



66th ASH® Annual Meeting and Exposition

# American Society of Hematology 2024



## Abstracts of Interest: *Non-malignant Hematology*

### Anchor Point Insight:

New and updated data at the 2024 ASH Annual Meeting showcase advances across the non-malignant hematology landscape, including in SCD, ITP, and autoimmune indications.



Anchor Point  
Insights



# Non-malignant Hematology Abstracts of Interest

## **An Update on Lovotibeglogene Autotemcel (Lovo-cel) Clinical Trials for Sickle Cell Disease (SCD) and Analysis of Early Predictors of Response to Lovo-Cel**

Stacey Rifkin-Zenenberg, DO

ASH #511

## **Durable Clinical Benefits with Exagamglogene Autotemcel for Transfusion-Dependent $\beta$ -Thalassemia**

Franco Locatelli, MD

ASH #512

### **Anchor Point Insight:**

Long-term data for commercial therapies in SCD (bluebird bio's Lovo-cel) and  $\beta$ -thalassemia (Vertex/CRISPR's Exa-cel) continue to confirm that these therapies appear to be potentially "one-time functional cures". However, it remains unclear how significant patient uptake will be due to the safety issues associated with the required myeloablative conditioning regimens.





66th ASH® Annual Meeting and Exposition

# Non-malignant Hematology Abstracts of Interest

**Initial Results from the BEACON Clinical Study: A Phase 1/2 Study Evaluating the Safety and Efficacy of a Single Dose of Autologous CD34+ Base Edited Hematopoietic Stem Cells (BEAM-101) in Patients with Sickle Cell Disease with Severe Vaso-Occlusive Crises**

Matthew M. Heeney, MD

ASH #513

**Etavopivat Reduces Incidence of Vaso-Occlusive Crises in Patients with Sickle Cell Disease: HIBISCUS Trial Phase 2 Results through 52 Weeks**

Matthew M. Heeney, MD

ASH #513

## Anchor Point Insight:

ASH'24 features interesting updates from early- to mid-stage clinical assets in SCD including preliminary data from Beam Therapeutics' BEACON trial of BEAM-101, a novel base-edited cell therapy, showing promising early outcomes. Novo Nordisk's etavopivat, an RBC pyruvate kinase activator, established POC showing a reduced annualized VOC rate and increased Hb levels compared to placebo.



Anchor Point  
Insights



# Non-malignant Hematology Abstracts of Interest

## Update on Monocentric CD19-CAR T-Cell Therapy in 30 Patients with Autoimmune Disease

Fabian Mueller, MD

ASH #684

## CD19 CAR T-Cell Therapy in Refractory Autoimmune Hemolytic Anemia

Jun Shi, MD

ASH #682

### Anchor Point Insight:

A key recent trend has seen numerous companies pivot away from hematologic malignancies and toward autoimmune (AI) indications, even with “aggressive” therapies like CAR-T. At ASH’24 we will see an updated 30 patient cohort from one of the original trials of a CD19 CAR-T in AI from Dr. Schett and University Hospital Erlangen. Also, Ph1 data for a CD19 CAR-T in refractory AIHA showcases the potential of cell therapy in an additional AI indication.





66th ASH® Annual Meeting and Exposition

# Non-malignant Hematology Abstracts of Interest

## **Efficacy and Safety of Oral Bruton Tyrosine Kinase Inhibitor (BTKi) Rilzabrutinib in Adults with Previously Treated Immune Thrombocytopenia (ITP): A Phase 3, Placebo-Controlled, Parallel- Group, Multicenter Study (LUNA 3)**

David J Kuter, MD, DPhil

ASH #5

### **Anchor Point Insight:**

An ASH'24 Plenary presentation highlights pivotal data from the Ph3 LUNA study of Sanofi's rilzabrutinib, a first-in-class BTK inhibitor, for the treatment of ITP. Rilzabrutinib showed promising efficacy (platelet response, reduced need for rescue therapy, etc.) and safety while also showing benefit in patient-centric measures such as fatigue and QoL.



Anchor Point  
Insights