Safi Biotherapeutics Granted Rare Pediatric Disease Designation and Orphan Drug Designation from the U.S. FDA

Designations support use of manufactured red blood cells in sickle cell disease chronic transfusion therapy

CAMBRIDGE, MA, UNITED STATES, December 18, 2024 / EINPresswire.com/ -- Safi Biotherapeutics, a biotech company producing stem-cell derived, human red blood cell (RBC) products for civilian and military transfusion needs, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation (RPDD) and Orphan Drug Designation (ODD) to their manufactured red blood cells (mRBCs) for use in the chronic transfusion of sickle cell patients.

Safi produces highly characterized, stem cell-derived human RBCs for civilian and military transfusion needs. The civilian commercial opportunity encompasses chronic transfusion indications for anemias such as sickle cell disease (SCD). About 10% of SCD patients require regular transfusions of RBCs, often monthly or even greater. Many of these patients tend to have rarer blood types and, due to the frequent transfusions, can also develop alloimmunizations that make it even more challenging to locate appropriately matched RBC units. Safi expects to begin accelerating the validation of cGMP manufacturing of mRBCs at clinically meaningful scale in the near-term and to complete IND-enabling activities for clinical trial initiation in 2027.

> We are pleased to receive these rare disease regulatory designations from the FDA, which underscore the need for an alternative supply of RBCs for sickle cell transfusions”
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> *Safi CEO and Co-founder Doug McConnell*

“We are pleased to receive these rare disease regulatory

designations from the FDA, which underscore the need for a blood supply alternative for sickle cell patients requiring chronic transfusions of RBCs," said Doug McConnell, Chief Executive Officer and Co-Founder of Safi Biotherapeutics. "We are on the path to making economically viable mRBCs at industrial scale to augment the existing donor blood supply with a product appropriately tailored to meet the specific needs of these patients. These designations will allow us to progress through the appropriate regulatory path and bring our innovations to the chronic, anemia-related transfusion space as quickly as possible."

The FDA grants RPDD and ODD for serious and life-threatening diseases that affect fewer than 200,000 people in the United States and, particularly for RPDD, for which the serious or life-threatening manifestations primarily affect individuals less than 18 years of age. The RPDD makes Safi eligible for the FDA's Rare Pediatric Disease Priority Review Voucher Program, designed to incentivize the development of treatments for unique and small patient populations. The ODD designation can provide for a seven-year window of exclusive marketing rights post-approval as well as exemption from user fees and eligibility for tax credits for qualified clinical trials. In addition to the financial benefits, it also has the potential to shorten clinical development due to closer collaboration with the FDA.

About Safi Biotherapeutics
Safi Biotherapeutics produces stem-cell derived, human RBCs with the goal of providing a highly characterized cell therapy product at industrial scale and viable economics for civilian and military transfusion needs. Safi's manufacturing blueprint for RBC production is the most advanced in the industry, and the company's readily addressable markets include chronic transfusion indications such as sickle-cell disease and acute transfusion settings such as civilian and military hospitals during critical times of need. Safi leadership, comprised of industry and cell therapy veterans from DARPA, Vertex Pharmaceuticals, and Loughborough University in the United Kingdom, launched the company in 2020 as part of the U.S. Department of Defense On-Demand Blood program.

For more information on Safi, visit: https://safi.bio/

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