Arcellx Announces FDA Orphan Drug Designation for CART-ddBCMA for the Treatment of Multiple Myeloma

– Phase 1 trial in relapsed and refractory multiple myeloma continues to enroll patients –

GAITHERSBURG, Md., March 12, 2020 (GLOBE NEWSWIRE) -- Arcellx, a clinical-stage biopharmaceutical company devoted to developing immune cell therapies for cancer and other diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to CART-ddBCMA, its engineered T cell therapy utilizing a novel binding domain, for the treatment of patients with multiple myeloma. Arcellx continues to enroll patients in its Phase 1 trial of CART-ddBCMA therapy for the treatment of relapsed and refractory multiple myeloma, the first in a series of clinical trials planned for the stepwise development of the Arcellx ARC-T + sparX cell therapy platform. This trial marks the first-in-human trial of the Arcellx binding domain, which the Company plans to evaluate in future ARC-T + sparX trials.

“We are pleased to be one of the first sites to test this new technology in the clinic,” said Matthew Frigault, M.D., Assistant Director of the Cellular Therapy Service at Massachusetts General Hospital Cancer Center and Instructor at Harvard Medical School. “The novel deimmunized binding domain utilized in this trial may be potentially less immunogenic than the single chain variable fragment (scFv) or camelid binders used in conventional CAR T-cell therapies.”

“Orphan Designation recognizes the unmet need of populations with rare diseases like multiple myeloma, particularly for those patients who have exhausted other therapeutic options,” stated Amy B. Fix, M.S., M.B.A., Senior Vice President of Regulatory Affairs of Arcellx. “Receiving both Orphan and Fast Track Designations further validates the potential of Arcellx technology and our novel binding domain. We hope to provide patients with a new therapy that may significantly improve treatment response for this debilitating disease.”

The FDA’s Office of Orphan Drug Products grants orphan status to drugs intended to treat rare diseases affecting fewer than 200,000 people in the U.S. The designation provides certain benefits to drug developers, including tax credits for clinical trial expenditures, waived user fees for marketing applications, and eligibility for seven years of marketing exclusivity.

About the Arcellx ddBCMA T Cell Therapy Phase 1 Trial

The open label Phase 1 trial is evaluating an engineered T cell therapy that uses the Company’s novel synthetic binding domain in the treatment of patients with relapsed and refractory multiple myeloma. In the trial, a patient’s T cells are engineered to express a receptor targeting the B-cell maturation antigen (BCMA) on the tumor cell surface using the novel binding domain. The binding domain, which is a deimmunized synthetic protein, is a key component of the Arcellx ARC-T + sparX cell therapy platform. The Arcellx ddBCMA cell therapy has been granted Fast Track Designation and Orphan Drug Designation by the U.S. Food and Drug Administration (FDA). The trial is currently enrolling patients. Additional information about the trial can be found at https://www.clinicaltrials.gov/ct2/show/NCT04155749.

About ARC-T + sparX Technology

Arcellx has developed a proprietary platform in which ARC-T (Antigen Receptor Complex T cells) are controlled by the administration of a tumor-targeting protein called a sparX (Soluble Protein Antigen-Receptor X-linker). A library of deimmunized sparX proteins that recognize different cell surface antigens are functional as monovalent, bivalent, or bispecific constructs, and could potentially be administered simultaneously or sequentially to address the inherent heterogeneity of diseases such as cancer. The ARC-T cells can be readily silenced, activated and reprogrammed by sparX, allowing dose control to minimize toxicities and multiple antigen targeting to improve efficacy and address relapse. The ARC-T + sparX therapeutic platform is designed to potentially provide enhanced efficacy, safety, patient accessibility and efficiency of manufacturing relative to existing cell therapies.

About Multiple Myeloma

Multiple myeloma is a blood cancer that may cause cancerous tumors in bone and soft tissue. It is characterized by abnormal plasma cells called myeloma cells. Plasma cells develop from B lymphocytes (B cells), a type of white blood cell that is made in the bone marrow. When myeloma cells collect in multiple bones, the disease is called multiple myeloma. The National Cancer Institute estimates that in the United States, there were 32,110 new cases of myeloma in 2019 and 131,392 people living with the disease in 2016.[1] The current standard of care includes chemotherapy, targeted therapy, high-dose chemotherapy with stem cell transplant, biologic therapy, radiation therapy and surgery.

[1] National Cancer Institute. Surveillance, Epidemiology, and End Results (SEER) Program. Cancer Stat Facts: Myeloma. Available at https://seer.cancer.gov/statfacts/html/mulmy.html. Accessed March 12, 2020.

About Arcellx, Inc.

Arcellx is a clinical-stage biopharmaceutical company devoted to providing patients with superior immune cell therapies through scientific innovation and accelerated development of next-generation technology. Arcellx initially is developing the ARC-T cell therapies for cancer indications, and in the future, broader indications, including autoimmune disease. More information can be found at www.arcellx.com.

Contact:
Zara Lockshin (media), Solebury Trout
O: +1 646-378-2960

M: +1 330-417-6250
Email: zlockshin@troutgroup.com

Alan Lada (investors), Solebury Trout
O: +1 646-378-2927
Email: alada@troutgroup.com