



MEDICAL AND RESEARCH ADVISORY COMMITTEE (MARAC)

MARAC Advisory: Pociredir Program Discontinuation

June 2026 – The Sickle Cell Disease Association of America, Inc. (SCDAA) Medical and Research Advisory Committee (MARAC) is saddened to hear the news that Fulcrum Therapeutics is discontinuing development of its pociredir program for the treatment of sickle cell disease.

Pociredir is an investigational oral therapy that was hoped would increase fetal hemoglobin. The U.S. Food and Drug Administration (FDA) expressed safety concerns linked to drugs targeting the protein complex which pociredir targeted.

Though progress has been made in the treatment of sickle cell disease, there is still a limited number of therapies available for individuals suffering from sickle cell disease. MARAC encourages the pharmaceutical industry and the FDA to work with the sickle cell community to continue to look for therapies that not only address the clinical outcomes that are often evaluated in sickle cell studies, but also explore outcome measures and endpoints that address patient-reported outcomes that impact quality of life. MARAC looks forward to continuing to partner with the pharmaceutical industry, government and the sickle cell community to find treatments for the sickle cell population.