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The Development of A Novel Therapeutic for the Treatment of Sickle Cell Disease

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Poster Presentation P43

THE DEVELOPMENT OF A NOVEL THERAPEUTIC FOR THE TREATMENT OF SICKLE CELL DISEASE

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Sickle Cell Disease is a genetic blood disorder caused by a point mutation in the gene which codes for hemoglobin in red blood cells. This mutation in the protein leads to the formation of long polymeric strands of hemoglobin that cause the red blood cells to misform into the characteristic sickled shape. These sickled red blood cells are too large to fit through capillaries and thus cause the problems associated with sickle cell disease such as anemia and tissue damage. Our approach towards developing novel therapeutics involves the production and screening of large libraries of small peptides which target the point mutation in an effort to discover ligands for the protein. Once ligands have been identified, we can determine if their interactions are sufficient to prevent protein polymerization. We will present some recent progress that we have made on this challenging biochemical problem.