The hematological disorders therapy area, which covers indications such as sickle cell disease, paroxysmal nocturnal hemoglobinuria, anemia, thalassemia and hereditary hemochromatosis, is experiencing a significant level of first-in-class innovation, with 92 of its 327 pipeline programs with a disclosed molecular target identified as first-in-class, according to business intelligence provider GBI Research.

The company's latest report states that the hematological disorders market is small with substantial unmet need and as such represents a significant investment opportunity. Due to few treatment options, many patients have a poor quality of life and poor prognosis, especially those with more severe disease states. The lack of pharmacotherapy treatment options means that alternative treatment approaches such as chronic blood transfusions and bone marrow transplants are used.

Callum Dew, Analyst for GBI Research, explains: "Although blood transfusions can manage certain red blood cell disorders, this approach itself can lead to iron overload. This is known as hemochromatosis, another hematological disorder that itself affects patients' quality of life and prognosis due to an increase in the risk of organ failure. Similarly, bone marrow transplants, which can treat a range of hematological disorders, carry many risks, ranging from anemia to more severe and potentially fatal disorders."

Investment in first-in-class development is particularly important within hematological disorders as it holds the potential of achieving clinical breakthroughs through radical pharmaceutical innovation. As such, it represents the most likely method of discovering new and effective drugs for a therapy area in which there has been a lack of funding and few recent breakthrough treatment discoveries.

GBI Research believes a successful first-in-class product has the potential to gain a large market share due to the significant level of unmet need with regard to effective marketed treatment options.

Dew continues: "There are a multitude of reasons for investing in the hematological disorders market. Aside from the small number of effective marketed products within the therapy area, there are also a wide range of indications which vary greatly in their prevalence and a number

of orphan diseases. Globally prevalent disorders such as anemias, of which the most common form – iron deficiency anemia – is estimated to affect over 13% of the world's population, also represent significant investment opportunities due to the number of patients eligible for treatment."