EHA2025
Congress

June 12 - 15, 2025 | Milan, Italy


# EHA Congress 2025

# Abstracts of Interest: *Non-malignant Hematology*

## Anchor Point Insght:

**Exciting data at the 2025 EHA Congress highlighted advances across the non-malignant hematology landscape, including in ITP, SCD/TDT, and autoimmune hemolytic anemias.**

Anchor Point Insights

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# Non-malignant Hematology Abstracts of Interest

## PHASE 3 LUNA3 STUDY: FIRST EFFICACY/SAFETY REPORT OF LONG-TERM EXTENSION PERIOD WITH RILZABRUTINIB IN ADULTS WITH PERSISTENT/CHRONIC IMMUNE THROMBOCYTOPENIA

Waleed Ghanima S310

## LONG-TERM EFFICACY AND SAFETY STUDY OF RILZABRUTINIB, ORAL BRUTON TYROSINE KINASE INHIBITOR, IN PATIENTS WITH WARM AUTOIMMUNE HEMOLYTIC ANEMIA (WAIHA): LUMINA PHASE 2B PART B

Bruno Fattizzo S298

## Anchor Point Insight:

**With Sanofi's rilzabrutinib (cBTKi) likely set to receive its initial approval in ITP with an 8/29/25 FDA PDUFA date, data at EHA'25 demonstrated persistent efficacy with extended use and a favorable safety profile. Similarly, long-term FU in wAIHA also showcased a sustained efficacy benefit with good safety, with only 27% TRAEs.**

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## A PILOT STUDY OF ORELABRUTINIB TREATMENT IN REFRACTORY/RELAPSED AUTOIMMUNE HAEMOLYTIC ANAEMIA/EVANS SYNDROME

Rong Fu

PF1205

## Anchor Point Insight:

**With the success of rilzabrutinib, other companies developing BTKi's for cancer have explored their molecules in various non-malignant indications. At EHA'25, InnoCare's orelabrutinib showed potential in AIHA with early signs of efficacy. Publication only presentations for BeOne's zanubrutinib in wAIHA (PB3576) and Lilly's pirtobrutinib in ITP (PB3692) highlight broader interest in testing BTKi's in non-malignant settings.**

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**A PHASE 2 STUDY OF IANALUMAB IN PATIENTS WITH PRIMARY IMMUNE THROMBOCYTOPENIA PREVIOUSLY TREATED WITH AT LEAST TWO LINES OF THERAPY (VAYHIT3)**

Charlotte Bradbury — S312

**SAFETY AND EFFICACY OF CM313 IN ADULTS WITH IMMUNE THROMBOCYTOPENIA: A RANDOMIZED, PLACEBO-CONTROLLED TRIAL**

Yanmei Xu — LB4004

## Anchor Point Insight:

**New therapeutics in ITP have been advancing rapidly over recent years, and at EHA'25 we saw promising early- to mid-stage data for Novartis' ianalumab (BAFF-R mAb) and Keymed Biosciences' CM313 (CD38 mAb) potentially setting up the introduction of more novel MOA's into the future ITP treatment paradigm.**

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**THREE-YEAR SAFETY, EFFICACY, AND RENAL OUTCOMES OF MITAPIVAT TREATMENT IN SICKLE CELL DISEASE: RESULTS FROM A PHASE 2, OPEN-LABEL STUDY**

Geoffrey Kuppens S299

**SATISFY: MITAPIVAT IN ADULTS WITH ERYTHROCYTE MEMBRANOPATHIES AND CONGENITAL DYSERYTHROPOIETIC ANEMIA TYPE II: A EUROBLOODNET, MULTICENTER, SINGLE-ARM, PHASE 2 STUDY**

Thomas Doeven S297

## Anchor Point Insight:

**Mid-stage data for Agios' mitapivat, a pyrvuate kinase activator, in SCD and RBC membranopathies continue to highlight the potential of this novel mechanism to support patients across a variety of disease states impacting RBCs.**

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# Non-malignant Hematology Abstracts of Interest

**EFFICACY AND SAFETY OF BRL-101 IN TDT AND SCD**

Tan Qian S290

**TREATMENT OF PATIENTS WITH SEVERE TRANSFUSION-DEPENDENT ß-THALASSEMIA WITH CS-101, AN AUTOLOGOUS, EX VIVO EDITED, CD34+ HEMATOPOIETIC STEM CELL PRODUCT USING INNOVATIVE TRANSFORMER BASE EDITOR (TBE)**

Jia Chen S291

**UPDATED SAFETY AND EFFICACY RESULTS OF RM-001, AUTOLOGOUS HBG1/2 PROMOTER-MODIFIED CD34+ HEMATOPOIETIC STEM AND PROGENITOR CELLS, IN TREATING TRANSFUSION-DEPENDENT ß-THALASSEMIA**

Hui Xu PS2181

## Anchor Point Insight:

**Despite limited commercial success for the two currently approved cell therapies for TDT and SCD, EHA'25 featured interesting data from China-only studies for novel cell therapies focused on increasing HbF expression in these same indications.**

Anchor Point Insights