GFM clinical trials

October 2026

Groupe Francophone des Myélodysplasies

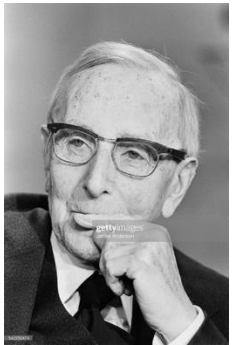
Pierre Fenaux, Fatiha Chermat



Université Paris Cité

UNIVERSITÉ PARIS 13





GFM clinical trials

- Higher risk MDS
  - First line
  - Second line or beyond
  - Allo SCT
  - CMML
  - MDS with TP 53 mutation

- Lower risk MDS
  - First line
  - Second line or beyond
  - Associated with autoimmune or auto inflammatory diseases /VEXAS

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Groupe Francophone des Myélodysplasies

Onureg-Ven : A phase 1b-2 study
(Colombe Saillard)

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Target population	Previously untreated higher-risk myelodysplastic syndromes ineligible for allogenic transplantation
Study design	Multicenter, I phase 1b-2 study
Objectives of the Trial	**Primary objective** in the phase 1b is to establish maximum tolerated dose (MTD) and determine recommended phase 2 dose-schedule (RP2DS) by evaluating safety and tolerability of Onureg (CC-486) and VENETOCLAX combination, in previously untreated patients with HR-MDS not eligible for transplant. When MTD/RP2D will be determined, phase 2 dose expansion part of the study will open for enrollment. **Primary objective in phase 2** is to assess preliminary efficacy (CR rate) of the R2PD of Onureg-VEN combination.

	Oral AZA	Venetoclax
Level -1	200 mg QD d1-d7	400 mg QD d1-d14
Level 1	200 mg QD d1-d14	400 mg QD d1-d14
Level 2	300 mg QD d1-d14	400 mg QD d1-d14

Leukemia www.nature.com/leu

ARTICLE

Ivosidenib monotherapy in *IDH1* mutated myelodysplastic neoplasm/syndrome

Marie Sébert[1,2], Emmanuelle Clappier[3], Sylvie Chevret[4], Hugo Bergugnat[3], Odile Rauzy[2,5], Aspasia Stamatoullas[2,6], Sophie Dimicoli-Salazar[2,7], Lamya Ait Si Selmi[2], Cendrine Chaffaut[4], Fatiha Chermat[2], Lise Larcher[3], Lauriane Goldwirt[8], Sylvain Thepot[2,9], Pierre Peterlin[2,10], Sophie Park[2,11], Marie-Pierre Gourin[2,12], Norbert Vey[2,13], Cécile Bally[14], Sébastien Maury[15], Gaelle Fossard[2,16], Maud D'Aveni[2,17], Anne-Laure Taksin[2,18], Thomas Cluzeau[19], Pierre Fenaux[1,2,20] and Lionel Adès[1,2,20]

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- **48 patients were included (median age 76.5 years)**
- **ORR after 3 cycles was 63.6% in cohort A, and 78.3% in cohort B;**
- **12-month OS rate in cohorts A and B was 18.2% and 91.3% , respectively.**
- **Higher baseline IDH1 mutant clone size predicted response**
  Molecular clearance was not required for clinical benefit.

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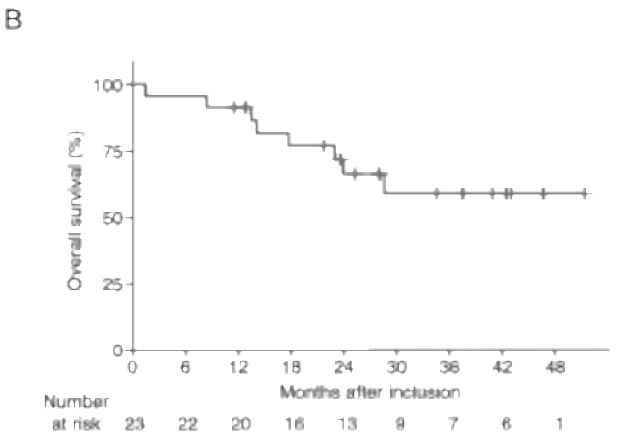


Fig. 5 Survival outcomes. A cohort A and **B** cohort B.

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Impress (cooperation with the German MDS group) (L Adès for the GFM)

- Imetelstat in higher risk MDS and AML having failed AZA (+/-Venetoclax)

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REGULAR ARTICLE

blood advances

Transplantation in patients with lower-risk MDS: a prospective phase 2 trial based on donor availability

Marie Sébert,[1] Sylvain Thepot,[2] Thomas Cluzeau,[3] Nicolas Duployez,[4] Thibaud Lefèbvre,[5] Cendrine Chaffaut,[6] Corentin Orvain,[2] Michael Loschi,[3] Pierre Peterlin,[7] Patrice Chevallier,[7] Maud D'Aveni,[8] Marie Thérèse Rubio,[8] Odile Rauzy,[9] Anne Huynh,[10] Amandine Charbonnier,[11] Rosa Sapena,[12] Fatiha Chermat,[12] Patrice Ceballos,[13] Gaelle Fossard,[14] Stéphanie Nguyen,[15] Sophie Park,[16] Lionel Adès,[1] Régis Peffault de Latour,[17] Claude Preudhomme,[4] Pierre Fenaux,[1,*] Sylvie Chevret,[6,*] and Marie Robin,[17] on behalf of Société Francophone de Greffe de Moelle et de Thérapie Cellulaire and the Groupe Francophone des myélodysplasies

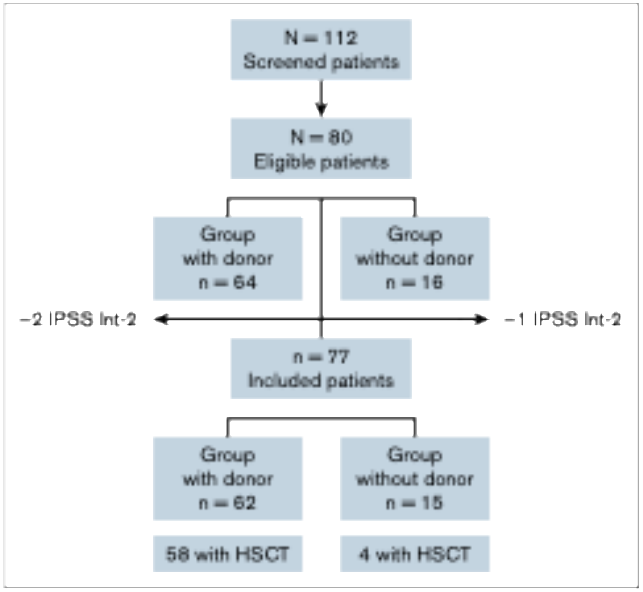
lower-risk MDS with additional high-risk features :

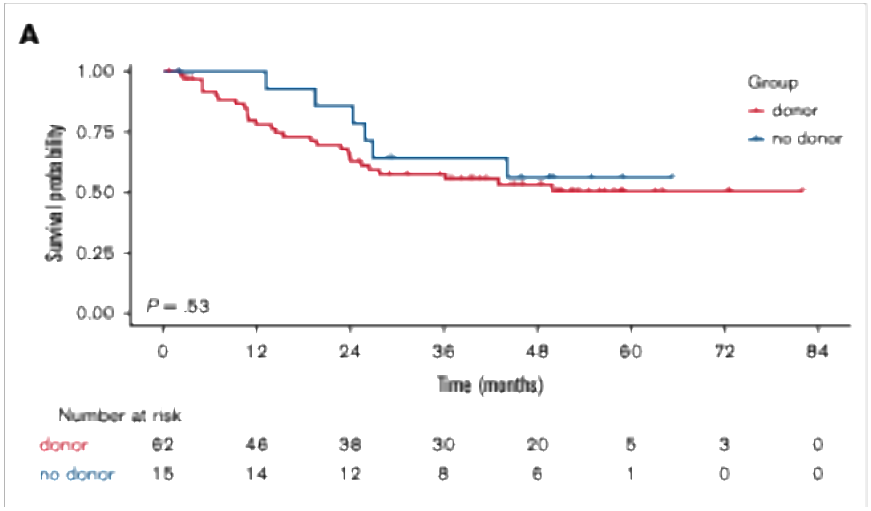
-intermediate or higher revised-IPSS risk

- Plts <20 × 10 9 /L, ANC <0.5 × 10 9 /L,

- failure of 2 lines of therapy.

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Allo SCT in MDS

1) VENTOGRAFT: Venetoclax + AZA + DLI in MDS relapsing post allo SCT (T Cluzeau,M Robin)

2)Upfront allo SCT in patients with marrow blasts <15% (M Robin)

3) VEXAS (M Robin, LP Zhao, A Mekinian)

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VENTOGRAFT, a Phase I/II prospective trial of Azacitidine, Venetoclax and Donor Lymphocyte Infusion for Relapsed AML and MDS after Allogeneic Stem Cell Transplantation

Thomas Cluzeau 1, Marie Sebert 2, Coralie Cruzel 3, Anne Huynh 4, Sylvain Thepot 5, Martin Carre 6, Hélène Labussiere-Wallet 7, Aspasia Stamatoullas 8, Clémence Mediavilla 9, Alice Garnier 10, Pascal Turlure 11, Ludovic Gabellier 12, Raphael Petit 13, Rosa Sapena 13, Fatiha Chermat 13, Claude Preudhomme 14, Lionel Ades 2, Nicolas Duployez 14, Eric Fontas 3, Pierre Fenaux 2 and Marie Robin 15

N=54

Groupe Francophone des Myélodysplasies

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Advanced CMML(R Itzykson, C Willekens)

- AZA + VEN (AVENHIR trial)
- AZA+Lenzilumab (GM-CSF inhibitor)
- Adjusted dose Hydroxyurea

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Higher risk MDS with TP53 mutation

(T Cluzeau)

- *First line*: AZA+ oral ATO

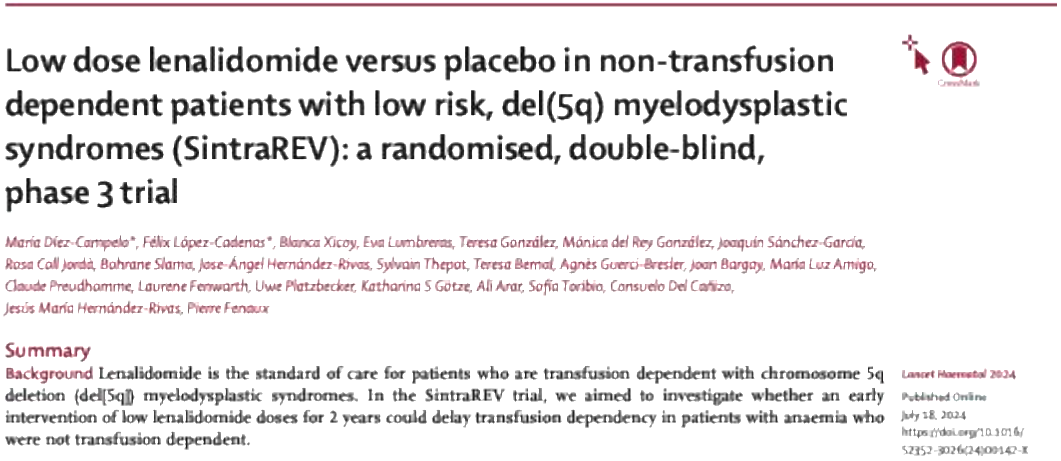
- *Second line*: AZA+Rezatapopt (Y220C mutation)

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Low dose lenalidomide versus placebo in non-transfusion dependent patients with low risk, del(5q) myelodysplastic syndromes (SintraREV): a randomised, double-blind, phase 3 trial

María Díez-Campelo, Félix López-Cadenas*, Blanca Xicoy, Eva Lumbreras, Teresa González, Mónica del Rey González, Joaquín Sánchez-García, Rosa Coll Jordà, Bahrane Slama, José-Ángel Hernández-Rivas, Sylvain Thepot, Teresa Bernal, Agnès Guerci-Bresler, Joan Bargay, María Luz Amigo, Claude Preudhomme, Laurene Fenwarth, Uwe Platzbecker, Katharina S Götze, Ali Arar, Sofía Toribio, Consuelo Del Cañizo, Jesús María Hernández-Rivas, Pierre Fenaux*

Summary

Background Lenalidomide is the standard of care for patients who are transfusion dependent with chromosome 5q deletion (del[5q]) myelodysplastic syndromes. In the SintraREV trial, we aimed to investigate whether an early intervention of low lenalidomide doses for 2 years could delay transfusion dependency in patients with anaemia who were not transfusion dependent.

Lancet Haematol 2024
Published Online
July 18, 2024
https://doi.org/10.1016/
S2352-3026(24)00142-X

- Patients with Hb <12g, no RBC TD

- randomized between LEN (5mg/d) or placebo

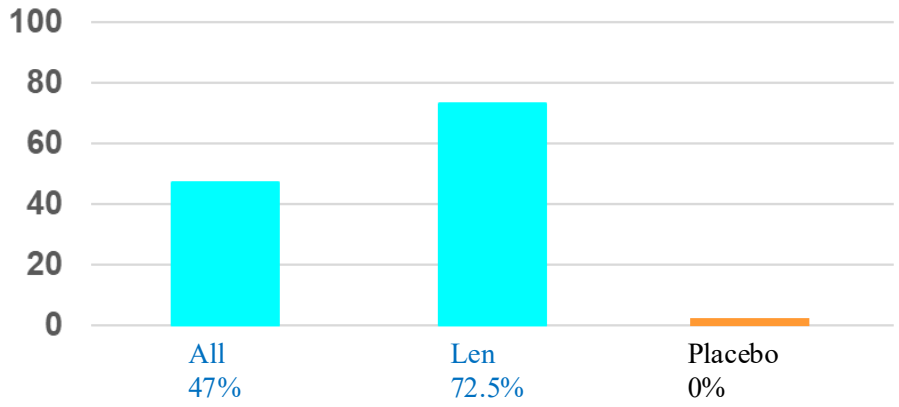
GESMD

Groupe Francophone des Myélodysplasies

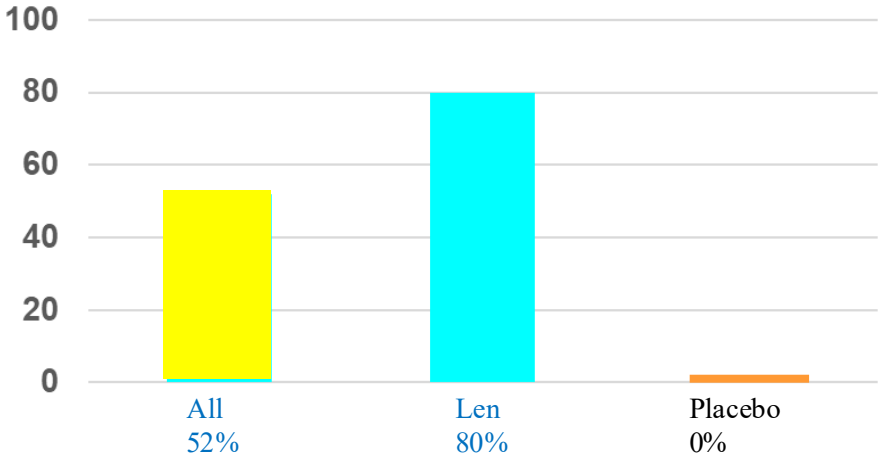
Sintra-Rev Clinical Trial

Low doses of Len improved Erythroid and Cytogenetic responses

- **Erythroid Response**



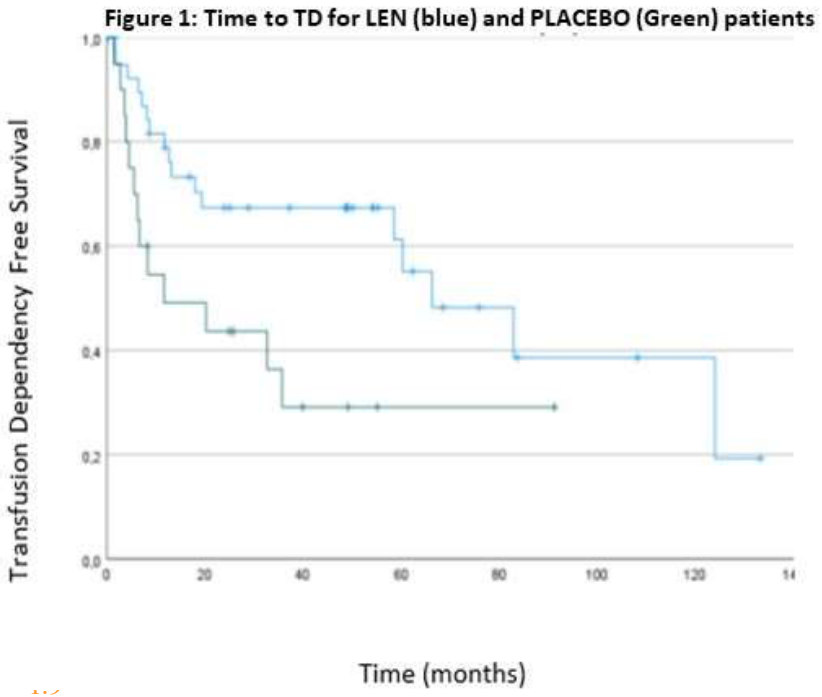
- Cytogenetic Response



Sintra-Rev Clinical Trial

Low doses of Len delayed and decreased transfusion dependency

Median time to Transfusion Dependency (median 66.3 vs 11.6 months)



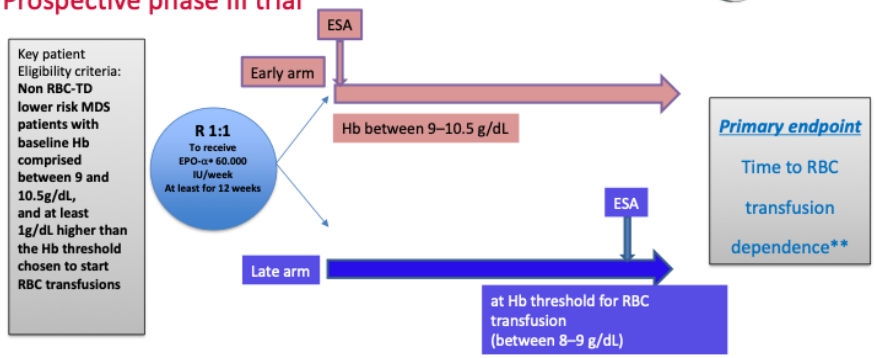
American Society *of* Hematology
Helping hematologists conquer blood diseases worldwide

Early Versus Late Onset of ESA in Lower Risk Anemic MDS Patients: Results of the GFM Randomized Phase III EPO-PRETAR Trial

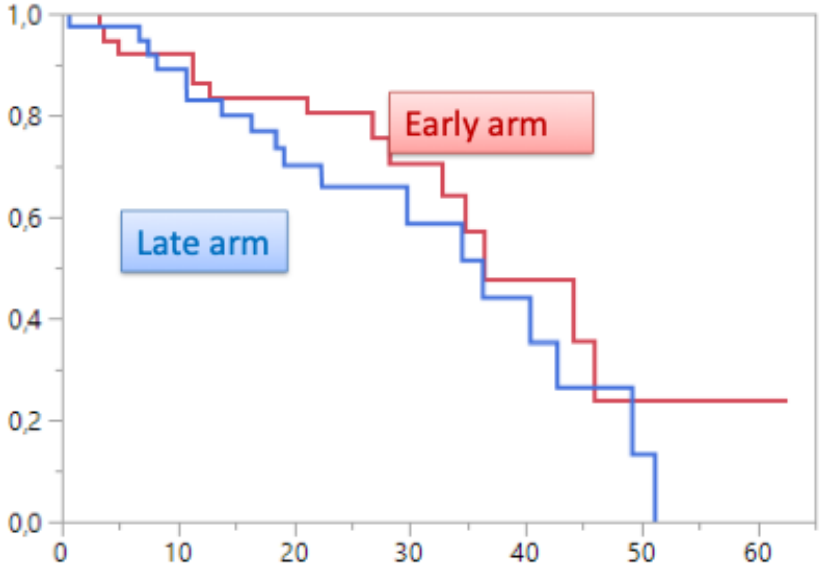
Sophie Park, Bohrane Slama, Marius Moldovan, David Rombaut, Marie Templé, Aspasia Stamatoullas, Maud D'Aveni, Stephane Cheze, Sylvain Thepot, Jose Miguel Torregrosa Diaz, Sophie Dimicoli, Mathieu Meunier, Kamel Laribi, Reza Tabrizi, Anne Banos, Alice Garnier, Celia Salanoubat, Adrien Contejean, Mael Heiblig, Laurent Pascal, Clemence Santana, Ali Arar, **Ramdane Tekrane, Fatiha Chermat, Olivier Kosmider, Michaela Fontenay, and Pierre Fenaux**

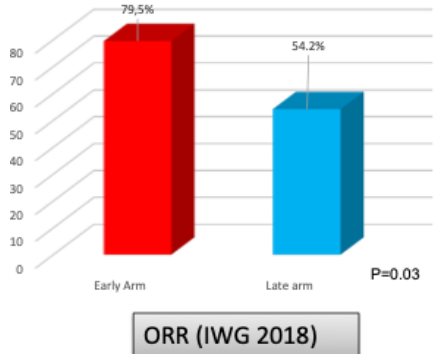
For the Groupe Francophone des Myélodysplasies

GFM-EPO-PRETAR: Prospective phase III trial

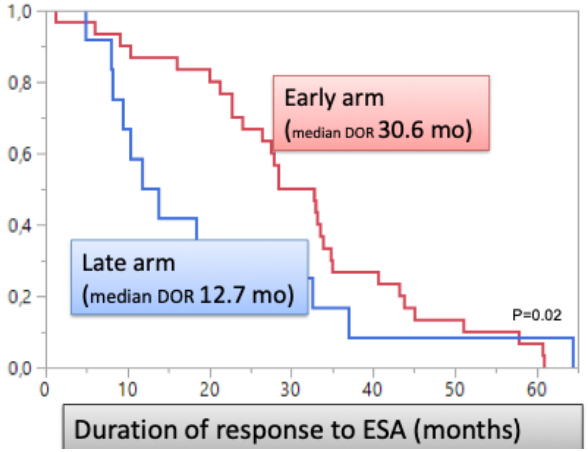


S PARK





ORR (IWG 2018)



Duration of response to ESA (months)

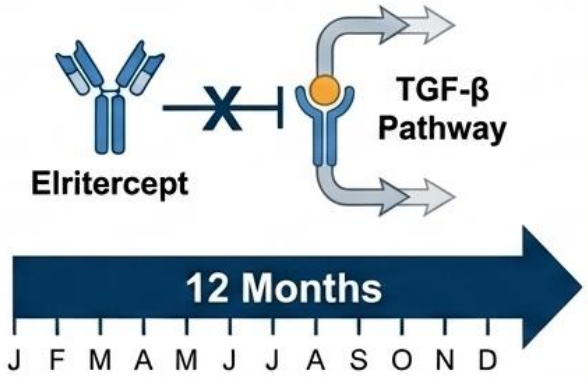
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Lower-Risk MDS — Upcoming First-Line Trial

(T Cluzeau, L Adès)

ELIOT
(Upcoming First-Line Phase II)

- **Patient Profile:** Newly diagnosed WHO 2022 LR-MDS without ring sideroblasts, naïve to ESAs and luspatercept, transfusion-dependent, no del(5q).
- **Intervention:** Elritercept, 3.75-5.0 mg/kg SC Q4W) for 12 months.



Composite Primary Endpoint (within 24 weeks):

RBC-Transfusion Independence (RBC-TI) ≥ 8 consecutive weeks

AND

Hemoglobin increase >1.5 g/dL sustained for ≥ 8 consecutive weeks

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Gemini Notebook

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haematologica
Journal of the Ferrata Storti Foundation

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Low-dose deferasirox for lower-risk myelodysplastic syndromes after erythropoiesis-stimulating agents (ESA) failure: results of the phase II LODEFI study

by Mathieu Meunier, Sylvain Thepot, Stanislas Nimubona, Mélanie Veloso, Thibaud Lefebvre, Zoubida Karim, Martine Ropert-Bouchet, Frederic Garban, Shanti Amé, Claire Reynes, Celia Salanoubat, Vincent Parinet, Kamel Laribi, Pirayeh Effekhari, Jacques Delaunay, Geoffroy Venton, Clémence Santana, Stéphane Cheze, Sophie Dimicoli-Salazar, Andréa Toma, Mathieu Molimard, Pierre Fenaux, Valérie Rolland-Neyret, Sandra David-Tchouda and Sophie Park

Received: June 13, 2026.
Accepted: September 15, 2026.

M MEUNIER, S PARK

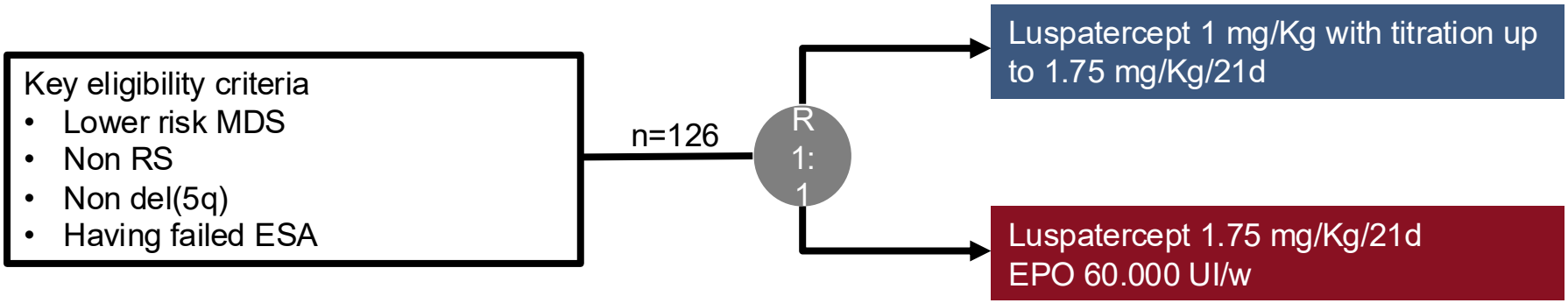
- Low Deferosirox concentrations < 7.5 µM enhance erythroid progenitor proliferation in MDS lower concentration of 3 µM selected, corresponding approximately to 5 mg/kg/day,
- 38 patients with ESA failure enrolled , 81% very low/low IPSS-R, 86.8% were not transfusion dependent

- .Median baseline Hb 86 g/L Baseline ferritin between 200 and 1000
- oral deferasirox at a starting dose of 3.5 mg/kg/day, with plasma DFX concentrations to adjust dosing toward a target concentration of 3 µM

- At 12 months of treatment, 18 of 38 patients (47.4%; 95% confidence interval [CI], 31.0–64.2) remained transfusion independent, with a median hemoglobin level of 88 g/L (range, 74–108),

Combola trial (L Adès)

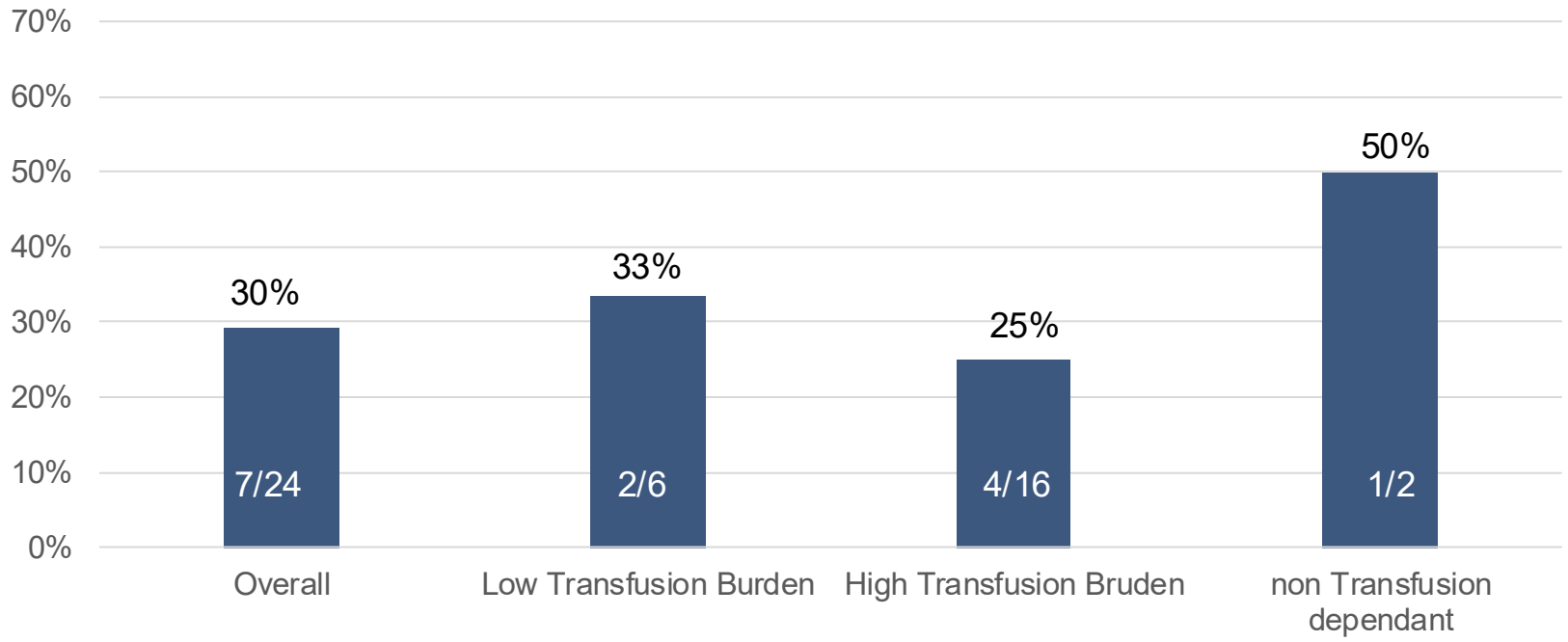
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Combining ESA and Luspatercept in Non-RS MDS Patients Having Failed ESA - Results of the Phase 1-2 Part a of the GFM Combola Study (Adès et al, ASH 2024)

Erythroid Response (IWG 2018) @w25 n=24

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2 patients achieved platelet response and 1 neutrophil response

Received: 28 March 2025 | Accepted: 20 June 2025
DOI: 10.1111/bjh.20247

BJHaem

RESEARCH LETTER

GFM Registries

Efficacy and safety of luspatercept with or without erythropoiesis-stimulating agent in patients with myelodysplastic neoplasms with ring sideroblasts: A multicentre real-life study by the GFM

M DAVENI, T COMONT

- Luspatercept alone:

171 RBC- TD patients (55 with LTB and 116 with HTB)

55 (32%) achieved TI by week 24 (64% of LTB and 17% of HTB patients)

41% in the 133 pts who received at least 7 injections

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- Luspa + EPO : 54 patients

HI- E obtained in 11 patients (20%): 0/4, 4/10 (40%) and 7/40 (18%) of NTD, LTB and HTB patients respectively)

29% of the 38 patients who received at least 16 weeks of combination treatment

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Ruxolitinib better than JAK2 i ?

TO THE EDITOR:

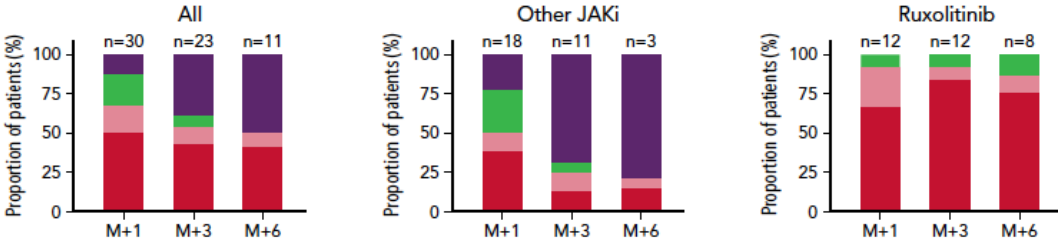
Ruxolitinib is more effective than other JAK inhibitors to treat VEXAS syndrome: a retrospective multicenter study

Maël Heiblig,[1] Marcela A. Ferrada,[2,*] Matthew T. Koster,[3,*] Thomas Barba,[4,*] Mathieu Gerfaud-Valentin,[5] Arsène Mékinian,[6] Henrique Coelho,[7] Gaelle Fossard,[1] Fiorenza Barraco,[1] Lionel Galicier,[8] Boris Bienvenu,[8] Pierre Hirsch,[9] Guillaume Vial,[10] Anne Blandine Boutin,[11] Joris Galland,[12] Guillaume Le Guenno,[13] Adrien Bigot,[14] Kenneth J. Warrington,[3] Tanaz A. Kermani,[15] Peter C. Grayson,[2] Bhavisha A. Patel,[16] David B. Beck,[17,18] Yvan Jamilloux,[5,†] Pierre Fenaux,[19,†] and Pierre Sujobert[20]

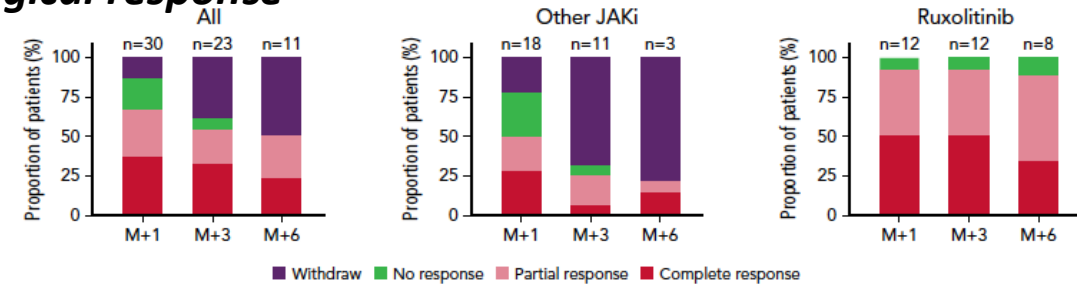
blood® 25 AUGUST 2022 | VOLUME 140, NUMBER 8 927

FRENVEX

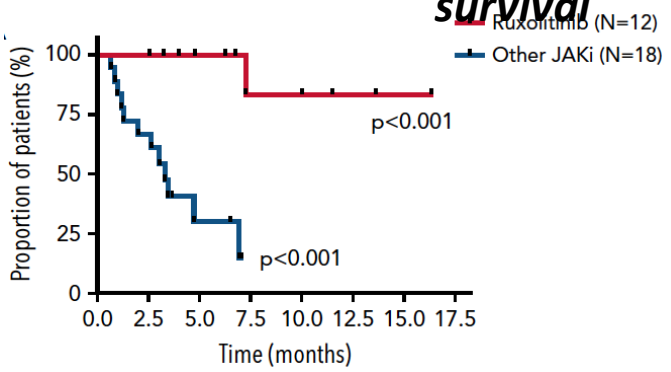
inical response



ological response



New treatment-free survival



Heiblig et al., Blood, 2022

blood® Regular Article

CLINICAL TRIALS AND OBSERVATIONS

Efficacy and safety of azacitidine for VEXAS syndrome: a large-scale retrospective study from FRENVEX

Vincent Jachiet,[1] Olivier Kosmider,[2,*] Maxime Beydon,[3,*] Jérôme Hadjadj,[1] Lin-Pierre Zhao,[4] Vincent Grobost,[5] Valentin Lacombe,[6] Guillaume Le Guenno,[5] Yann Nguyen,[7] Jean-Benoît Arlet,[8] Jérémie Dion,[9] Maël Heiblig,[10] Alice Garnier,[11] Maxime Samson,[12] Achille Aouba,[13] Sylvain Thépot,[14] Sophie Dimicoli-Salazar,[15] Fabien Dutasta,[16] Benoît Faucher,[17] Estibaliz Lazaro,[18] Véronique Morel,[19] Antoine Néel,[20] Roderau Outh,[21] Holy Bezanahary,[22] Julien Rossignol,[23] Anne-Sophie Alary,[24] Audrey Bidet,[25] Pauline Blateau,[26] Anne Bouvier,[27] Guilaine Boursier,[28] Matthieu Decamp,[29] Benjamin Lebecque,[30] Yannick Le Bris,[31] Pierre Sujobert,[32] Alice Marceau-Renaut,[33] Cédric Pastoret,[34] David Rizzo,[35] Nathalie Boiret-Dupré,[30] Lara Boucher,[2] Stéphanie Dulucq,[25] Franck Genevieve,[27] Cassandra Jadeau,[35] Pierre Lemaire,[36] Romain Vazquez,[2] Jean-Baptiste Rieu,[37] Olivier Fain,[1] Sophie Georgin-Lavialle,[38] Lucie Rigolot,[37] Lise Larcher,[36] Pierre Hirsch,[39] Benjamin Terrier,[40] Pierre Fenaux,[4,†] Arsène Mékinian,[1,†] and Thibault Comont,[9] on behalf of FRENVEX

FRENVEX

Groupe Francophone des Myélodysplasies

V JACHIET , T COMONT

- 88 pts, 80% SMD
- 54% et 61%

CLINICAL TRIALS AND OBSERVATIONS

Efficacy and safety of azacitidine for VEXAS syndrome: a large-scale retrospective study from FRENVEX

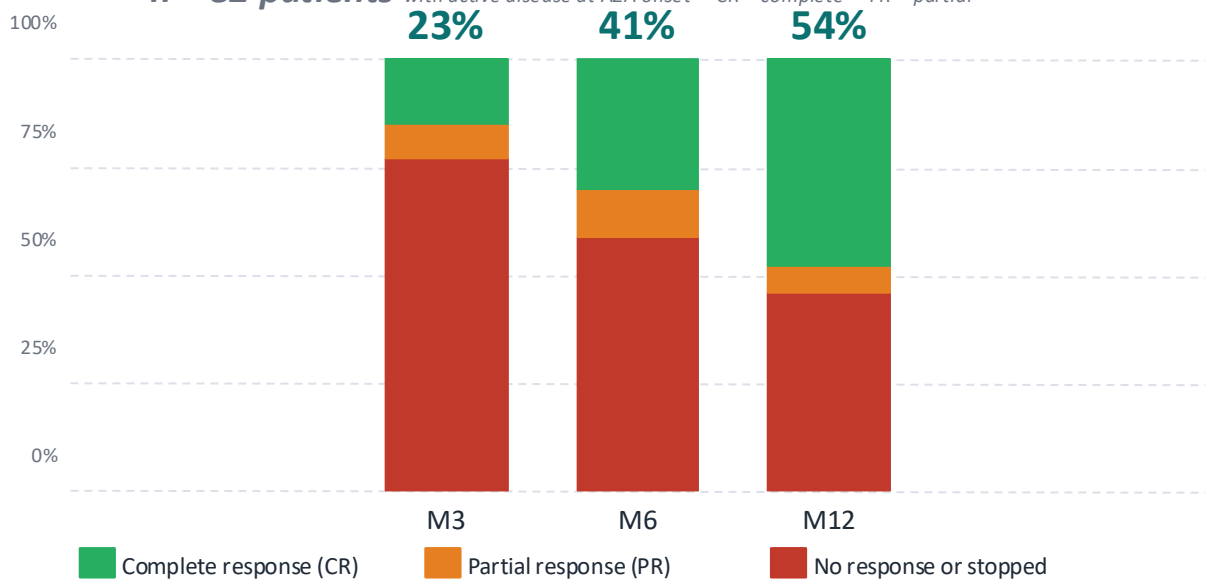
Vincent Jachiet,[1] Olivier Kosmider,[2,*] Maxime Beydon,[3,*] Jérôme Hadjadj,[1] Lin-Pierre Zhao,[4] Vincent Grobost,[5] Valentin Lacombe,[6] Guillaume Le Guenno,[5] Yann Nguyen,[7] Jean-Benoît Arlet,[8] Jérémie Dion,[9] Maël Heiblig,[10] Alice Garnier,[11] Maxime Samson,[12] Achille Aouba,[13] Sylvain Thépot,[14] Sophie Dimicoli-Salazar,[15] Fabien Dutasta,[16] Benoît Faucher,[17] Estibaliz Lazaro,[18] Véronique Morel,[19] Antoine Néel,[20] Roderau Outh,[21] Holy Bezanahary,[22] Julien Rossignol,[23] Anne-Sophie Alary,[24] Audrey Bidet,[25] Pauline Blateau,[26] Anne Bouvier,[27] Guillaine Boursier,[28] Matthieu Decamp,[29] Benjamin Lebecque,[30] Yannick Le Bris,[31] Pierre Sujobert,[32] Alice Marceau-Renaut,[33] Cédric Pastoret,[34] David Rizzo,[35] Nathalie Boiret-Dupré,[30] Lara Boucher,[2] Stéphanie Dulucq,[25] Franck Genevieve,[27] Cassandra Jadeau,[35] Pierre Lemaire,[36] Romain Vazquez,[2] Jean-Baptiste Rieu,[37] Olivier Fain,[1] Sophie Georgin-Lavialle,[38] Lucie Rigolot,[37] Lise Larcher,[36] Pierre Hirsch,[39] Benjamin Terrier,[40] Pierre Fenaux,[4,†] Arsène Mékinian,[1,†] and Thibault Comont,[9] on behalf of FRENVEX

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FRENVEX

Comparable response in MDS and non-MDS VEXAS

n = 82 patients *with active disease at AZA onset · CR = complete · PR = partial ·*



Delayed response: 30% of responders reach response after M6

Jachiet V, et al. Blood 2025 (n = 88)

Haematological response (IWG 2018)

69% HI-E · erythroid	**65%** RBC-TI · transfusion indep.
77% HI-P · platelets	**78%** HI-N · neutrophils

Early HI predicts subsequent inflammatory response

HI-P at M3: 100% vs 44% ($p < 0.001$)
HI-E at M3: 54% vs 15% ($p = 0.002$)

Durability

On AZA: RFS 85% at 5 years

Off AZA: **75% relapse — median 3.1 years → suspensive therapy**

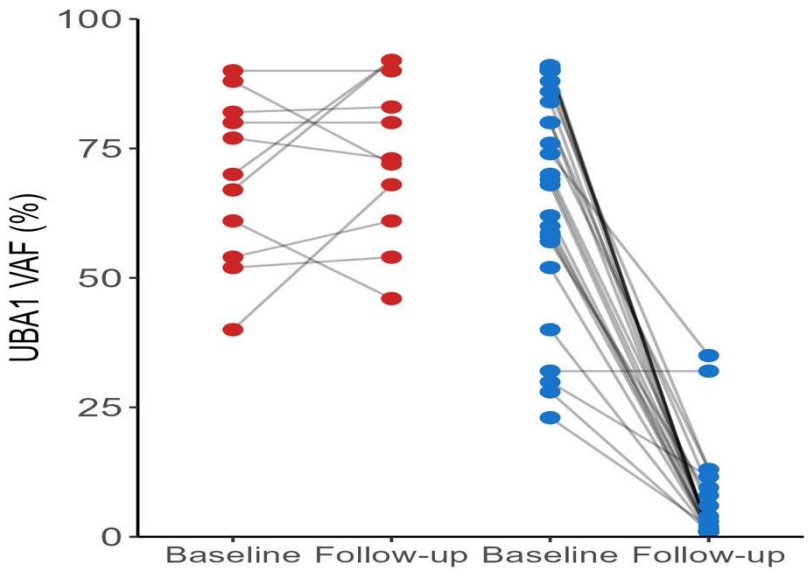
Azacitidine: disease-modifying therapy

Molecular response — UBA1 VAF reduction

65%
≥25% reduction in UBA1 VAF

43%
VAF reduction below 2%

Correlation:
molecular response ↔ inflammatory response & HI-E



Jachiet V, et al. Blood 2025 (n = 88)

Clinical trials in VEXAS (+/- MDS)

Vexas

- Momelotinib (M Heiblig)
- ASTX 030 (T Comont)
- Allogeneic SCT (M Robin, LP Zhao)





Department of hematology and immunology of Hospitals St Louis, R Debré, Avicenne APHP and University of Paris

Hôpital St Louis

- 7 services of adult hematology (H Dombret, N Boissel, G Socié, B Arnulf, E Oksenhendler, P Fenaux, C Thiéblemont)
- ICU (E Azoulay)
- pneumology (A Tazi)

Hôpital Robert Debré

- pediatric hématology service (A Baruchel)
- Sickle cell disease unit(M Benkerrou)

Hôpital Avicenne

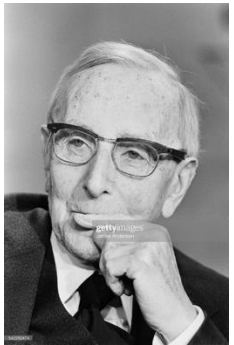
- Adult hematology service (C Gardin)



Université de Paris

UNIVERSITÉ PARIS 13





Groupe Francophone des Myélodysplasies

- Activates clinical trials in MDS (35 centers in France and Belgium
  + Switzerland)

- Website: www. gfmgroup.org

- Online registry of French MDS cases

- Close cooperation with:
  - *a patient support group: CCM (recently involved in Vexas)*
  - the International MDS Foundation
  - the European Leukemia Net

Groupe Francophone des Myélodysplasies

FRENVEX Group

Médecine interne, St Antoine



Jérôme HADJADJ



Arsène MEKINIAN



Olivier FAIN

AZA team



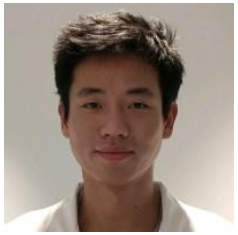
Thibault COMONT



Olivier KOSMIDER



Pierre FENAUX



Lin-Pierre ZHAO

Arsène MEKINIAN
Sophie GEORGIN
LAVIALLE
Benjamin TERRIER
Olivier KOSMIDER

CHASSET François (Paris)
LACOMBE Valentin (Angers)
BOUAZIZ Jean-David (Paris)
BORIE Raphaël (Paris)
MATHIAN Alexis (Paris)
ALLENBACH Yves (Paris)
MELKI Isabelle (Paris Debré)
RODRIGUES François (Paris)
BEYNE-RAUZY Odile (Toulouse)
DION Jérémie (Toulouse)
EL-KAROUI Khalil (Paris)
TERRIOU Louis (Lille)
EBBO Mikael (Marseille)
BOURGUIBA Rim (Tunisie)
ROBIN Marie (Paris)

AUDEMARD Alexandra (Tours)
AOUBA Achille (Caen)
GUILPAIN Philippe (Montpellier)
LAZARO Estibaliz (Bordeaux)
MOULINET Thomas (Nancy)
JAMILLOUX Yvan (Lyon)
BELOT Alexandre (Lyon)
HEIBLIG Mael (Lyon)
SUJOBERT Pierre (Lyon)
ARNAUD Laurent (Strasbourg)
BENHAMOU Ygal (Rouen)
SAUVETRE Gaetan (Rouen)
SAMSON Maxime (Dijon)
OUTH Roderau (Perpignan)
LOBBES Herve (Clermont-Ferrand)

The "PP": Benjamin Terrier, Jerôme Hadjadj, Thibault Comont, Valentin Lacombe VNI, Lin-Pierre Zhao, Paul Breillat, Stanislas Riescher & Chloé McAvo


EMSCO
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SYNDROMES


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Myélodysplasies


FISM
FONDAZIONE
ITALIANA
SINDROMI
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ONGOING ACADEMIC CLINICAL TRIALS IN MDS ACROSS THE EU

Spain

María Diez Campelo, MD PhD



Low-risk MDS Platforms

Evidence-driven | Multicenter / National | Patient-centered | Improving outcomes in LR-MDS

CLINICAL STUDY 1: MDS-QUAL (NCT06614595)

OBJECTIVE

Evaluate the impact of anemia and transfusion burden on QoL and identify predictors of transfusion benefit.

ELIGIBLE PATIENTS

- LR-MDS (very low/low/intermediate IPSS-R)
- RBC transfusion-dependent
- Adults ≥18 years
- ECOG 0–3
- n = 30 patients

STUDY DESIGN

Prospective • Multicenter
Observational cohort study

KEY ASSESSMENTS

- QoL / QUALMS and PROs
- Functional evaluation (cardiorespiratory, ergospirometry)
- Cardiac biomarkers (troponin, NT-proBNP)
- Cytokine profiling (plasma)
- Hemoglobin and transfusion burden

EXPECTED IMPACT

Develop a predictive model to identify patients who benefit from RBC transfusions and personalize transfusion strategies.

CLINICAL TRIAL 2: LENALIDOMIDE DISCONTINUATION IN DEL(5Q) LR-MDS

OBJECTIVE

Evaluate treatment discontinuation strategies after sustained response to lenalidomide.

ELIGIBLE PATIENTS

- LR-MDS with del(5q)
- Sustained response to lenalidomide

STUDY DESIGN

National • Phase III
Investigator-initiated clinical trial

KEY ASSESSMENTS

- Molecular analyses
- Immune profiling
- Quality-of-life assessments
- Evaluation of treatment-free period

EXPECTED IMPACT

Identify clinical and biological determinants of durable responses and support a more personalized, toxicity-sparing treatment approach.

GOAL: Advance evidence-based, patient-centered strategies to improve outcomes and quality of life for patients with low-risk MDS.

High-risk MDS Platforms

Innovative therapies | Precision medicine | Safety first | Improving outcomes in HR-MDS

CLINICAL TRIAL 3: CD33 CAR-T for R/R High-risk MDS
First-in-Human Academic Phase I/II Trial

OBJECTIVE

Evaluate the safety, feasibility and preliminary efficacy of allogeneic CD33 CAR-T therapy in relapsed/refractory high-risk MDS.

ELIGIBLE PATIENTS

- Relapsed/Refractory high-risk MDS
- Prior treatment failure or no standard therapeutic options

STUDY DESIGN

- First-in-human academic phase I/II trial
- Dose escalation (Phase I)
- Dose expansion (Phase II)
- Advanced regulatory preparation

KEY ASSESSMENTS

- Response rate and duration
- Safety and tolerability
- Molecular response by sensitive techniques (e.g., NGS, flow cytometry, qPCR)
- Long-term follow-up

EXPECTED IMPACT

Establish safety and preliminary efficacy of allogeneic CD33 CAR-T in high-risk MDS and lay the foundation for future late-phase trials.

CLINICAL STUDY 4: Aza + Ven Bridge-to-Transplant
Prospective MRD-Guided Protocol

OBJECTIVE

Assess azacitidine plus venetoclax as bridge-to-transplant therapy in high-risk MDS candidates for allogeneic HSCT using MRD-guided decision-making.

ELIGIBLE PATIENTS

- High-risk MDS
- Candidates for allogeneic HSCT

STUDY DESIGN

- Prospective clinical study
- Azacitidine + venetoclax as bridge-to-transplant
- MRD-guided decision-making

KEY ASSESSMENTS

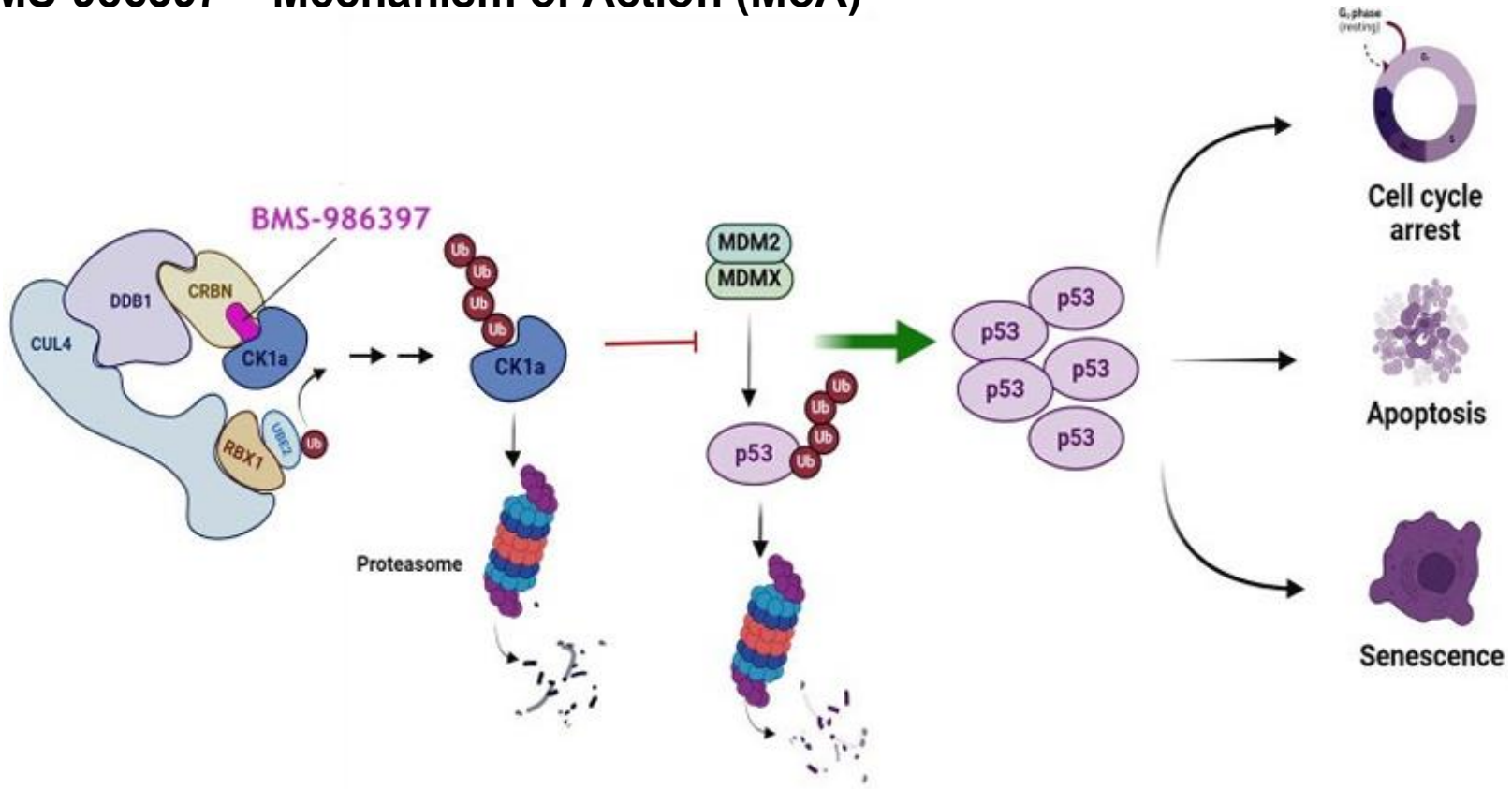
- Response depth and MRD dynamics
- Toxicity and safety
- Transplant readiness
- Quality of life (QoL)

EXPECTED IMPACT

Improve response depth and transplant readiness through MRD-guided therapy and integrated assessment of efficacy, safety and QoL.

GOAL: Advance innovative, patient-centered strategies to improve outcomes and survival for patients with high-risk MDS.

BMS-986397 – Mechanism of Action (MoA)



- BMS-986397 is a first-in-class **CK1α degrader** using a **CELMoD mechanism.**
- Selective CK1α degradation via **p53 activation.**
- Induces cell-cycle arrest, apoptosis, and senescence in leukemia cells.
- Dose-dependent target engagement.
- Strong biological rationale for **TP53 WT HR-MDS/AML.**

BMS-986397 in R/R AML and High-Risk MDS: First-in-Human (FIH) Phase 1 Study

Phase I | Open-label | Multicenter | Single-arm

BACKGROUND

BMS-986397 is a first-in-class CK1α degrader that activates p53 and promotes leukemic blast cell death.

FIH study in patients with R/R AML or HR-MDS with limited options.

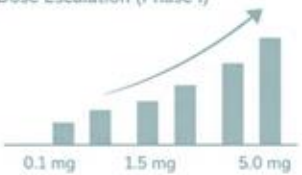
52 patients treated

AML: 38 (73%)
HR-MDS: 14 (27%)

STUDY DESIGN (METHODOLOGY)

28-day cycles
Oral, once daily (QD)
Days 1–3, 1–5, or 1–7

Dose Escalation (Phase I)



Primary: Safety, MTD/RP2D

Secondary: Preliminary efficacy, PK

Exploratory: PD biomarkers (CK1α degradation, p53, MIC-1)

DLT window: Cycle 1 (28–42 days)

RESULTS

SAFETY

TEAEs in 98%
Grade 3/4 in 90%

Most common Grade 3/4 (≥10% of patients)

Thrombocytopenia	42%
Anemia	39%
Neutropenia	33%
Asthenia	35%
Diarrhea	35%
ALT increase	31%

DLTs in 4 patients (7.7%)

- QT prolongation (2)
- Prolonged cytopenias (2)

PK / PD

Exposure

Dose-dependent exposure

Steady state by Day 5

~15% accumulation (Day 1 to Day 5)

Target Engagement & PD

≥1.5 mg: up to 90% CK1α degradation

≥2.2 mg: sustained p53 stabilization

Dose-dependent ↑ MIC-1

Translates to rapid reduction in proliferation (Ki-67) in circulating blasts

EFFICACY

Best Response

AML (n=38)



HR-MDS (n=14)



3/4 (75%) AML responders had sAML from prior MDS

CONCLUSIONS

BMS-986397 was generally well tolerated at doses up to 5.0 mg.

Demonstrated target engagement, p53 activation, and anti-leukemic activity in R/R AML and HR-MDS.

RP2D established.

Expanding in Phase II.

GOAL: Develop BMS-986397 as a potential new therapy option for patients with R/R AML and high-risk MDS.

Sponsor: Bristol Myers Squibb

DiNardo et al., Blood, 2025.

BMS-986397 + Azacitidine in TP53 Wild-Type HR-MDS

Phase I/II | Open-label | Multicenter | Single-arm

OBJECTIVE

Evaluate safety, tolerability, RP2D and preliminary efficacy of BMS-986397 + azacitidine in previously untreated HR-MDS (TP53 wild-type)

Phase I (Dose Escalation)
- Safety and tolerability
- Determine RP2D

Phase II (Expansion)
- Preliminary efficacy
- CR rate (IWG 2023) as primary endpoint

ELIGIBLE PATIENTS

- Adults ≥18 years
- HR-MDS
- *TP53* wild-type
- IPSS-M: moderate-high, high or very high risk
- Previously untreated (no prior disease-modifying therapy)

Total planned sample size: ≈ 30–32 patients

Phase I: 9–12
Phase II: ~20

Approximately 5 sites in Spain

STUDY DESIGN (METHODOLOGY)

PART A
Dose Escalation (Phase I)

BOIN design

n = 9–12 patients

DL-1 (0.8 mg) → DL1 (1.5 mg) → DL2 (2.2 mg)

Evaluate DLTs, safety and tolerability

RP2D

PART B
Dose Expansion (Phase II)

Preliminary efficacy evaluation

n ≈ 20 patients

Assess efficacy at RP2D

BMS-986397 Oral | Days 1–5 + **Azacitidine** Subcutaneous | Days 1–7

28-day treatment cycles
Until disease progression, loss of clinical benefit, unacceptable toxicity, withdrawal of consent, death or study termination

OUTCOMES

PRIMARY ENDPOINTS

Phase I
- Safety, DLTs, RP2D

Phase II
- CR rate (IWG 2023)

SECONDARY ENDPOINTS

- Overall response rate (ORR)
- Hematologic improvement (HI)
- Transfusion independence (TI)
- Duration of response (DoR)
- Event-free survival (EFS) / Overall survival (OS)
- Patient-reported outcomes (PROs)

GOAL: Improve outcomes and quality of life for patients with HR-MDS through an effective and well-tolerated treatment strategy.

Safety oversight by Safety Review Committee (SRC)

Sponsor: GESMD / SEHH



Clinical Trials of the D-MDS Study Group

- *Katharina Götze, MD*
- *Associate Professor of Medicine*
- *Dept. of Medicine III, Hematology/Oncology*
- *Technical University of Munich School of Medicine and Health*

Updated D-MDS Organizational Structure

Chair





D·MDS
Deutsche MDS-Studiengruppe

K. Götze
W.-K. Hofmann

Steering Committee


U. Germing


A. Giagounidis


D. Haase


T. Illmer


K. Sockel


S. Saußele


F. Thol

D·MDS
Deutsche MDS-Studiengruppe

D-MDS Working Groups

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T. Illmer
S. Saußele
K. Sockel
F. Thol

Coordination

Beatrice Berneck

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D. Sasca

Clinical Research
S. Saußele
A. Hecht-So

Stem Cell Transplantation and Cellular Therapies
G. Bug
C. Kobbe

Translational Research
F. Thol
C. Khandanpour

Registries & Biobanking
U. Germing
T. Illmer

Active interventional Studies (according to AMG/CTR)

Acronym	Title	IMP	Countries	Sample size	Status	Presented
LUSPLUS	*A phase IIIb, open-label, single arm study to evaluate the efficacy and safety of luspatercept in patients with lower-risk MDS and ring-sideroblastic phenotype (MDS-RS)*	**Luspatercept**		N=55 (single-arm)	recruitment completed **N=58**	**ASH 2024** Manuscript pending
LENNON	*A phase II, open-label, single arm study to evaluate the efficacy of luspatercept in erythropoiesis-stimulating agent naive lower-risk MDS patients with or without ring sideroblasts who do not require RBC transfusions*	**Luspatercept**		N=30 (single-arm)	recruitment completed prematurely **N=18/30**	**ASH 2026** Manuscript pending
PALOMA	*Primary comparison of liposomal anthracycline based treatment versus conventional care strategies before allogeneic stem cell transplantation in patients with higher risk MDS and oligoblastic AML*	**CPX-351** (Vyxeos® vs. CCR)		N=150 (two-arm)	recruitment completed N=150	**EHA 2026** Manuscript pending
PATROL	*A Phase II study of Azacitidine (AZA) combined with Venetoclax (VEN) and Tagraxofusp (TAG) in patients with higher higher-risk chronic myelomonocytic leukemia (CMML)*	**Tagraxofusp** (+ AZA/VEN)		N=24 (single-arm)	Recruiting **N=10**	ongoing
AZALOX	*A Phase Ib/II multicenter open-label study evaluating the safety and efficacy of escalating doses of PXS-5505 in combination with 5-Azacitidine for pan LOX/LOXL inhibition in patients diagnosed with Myelodysplastic Neoplasms (MDS) or Chronic Myelomonocytic Leukemia (CMML)*	**PXS 5505** (+ AZA)		N=42 (single-arm)	**Recruiting** **N=5**	ongoing

LENNON

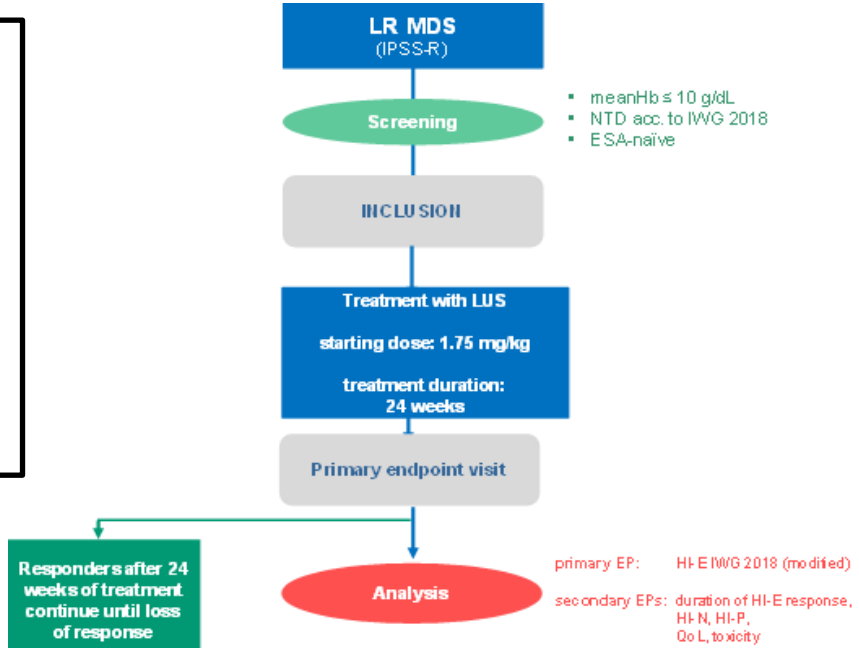
LR/HR	Acronym	Title	IMP	Countries	Sample size	Status
LR	**LENNON**	*A phase II, open-label, single arm study to evaluate the efficacy of luspatercept in erythropoiesis-stimulating agent naive lower-risk MDS patients with or without ring sideroblasts who do not require RBC transfusions*	Luspatercept		N=30 (single-arm)	completed prematurely **N=18/30**

Essential inclusion criteria

- Diagnosis of MDS according to WHO classification
- Very low, low, or intermediate-risk disease with up to 3.5 points according to the Revised International Prognostic Scoring System (IPSS-R)
- Non-transfusion-dependent (NTD) according to IWG 2018
- Symptomatic anemia: mean baseline Hb < 10 g/dL

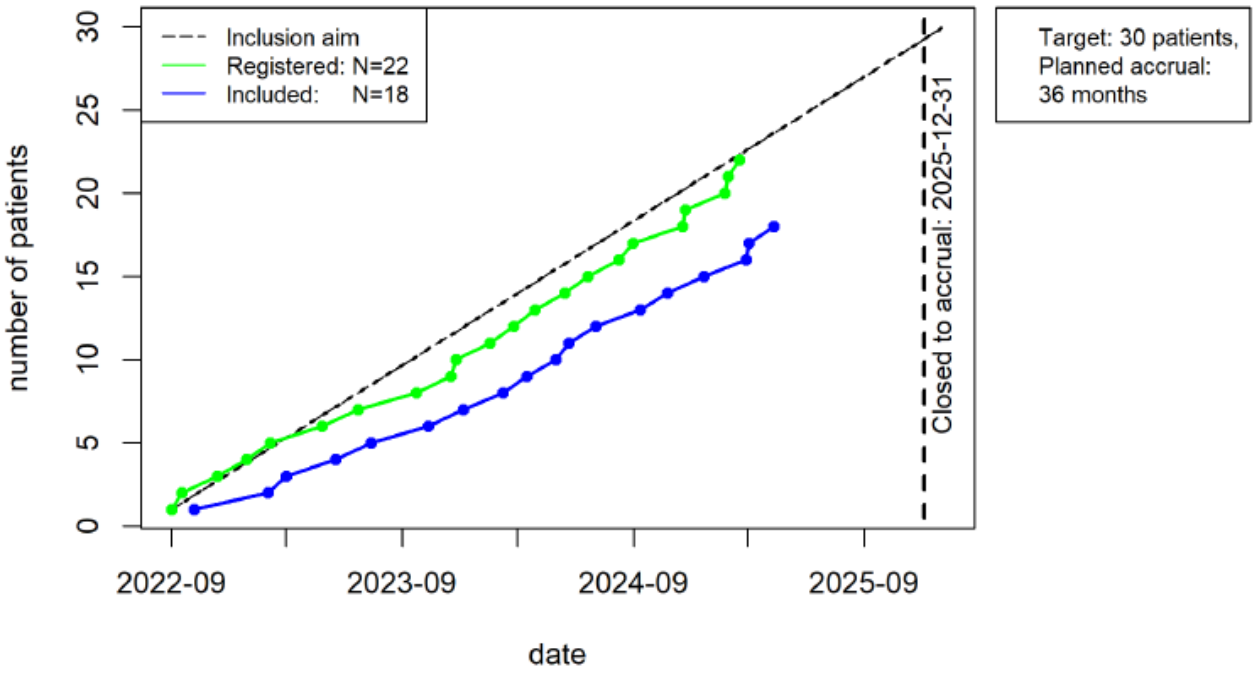
Essential exclusion criteria

- Secondary MDS
- Known clinically significant anemia due to iron, vitamin B12, or folate deficiency
- autoimmune disorders, hereditary hemolytic anemia, or gastrointestinal bleeding
- Prior allogeneic or autologous stem cell transplantation
- ECOG > 2
- Previous ESA treatment



ASH 2026

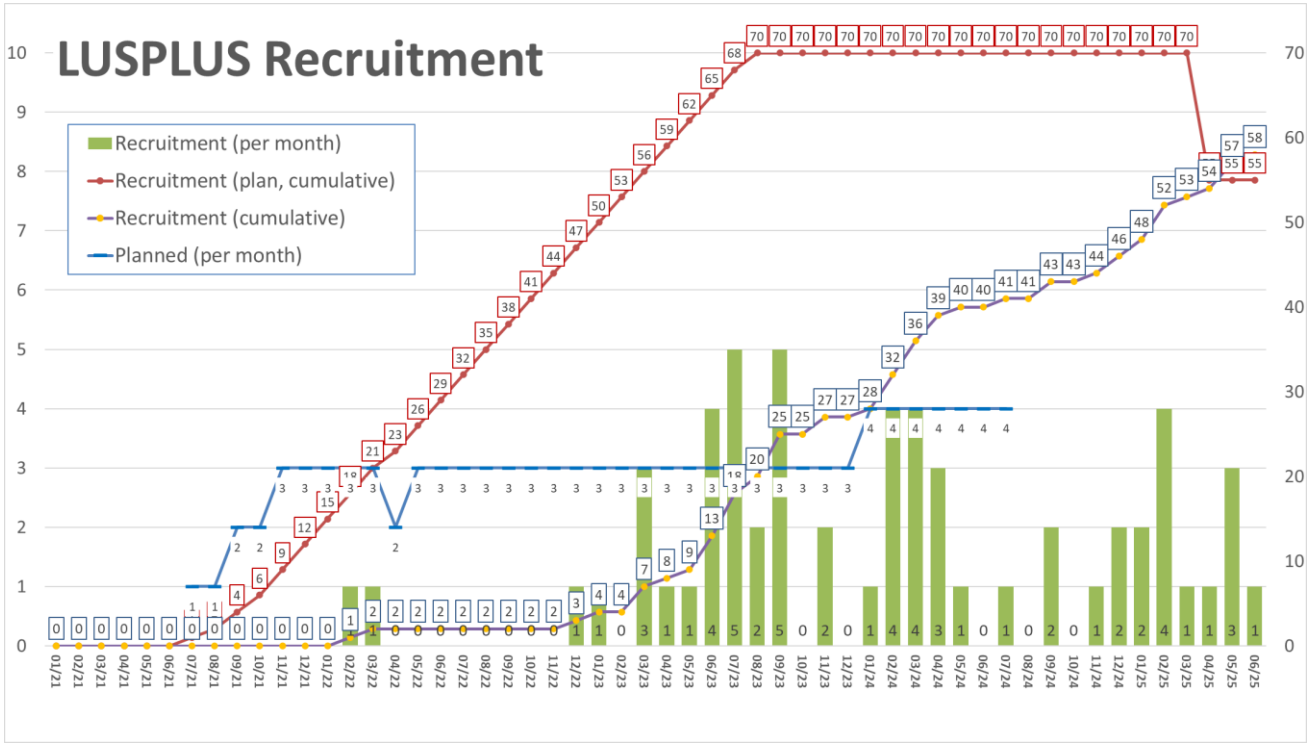
LENNON: Current status



- Recruitment ended on December 31, 2025 with 18 of 30 planned patients enrolled
- At last follow-up, 10/18 patients were still on treatment
- Presentation accepted for ASH 2026

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LUSPLUS: Current Status



ASH 2024

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LUSPLUS: Current Status

Milestone Overview



- In total, 58 patients have been recruited
- Recruitment took place in Germany (2 sites), Austria (1 site), Switzerland (3 sites) and Spain (7 sites)
- As of now (June 2026), one patient is still on treatment

CA Approval – Competent Authority Approval
FPI – First Patient In
LPI – Last Patient in
LPO – Last Patient Out
SAR – Statistical Analysis Report
CSR – Clinical Study Report

Actual finish date
Planned finish date

PALOMA Trial Design

NCT04061239

PALOMA (**P**rimary comp**A**rison of **L**ipos**O**mal anthracycline based treatment versus conventional care strategies prior to allogeneic stem cell transplantation in patients with higher risk **M**DS or oligoblastic **A**ML)

A phase 2, multicenter, randomized open-label, study of CPX-351 versus CCR as first-line treatment before alloHCT[1]

Key eligibility criteria

- 18–75 years of age
- Untreated HR-MDS including oligoblastic non-proliferative (WBC <13 Gpt/L) AML (≤29% blasts **per central morphology**) per WHO 2016 criteria[2]
- Bone marrow blasts ≥5% (per central morphology)
- ECOG PS of 0 or 1
- AlloHCT intended within next 6 months
- ALT or AST <3 x ULN
- LVEF ≥50%

N=150

R 1:1

Up to two induction courses

CPX-351 (daunorubicin 44 mg/m² IV and cytarabine 100 mg/m² IV in liposomes)
Induction 1: D1, D3, and D5
Induction 2: D1 and D3

Consolidation

CPX-351 (daunorubicin 29 mg/m² IV and cytarabine 65 mg/m² IV in liposomes) on D1 and D3
Patients <60 years up to 3 cycles
Patients ≥60 years up to 2 cycles

7+3 cytarabine/daunorubicin chemotherapy regimen (IV) OR
azacitidine 75 mg/m² for 7 days, **minimum of 2 and up to 6 cycles** (SC)

Key exclusion criteria

- History of MPN or combined MDS/MPN
- **WHO-2016 defined AML entities: t(15;17), *PML-RARA*; t(8;21), *RUNX1-RUNX1T1*; inv(16)/t(16;16), *CBFB-MYH11*; biallelic *CEBPA* mutation; or mut *FLT3* or *NPM1***
- Active CNS leukemia
- currently active secondary malignancy (except NMSC)

Endpoints

- **Primary endpoint:** Data cutoff for final analysis Sept 30, 2025
  - EFS defined as time from randomization to **primary refractory disease, relapse, death from any cause, or treatment change**
- **Secondary endpoints:** Data cutoff for pre-planned interim analysis Sept 30, 2025; final Sept 2026
  - OS (key secondary)
  - Best and overall response rate, proportion of patients proceeding to alloHCT, MRD, QoL, and safety

EHA 2026

D·MDS Deutsche MDS-Studiengruppe

PALOMA baseline characteristics

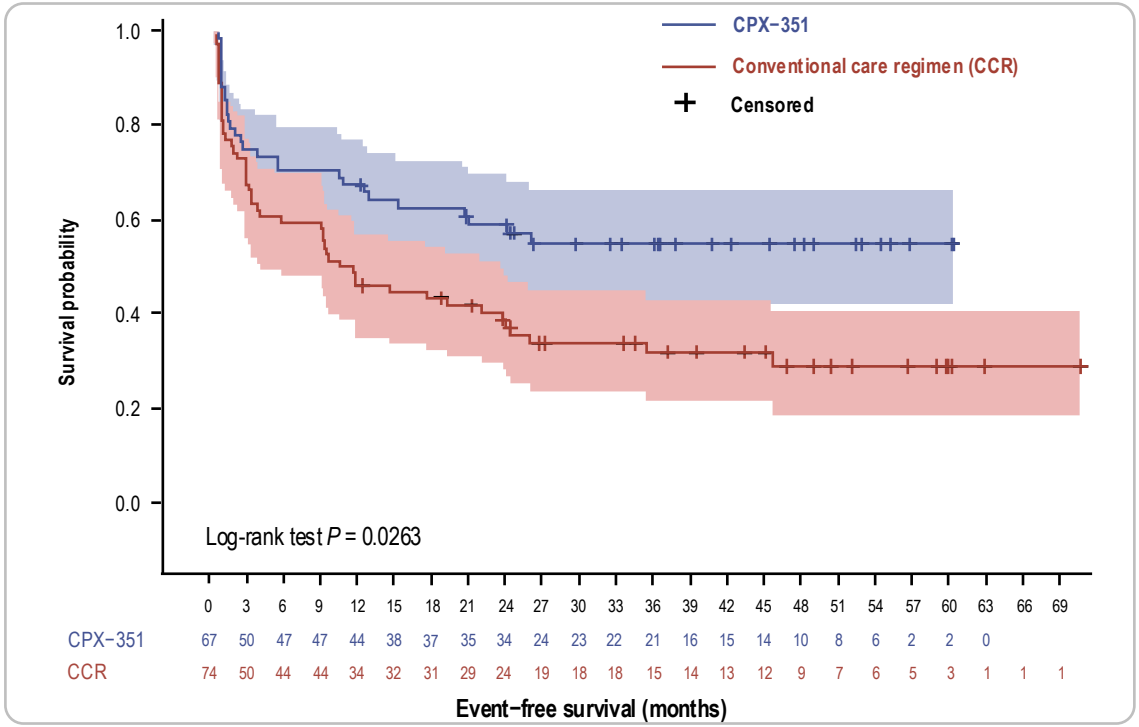
Baseline characteristics were generally well balanced between the treatment arms.
The CCR arm included 52 patients (69.3%) treated with 7+3 cytarabine/daunorubicin and 23 patients (30.7%) treated with azacitidine.

	Total (n = 150)	CPX-351 (n = 75)	CCR (n = 75)
Sex, n (%)			
Male	92 (61.3)	41 (54.7)	51 (68.0)
Female	58 (38.7)	34 (45.3)	24 (32.0)
Age, median (range) years	63 (31–75)	64 (41–75)	63 (31–73)
Age ≥60 years, n (%)	97 (64.7)	48 (64.0)	49 (65.3)
WHO classification, n (%)			
AML/myeloid sarcoma	31 (20.7)	16 (21.3)	15 (20.0)
MDS	117 (78.0)	59 (78.7)	58 (77.3)
Missing	2 (1.3)	0	2 (2.7)
BM blast counts, n (%)			
< 20%	116 (77.3)	58 (77.3)	58 (77.3)
20%–30%	34 (22.7)	17 (22.7)	17 (22.7)
ECOG PS, n (%)			
0	78 (52.0)	40 (53.3)	38 (50.7)
1	72 (48.0)	35 (46.7)	37 (49.3)

D·MDS
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PALOMA primary endpoint

Median EFS was significantly longer with CPX-351 compared to CCR



Median EFS

CPX-351: NR
(95% CI 15.3 months – NR)

CCR: 11.1 months
(95% CI 4.1 – 23.8 months)

Efficacy endpoints were evaluated in the full analysis set (FAS)
= all randomized patients excluding patients without informative data for EFS assessment, n=141

D·MDS
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Active interventional Studies (according to AMG/CTR)

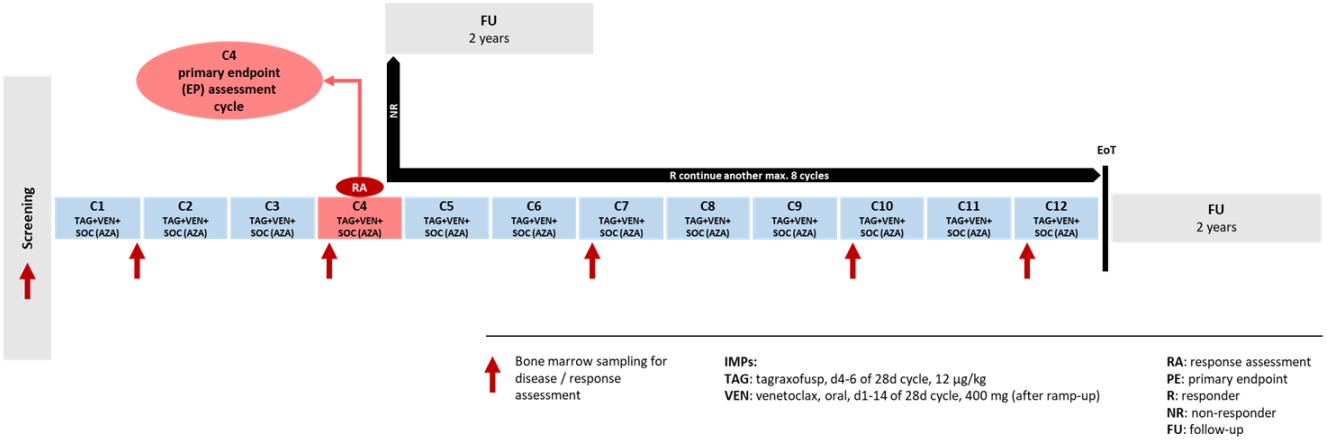
LR/HR	Acronym	Title	IMP	Countries	Sample size	Status
CMML	**PATROL**	*A Phase II study of Azacitidine (AZA) combined with Venetoclax (VEN) and Tagraxofusp (TAG) in patients with higher higher-risk chronic myelomonocytic leukemia (CMML)*	**Tagraxofusp** (in comb. with AZA and VEN)		N=30 (single-arm)	Recruiting **N=10**

Essential inclusion criteria

- CMML diagnosis according to WHO 2022 criteria
- CPSS/CPSSmol risk: intermediate-2 or high-risk (HR) CMML
- Eligible for AZA as SOC
- ECOG: 0–2

Essential exclusion criteria

- CMML with t(5;12) or PDGFRB rearrangement, treatable with imatinib
- Blasts in bone marrow or peripheral blood ≥ 20%
- Patients with known CNS involvement



Primary endpoint: efficacy, safety, tolerability

Secondary endpoints: OS, TLT, duration of response, HI-E, TI, QoL

PI: Katja Sockel, Dresden

Active interventional Studies (according to AMG/CTR)

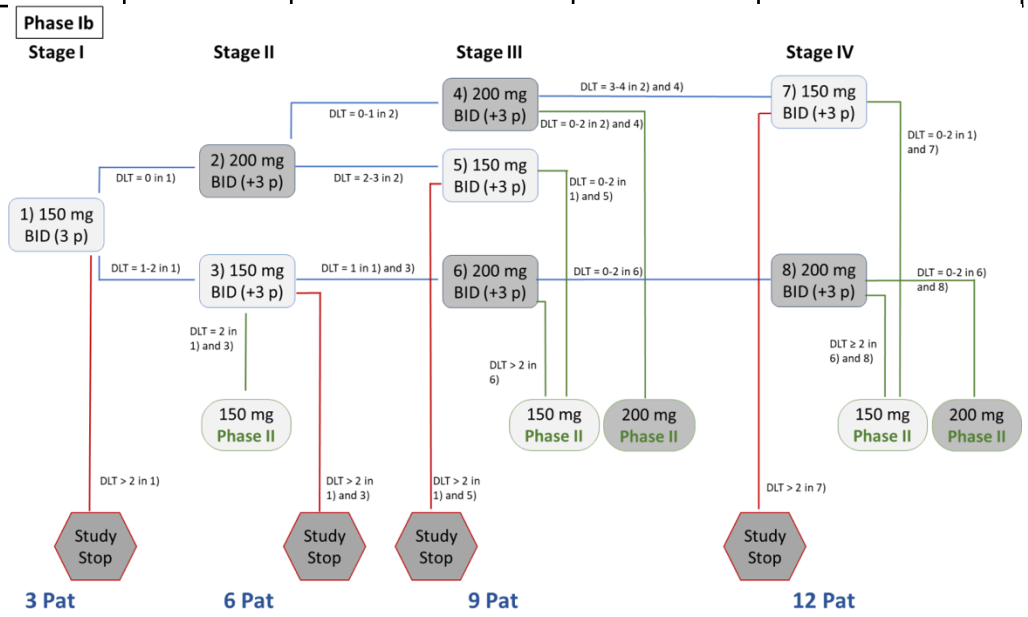
LR/HR	Acronym	Title	IMP	Countries	Sample size	Status
HR-MDS, CMML	**AZALOX**	*A Phase Ib/II multicenter open-label study evaluating the safety and efficacy of escalating doses of PXS-5505 in combination with 5-Azacitidine for pan LOX/LOXL inhibition in patients diagnosed with Myelodysplastic Neoplasms (MDS) or Chronic Myelomonocytic Leukemia (CMML)*	**PXS 5505** (in comb. with AZA)		N=42 (single-arm)	Recruiting **N=5**

Essential inclusion criteria

- For MDS: IPSS-R: high or very high with Hb < 8 g/dl
- For CMML: CPSS classification: intermediate-2 or high
- ECOG ≤ 2
- Transfusion dependency within the last 16 weeks

Essential exclusion criteria

- Previous combination treatment with AZA and VEN
- Allogeneic HSCT or solid organ transplantation
- Use of cytotoxic chemotherapeutic agents, corticosteroids (prednisone ≤ 10 mg/day or corticosteroid equivalent permitted), or immunomodulatory agents (e.g., thalidomide, lenalidomide) within 28 days prior to study entry



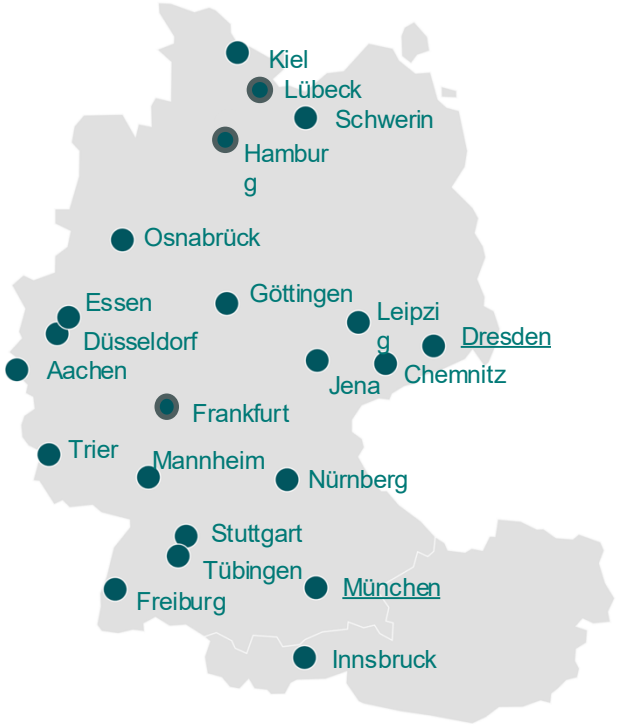
PI: Susanne Saußele, Mannheim

supported by Deutsche Krebshilfe HELFEN. FORSCHEN. INFORMIEREN.

Active non-interventional Studies

LR/HR	Acronym	Title	IMP	Countries	Sample size	Status
LR	PRO-RED	*Longitudinal, App-based Assessment of Varying Red Blood Cell Transfusion Strategies and their Association with Patient-Reported and Clinical Outcomes in Lower-Risk MDS Patients*	**n.a.**		N=60 (single-arm)	Recruiting **N=47/60**
LR/HR	MDS-Registry	*Prospective and retrospective data collection on diagnosis, treatment and course of disease of myelodysplastic syndromes in adults*	**n.a.**		Appr. N=1200 newly documented patients + appr. N=8400 already documented patients	Recruiting **N>7000/9600**

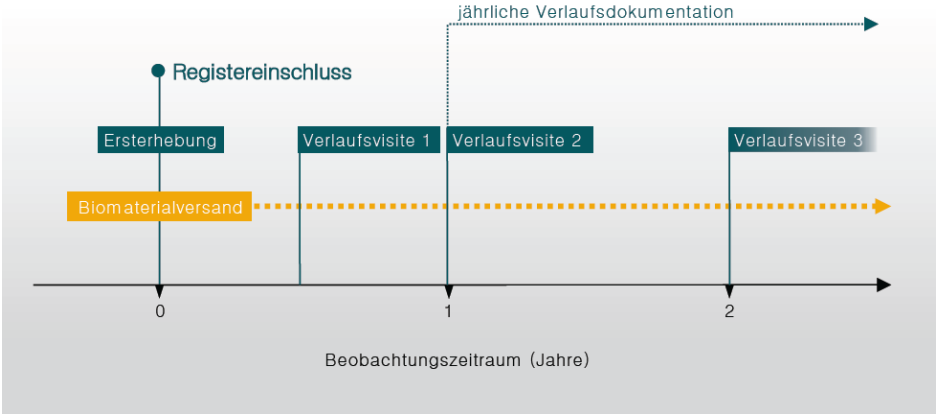
German VEXAS Registry



Registry initiation 08/2024

N=150

VEXAS REGISTER







Inclusion criteria:

- UBA1 mutation
- ≥ 18 years
- Informed consent (Registry +Biobanking)

Chairs: Katja Sockel, Dresden & Katharina Götze, Munich

Active non-interventional Studies

VEXAS REGISTER

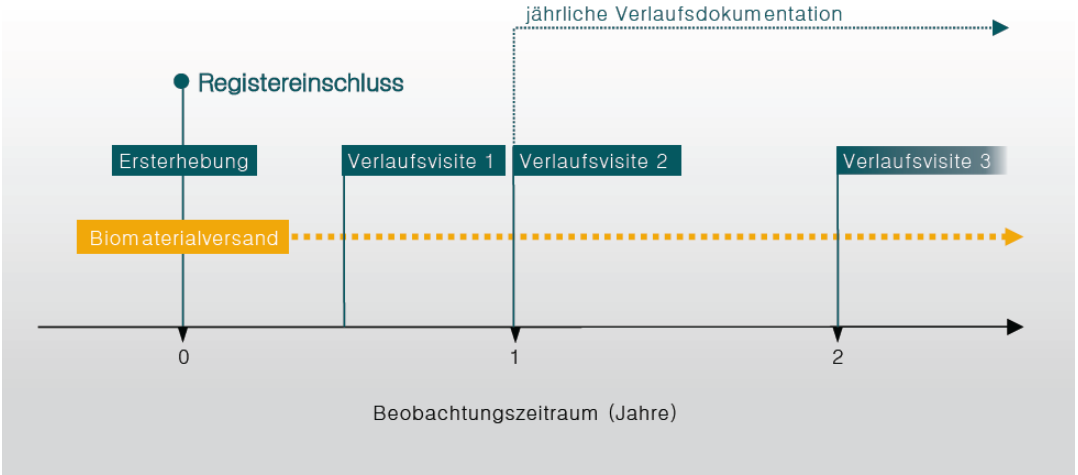
LR/HR	Acronym	Title	IMP	Countries	Sample size	Status
	VEXAS-Registry	*Multicenter national VEXAS registry with accompanying biomaterial collection*	**n.a.**		N=45 per year	Recruiting **N=150**

Essential inclusion criteria

- Confirmed diagnosis of VEXAS syndrome (molecular genetic evidence of a UBA1 mutation)

Essential exclusion criteria

- Patients with comorbidities and a non-curative intended therapy



→ https://vexas.net/

ASH 2025

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Active non-interventional Studies

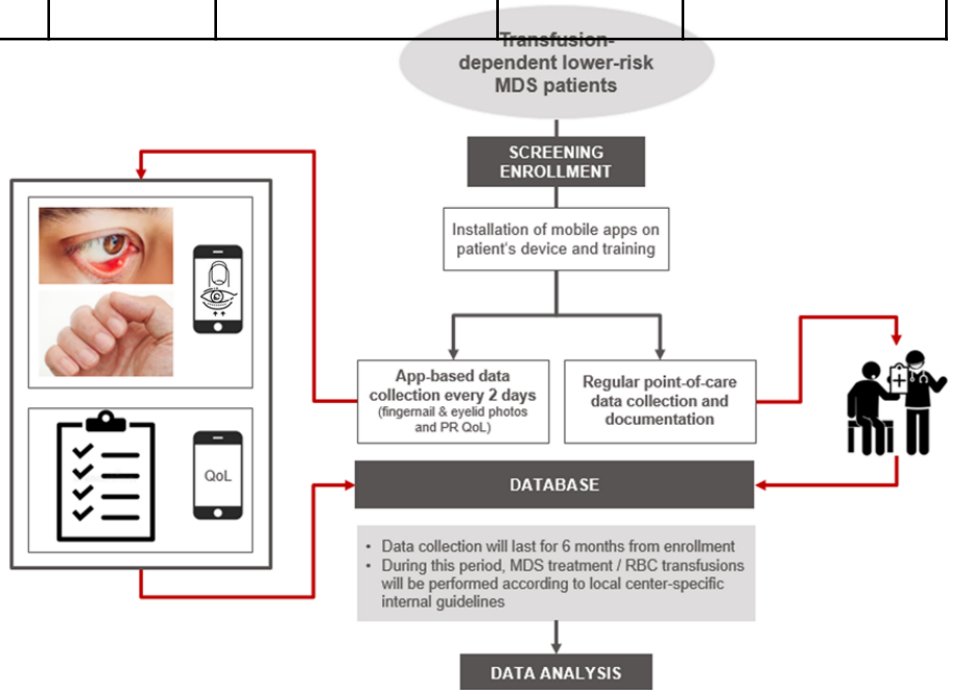
LR/HR	Acronym	Title	IMP	Countries	Sample size	Status
LR	PRO-RED	*Longitudinal, App-based Assessment of Varying Red Blood Cell Transfusion Strategies and their Association with Patient-Reported and Clinical Outcomes in Lower-Risk MDS Patients*	n.a.		N=60 (single-arm)	Recruiting **N=47/60**

Essential inclusion criteria

- Confirmed diagnosis of lower-risk MDS (IPSS-R very low, low, intermediate up to 3.5 points) or MDS/MPN overlap including MDS/MPN-RS-T, MDS/MPNu, aCML or non-proliferative CMML according to WHO criteria as determined by microscopic analyses of the bone marrow and peripheral complete blood count (CBC)
- Symptomatic transfusion dependent anemia defined as having received ≥3 units of RBCs within the last 16 weeks prior to screening

Essential exclusion criteria

- Patients who are unable to use the PRO-RED app or questionnaires provided for the study



Active non-interventional Studies

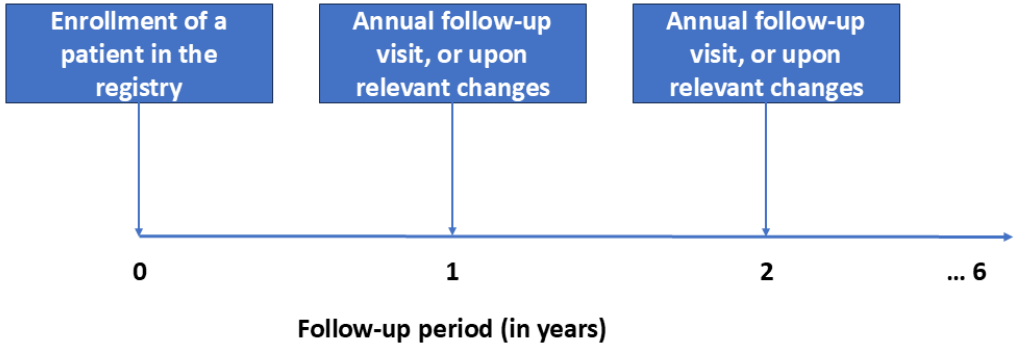
LR/HR	Acronym	Title	IMP	Countries	Sample size	Status
LR/HR	MDS-Registry	*Prospective and retrospective data collection on diagnosis, treatment and course of disease of myelodysplastic syndromes in adults*	**n.a.**		Appr. N=1200 newly documented patients + appr. N=8400 already documented patients	Recruiting **N>7000/9600**

Essential inclusion criteria

- MDS, MDS-MPD-Disease including AML with signs of dysplasia (AML-MRC) according to WHO-classification, t-MDS, t-AML with blasts <30%, MDS-associated diseases: ICUS, CHIP & CHOP
- Availability of follow-up

Essential exclusion criteria

- Other myeloid neoplasms



→ https://www.mds-register.de/

PI: Ulrich Germing, Düsseldorf

Finished Trials

LR/HR	Acronym	Title	IMP	Countries	Sample size	Status
HR	SAMBA	*Single agent JNJ-56022473 in MDS and AML patients failing hypomethylating agent based therapy*	**Talacotuzumab**		N=43	Leukemia
LR	EUROPE	*Prospective validation of a predictive model of response to Romiplostim in patients with IPSS low or intermediate-1 risk myelodysplastic syndrome (MDS) and thrombocytopenia*	**Romiplostim**		N=75	Leukemia
CMML	DACOTA	*A randomized phase III study of Decitabine (DAC) with or without hydroxyurea (HY) versus HY in patients with advanced proliferative chronic myelomonocytic leukemia (CMML)*	**Decitabine**		N=168	JCO
HR	BERGAMO	*A phase II study evaluating the efficacy and safety of Bemcentinib in patients with myelodysplastic syndromes failing standard of care therapy*	**Bemcentinib**		N=43	Leukemia
LR	SINTRA-REV	*Multicenter, randomized, double-bling, phase III study of REVLIMID (Lenalidomide) versus placebo in patients witn low risk myelodysplastic syndrome (low and internediate-1 IPSS) with alteration in 5q- and anemia without the need of transfusion*	**Lenalidomid**		N=61	Lancet Haematol
LR & HR	IDEAL	*A single-arm phase II multicenter study of IDH2 (AG-221) inhibitor in patients with IDH2 mutated myelodysplastic syndrome*	**Enasidenib**		N=68 (3 cohorts)	
LR	LUCAS	*A Phase II, Open-Label, Multicenter Study of Orally Administered CA-4948 for the Treatment of Anemia in Patients With Very Low, Low or Intermediate Risk Myelodysplastic Syndromes (MDS)*	**Emavusertib (CA-4948)**		N=38/84 (2 cohorts)	Comm Med 2026
LR	CANFIRE	*A Phase II, Single-Arm, Open-Label Study to Assess the Efficacy and Safety of Canakinumab for the Treatment of Anemia in Patients With IPSS-R Very Low, Low, or Intermediate Risk Myelodysplastic Syndromes or MDS/MPN*	**Canakinumab**		N=11/41	
LR	IMerge	*A Study to Evaluate Imetelstat (GRN163L) in Transfusion-Dependent Subjects With IPSS Low or Intermediate-1 Risk Myelodysplastic Syndrome (MDS) That is Relapsed/Refractory to Erythropoiesis-Stimulating Agent (ESA) Treatment*	**Imetelstat**	among others: 	N=178	Lancet

Completed Trials

LR/HR	Acronym	Title	IMP	Countries	Sample size	Status
LR	REMARK	*A phase II, open-label, multicenter study of orally administered RVU120 for the treatment of anemia in patients with lower-risk myelodysplastic neoplasms (MDS)*	**RVU120**		N=41 (single-arm)	*ASH 2025*
HR	IMPRESS	*A phase II study evaluating the efficacy and safety of imetelstat in patients with HR myelodysplastic syndromes or AML failing HMA-based therapy*	**Imetelstat**		N=46 (single-arm)	*ASH 2025*
LR	LUCAS	*A Phase II, Open-Label, Multicenter Study of Orally Administered CA-4948 for the Treatment of Anemia in Patients With Very Low, Low or Intermediate Risk Myelodysplastic Syndromes (MDS)*	**Emavusertib (CA-4948)**		N=38/84 (2 cohorts)	published Platzbecker AS et al, Commun Med 2026
LR	CANFIRE	*A Phase II, Single-Arm, Open-Label Study to Assess the Efficacy and Safety of Canakinumab for the Treatment of Anemia in Patients With IPSS-R Very Low, Low, or Intermediate Risk Myelodysplastic Syndromes or MDS/MPN*	**Canakinumab**		N=11/41	
LR	LusPlus	*A phase IIIb, open-label, single arm study to evaluate the efficacy and safety of luspatercept in patients with lower-risk MDS and ring-sideroblastic phenotype (MDS-RS)*	**Luspatercept**		N=55	ASH 2024 Final analysis pending
HR	RELAZA2	*Azacitidine to treat measurable residual disease in patients with MDS/AML*	**AZA in MRD+ relapse after allo**		N=119	published Platzbecker AS et al, Blood 2026
LR	LENNON	*A phase II, open-label, single arm study to evaluate the efficacy of luspatercept in erythropoiesis-stimulating agent naive lower-risk MDS patients with or without ring sideroblasts who do not require RBC transfusions*	**Luspatercept**		N=18	ASH 2026 Final analysis pending


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