

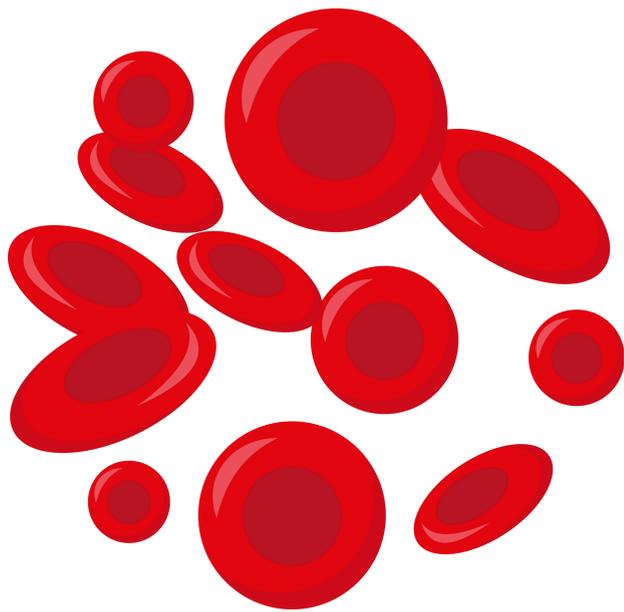


67th ASH[®] Annual Meeting
and Exposition
DECEMBER 6-9, 2025 | ORLANDO, FLORIDA



Anchor Point
Insights

American Society of Hematology 2025



Abstracts of Interest: *Non-malignant Hematology*

James Perry, Sr. Consultant
perry@anchorpointinsights.com

Key Highlights

- Across immune thrombocytopenia & other non-malignant heme indications, **BTK inhibitors offer a novel MOA** in line with other B-cell targeted therapies showing **promise in autoimmune diseases**
- Sickle cell disease remains a difficult indication to treat, although novel therapies hope to improve upon initial approvals. **Expansion of effective agents into pediatric SCD** offers the potential to **limit life-threatening complications** associated with accumulating end-organ damage
- **Expansion of oncology therapies**, including CAR-T cells, into autoimmune indications raises questions regarding **therapeutic safety** in non-malignant indications



**67th ASH[®] Annual Meeting
and Exposition**
DECEMBER 6-9, 2025 | ORLANDO, FLORIDA



Anchor Point
Insights

Immune Thrombocytopenia

BTK inhibitors, as a newly approved therapeutic class in ITP, offer a novel MOA that may target the underlying cause of disease

Reduction in corticosteroid use with rilzabrutinib and sustained response in adults with persistent/chronic immune thrombocytopenia in the long-term extension period of the Phase 3 LUNA3 study

David Kuter, MD, DPhil

ASH #1260

Improved health-related quality of life (HRQoL) and bleeding scores with oral bruton tyrosine kinase (BTK) inhibitor rilzabrutinib in the open-label (OL) period of the multicenter Phase 3 LUNA3 study in adults with immune thrombocytopenia (ITP)

Nicola Cooper, MD, FRCP, FRCPath, MA

ASH #1254

Anchor Point Insight:

Analyses from the Ph3 LUNA3 trial reinforce the benefit for rilzabrutinib, which in August 2025 became the **first FDA approved BTKi** for immune thrombocytopenia

Attending ASH? Have a question? Want to learn more?

We can help! Let's connect!



67th ASH[®] Annual Meeting
and Exposition
DECEMBER 6-9, 2025 | ORLANDO, FLORIDA



Anchor Point
Insights

Rilzabrutinib Across Indications

*Following its success in 2L+ ITP, rilzabrutinib is being tested in multiple non-malignant indications, continuing a trend of B-cell targeted therapies showing **promise in autoimmune indications***

TIP - Early multi-immune modulation with rilzabrutinib in patients with primary ITP after first-line treatment failure: A phase 3b study (LUNA 4)

David Kuter, MD, DPhil

ASH #4827

Long-term efficacy and safety of rilzabrutinib, an oral bruton tyrosine kinase (BTK) inhibitor in patients with warm autoimmune hemolytic anemia (wAIHA) in the LUMINA Phase 2b part B study: A 74-week follow-up

Bruno Fattizzo, MD

ASH #4655

TIP - A randomized, double-blind, placebo-controlled trial of a novel BTK inhibitor (rilzabrutinib) in patients with sickle cell disease (SCD) aged 10-65 years: LIBRA Study

Kenneth Ataga, MD

ASH #2975

Anchor Point Insight:

Additional abstracts showcase rilzabrutinib's **expansion across non-malignant hematology indications**, including potential future expansions into **earlier lines of ITP treatment**, continued advancement in wAIHA, and an initial look at a future study in sickle cell disease

We'd love to hear from you!

accounts@anchorpointinsights.com



**67th ASH[®] Annual Meeting
and Exposition**
DECEMBER 6-9, 2025 | ORLANDO, FLORIDA



Anchor Point
Insights

Sickle Cell Disease

*SCD has had limited recent success but numerous high profile failures. **Approval of gene therapies** offers some promise, but additional novel treatments are hoping to improve upon initial approvals*

Long-term follow-up demonstrates durable clinical benefits of exagamglogene autotemcel for sickle cell disease with recurrent vaso-occlusive crises: Final results of climb SCD-121

Stephan Grupp, MD

ASH #2537

Robust HbF induction and improvement of anemia and hemolysis with base editing in sickle cell disease: Safety and efficacy findings from the ongoing

Ashish Gupta, MD **BEACON study**

ASH #2532

CRISPR-Cas12a gene editing of the HBG1/2 promoters leads to sustained normalization of total hemoglobin and increased fetal hemoglobin in patients with severe sickle cell disease: Updated Results from the RUBY trial

Rabi Hanna, MD

ASH #4314

Anchor Point Insight:

Long-term follow-up from Vertex/CRISPR's CLIMB SCD-121 and CLIMB-131 studies of Casgevy (exagamglogene autotemcel) **establishes a comparator for novel therapies from Beam Therapeutics (BEAM-101) and Editas Medicine (Renizgamglogene autogedtemcel) in sickle cell disease**

Attending ASH? Have a question? Want to learn more?

We can help! Let's connect!



67th ASH[®] Annual Meeting
and Exposition

DECEMBER 6-9, 2025 | ORLANDO, FLORIDA



Anchor Point
Insights

Pediatric Sickle Cell Disease

*Expansion of effective agents into pediatric SCD offers the potential to **limit life-threatening complications associated with end-organ damage** that accumulates throughout SCD patients lives*

First results of exagamglogene autotemcel in pediatric patients aged 5-11 years with transfusion-dependent β -thalassemia or sickle cell disease with recurrent severe vaso-occlusive crises

Haydar Frangoul, MD

ASH #379

Etavopivat in adolescents with sickle cell disease: Emerging safety and efficacy findings from the first cohort of the ongoing Phase 1/2 hibiscus kids study

Adlette Inati, MD

ASH #618

Anchor Point Insight:

While therapeutic developments in pediatric sickle cell disease are **typically less prominently featured** than those for adult patients, ASH'25 will feature several interesting updates in this vulnerable population

We'd love to hear from you!

accounts@anchorpointinsights.com



67th ASH[®] Annual Meeting
and Exposition
DECEMBER 6-9, 2025 | ORLANDO, FLORIDA



Anchor Point
Insights

Autoimmune Indications

The expansion of oncology therapies, including CAR-T cells, into autoimmune indications raises questions regarding therapeutic safety in non-malignant indications

Safety and preliminary efficacy of anti-CD19 CAR T cells zorpocabtagene-autoleucel in systemic autoimmune diseases: Final analysis of the castle trial

Fabian Müller, MD

ASH #2380

One-year follow-up of CD19/BCMA dual-targeting fastcar-T GC012F (AZD0120) therapy in patients with refractory systemic lupus erythematosus

Jingjing Feng, MD

ASH #2384

Safety and preliminary efficacy of a novel loop-optimized, BCMA/CD19 bispecific CAR-T therapy for refractory or relapsed autoimmune diseases patients

SiYu Liu, MD

ASH #2370

Anchor Point Insight:

ASH'25 will see multiple updates highlighting the potential of cell therapies for the treatment of autoimmune indications, including several dual CD19/BCMA directed CAR-T's that are anticipated to **lead to deeper B-cell depletion** including plasma cells

Attending ASH? Have a question? Want to learn more?

We can help! Let's connect!