wide range of inflammatory and autoimmune diseases."

In preclinical studies, ADX-097 demonstrated the potential to potently inhibit disease-causing complement activation in the tissue while avoiding systemic complement inhibition. The biochemical and functional profile, consisting of a humanized anti-C3d monoclonal antibody linked to two moieties of the first five consensus repeats of the complement negative regulatory protein human factor H (fH_{1-5}), demonstrates effective tissue localization, potent inhibition of the alternative complement pathway and attractive drug-like properties. Additionally, pre-clinical results highlight the potential for a differentiated dosing regimen and safety profile.

The Phase 1, first-in-human, clinical study is designed to assess the safety, pharmacokinetics and pharmacodynamics of ADX-097 in two parts. The study consists of a randomized, placebo-controlled, double-blind SAD/MAD component in healthy volunteers and an open-label, SAD/MAD component in patients with complement-related skin diseases.

About ADX-097

ADX-097 is a first-in-class fusion protein that Q32 Bio designed to provide a unique targeted therapeutic approach to restore proper complement regulation in diseased tissue. The Company believes that ADX-097 can inhibit AP convertases in tissues where activity is pathologically high while leaving systemic, homeostatic and protective AP activity intact. In preclinical studies, ADX-097 has proven *in vivo* distribution to affected tissues/organs, durable tissue pharmacokinetics (PK)/pharmacodynamics (PD), robust *in vivo* efficacy, and attractive drug properties.

About Q32 Bio

Q32 Bio is a clinical stage biotechnology company developing biologic therapeutics targeting powerful regulators of the innate and adaptive immune systems to re-balance immunity in severe 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, ADX-914, is a fully human anti-IL-7Ra antibody. 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 has completed dosing in a Phase 1 trial of ADX-914 in healthy volunteers and plans to initiate Phase 2 studies in the first half of 2022.

Q32 Bio's lead program for innate immunity, ADX-097, is based on a pioneering approach enabling tissue-targeted regulation of the complement system without long-term systemic blockade – a key differentiator versus current complement therapeutics. Q32 Bio plans to initiate first-in-human trials for ADX-097 in the first half of 2022. For more information, please visit www.Q32bio.com.

Contact

Investors: Brendan Burns
Media: Sarah Sutton
Argot Partners
212.600.1902
Q32Bio@argotpartners.com

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