Mustang Bio, Inc.
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Revving up CAR T and gene therapy development for cancer

Mustang Bio is developing next-generation CAR T and gene therapies for cancer and rare genetic diseases, with lead clinical programs in non-Hodgkin lymphoma, chronic lymphocytic leukemia, and X-linked severe combined immunodeficiency.

Massachusetts-based Mustang Bio is a clinical-stage biopharmaceutical company focused on advancing chimeric antigen receptor T cell (CAR T) therapies across multiple hematologic cancers and solid tumors, as well as gene therapies for rare genetic diseases. The company's extensive pipeline of next-generation and potentially first-in-class therapies leverages Mustang's core competencies in translational science and clinical development to advance product candidates in collaboration with world-leading academic institutions.

Lead programs include CD20-targeting CAR T cells for non-Hodgkin lymphoma (NHL) and chronic lymphocytic leukemia (CLL), being developed in collaboration with Fred Hutchinson Cancer Research Center (Fred Hutch), and a lentiviral gene therapy for X-linked severe combined immunodeficiency (XSCID) developed at St. Jude Children's Research Hospital (St. Jude).

"At Mustang, we partner with leading academic institutions to advance post-discovery programs toward clinical development," said Manuel Litchman, Mustang's President and CEO. "Our translational experience and sophisticated manufacturing capabilities ensure that we can maximize the potential of these exciting next-generation therapies for patients in need."

With a deep pipeline of cell and gene therapies (Fig. 1), a 27,000-square-foot state-of-the-art manufacturing facility, and a team with a track record in pioneering cell-based gene therapies, Mustang is the partner of choice for anyone seeking to deliver the next breakthroughs in this field.

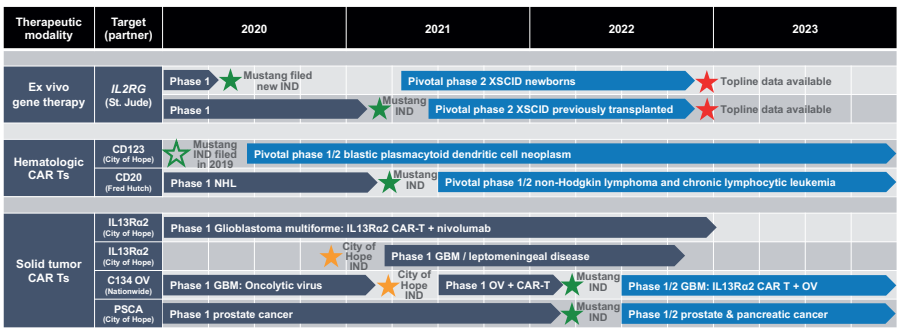

Fig. 1 | Mustang Bio's pipeline of therapies. City of Hope, City of Hope National Medical Center; Fred Hutch, Fred Hutchinson Cancer Research Center; GBM, glioblastoma multiforme; IND, investigational new drug; Nationwide, Nationwide Children's Hospital; NHL, non-Hodgkin lymphoma; OV, oncolytic virus; St. Jude, St. Jude Children's Research Hospital; XSCID, X-linked severe combined immunodeficiency.

Putting the CAR T brakes on NHL and CLL

CAR T cell therapies are customized treatments created from a patient's own T cells—collected from the patient and sent to a manufacturing facility where they are genetically modified to express a CAR that helps direct the T cells to target specific cancer cells. The modified cells are infused back into the patient where they attack cancer cells that display on their surface the specific antigen targeted by the CAR.

Mustang's lead CAR T cell program, MB-106, is a third-generation CAR T cell therapy with a fully human target-binding domain for the treatment of NHL and CLL. MB-106 targets CD20, a clinically and commercially validated oncology target.

As reported in December 2020, Fred Hutch developed a novel MB-106 cell process and treated nine patients with this process in a phase 1 clinical trial. Only one patient experienced cytokine release syndrome—assessed as grade 1—and no CAR T-related neurotoxicity of any grade was observed. Additionally, MB-106 exhibited high response rates (objective response rate [ORR 89%]; complete responses [CR 44%]) and robust CAR T expansion and persistence. All complete response patients remain in remission as of the last data announcement in December 2020. Fred Hutch expects to provide updated data for this trial in June 2021.

Mustang is planning to enroll the first patient in a pivotal phase 1/2 clinical trial in NHL and CLL in the third quarter of 2021.

Going viral on XSCID

XSCID is an inherited disorder of the immune system caused by mutations of interleukin-2 receptor subunit gamma (*IL2RG*). XSCID predominantly affects males, and without treatment, most XSCID patients do not live much beyond infancy. While standard-of-care treatment—immune reconstitution via allogeneic hematopoietic stem cell transplant—has a high success rate, gene therapy holds the promise of a more permanent and safer treatment for XSCID.

Mustang's lead gene therapy program is a lentiviral gene therapy for XSCID developed in collaboration with St. Jude. The therapy involves ex vivo lentiviral transduction of the patient's own hematopoietic stem cells with a normal copy of the mutated gene. The overall process used to obtain, modify, and infuse the cells back into the patient is analogous to the process developed for Mustang's CAR T cell platform.

Mustang's XSCID gene therapy is in two ongoing first-in-human clinical trials, led by St. Jude and NIH, in newborn and previously transplanted XSCID patients, respectively. Based on highly compelling safety and efficacy data reported for both trials, Mustang is targeting IND approvals for pivotal multicenter trials by the middle of 2021.

Partnering cell and gene therapies

A critical aspect of bringing the cell and gene therapies to patients is consistent and reliable production. Mustang has built an industry-leading cell therapy manufacturing facility capable of processing therapies for both clinical development and commercialization. This facility, together with the company's deep pipeline, gives Mustang a competitive advantage for the development of novel, groundbreaking cell and gene therapies.

"We are committed to strategic growth through targeted product acquisitions, including through program purchases, in-licensing, and co-development opportunities aligned with our areas of expertise," said Litchman. "The goal throughout is to harness the potential of next-generation gene and cell therapies in order to revolutionize patient care."

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