ERN | EuroBloodNet

13th June 2025 - EHA Congress

EuroBloodNet sponsored clinical trials

Speaker: Pierre Fenaux

Université Paris Cité

UNIVERSITÉ PARIS 13

Groupe Francophone des Myélodysplasies

European Reference Network | Hematological Diseases (ERN EuroBloodNet)

Funded by the European Union

EuroBloodNet sponsored clinical trials

- Academic trials differing from industry sponsored trials and large institution sponsored trials
- Often in a field not largely explored by pharma companies
- Close cooperation with a pharma company which provides
  - The study drug
  - research funding
- International (at least 2 EU countries)
- Centralized submission (CTIS)

EuroBloodNet sponsored clinical trials

- EuroBloodNet performs all trial sponsor requirements
  - Submission
  - Drug shipping
  - Data monitoring
  - Pharmacovigilance
  - Publication…
- Currently 2 trials (both in inherited anemias)
  - Satisfy (E Van Beers, A Glenthoj)
  - Luspara (T Leblanc)

Luspara trial :Luspatercept in rare inherited anemias

- Congenital
  - *sideroblastic anemia*
  - *Dyserythropoietic anemia*
  - *Blackfan Diamond anemia (not transfusion dependent)*
- Luspatercept at increasing dose
- 3 countries: F, I,E

ESH EUROPEAN SCHOOL OF HAEMATOLOGY

MARK YOUR AGENDA

10th Translational Research Conference
Myelodysplastic Syndromes,
Overlap MDS/MPN Disorders and Clonal Hematopoiesis

Malahide (Dublin), Ireland
October 16-18, 2026
#ESHMDS2026

Chairs: Pierre Fenaux, Katharina Götze, Mikkael Sekeres

DEADLINE FOR ABSTRACTS: JULY 6th, 2026

To register and for further information: www.esh.org - info@esh.org

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Investigator initiated trials

sponsored by ERN-EuroBloodNet

Speaker: Dr. Eduard J. van Beers

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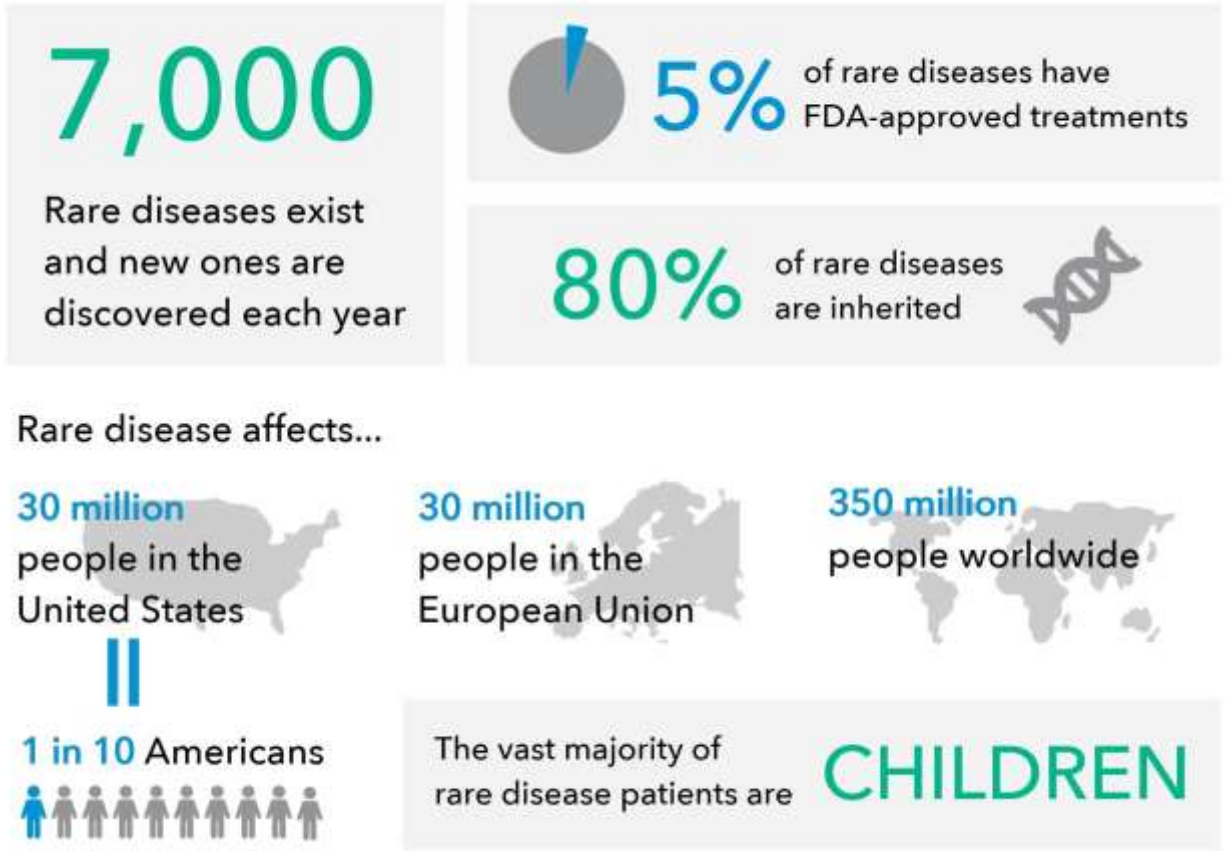
Disclosure of Conflict of Interest

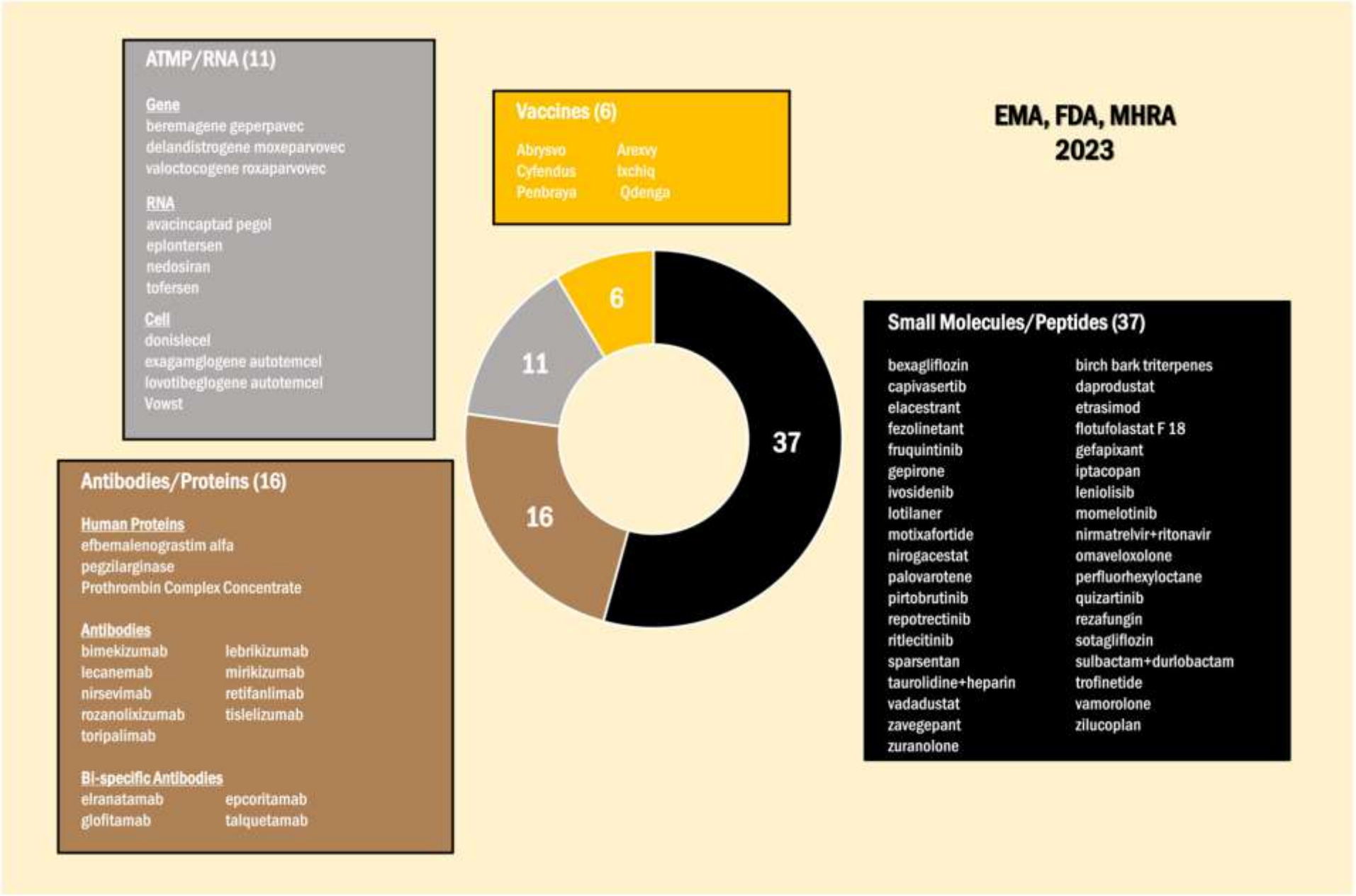
Research funding: Vertex, Agios, Horizon Europe,
Dutch National Science Council

Co-Ordinator TFA Research and Trials Eurobloodnet
Associated Professor Rare Anemia University
Medical Center Utrecht, the Netherlands
Chair Sickle Cell Research Consortium and Registry
(SCORE), the Netherlands
Chair HOVON benign hematology working group

Patients for whom drugs need to be developed



From: www.chisite.org/blog/equitable-access-to-rare-disease-therapies-workshop
Center for Healthcare innovation



Novel drugs approved by the EMA, the FDA, and the MHRA in 2023: A year in review
Br J Pharm. Volume181, Issue11 June 2024 P1553-1575 https://doi.org/10.1111/bph.16337

U-TRIAL
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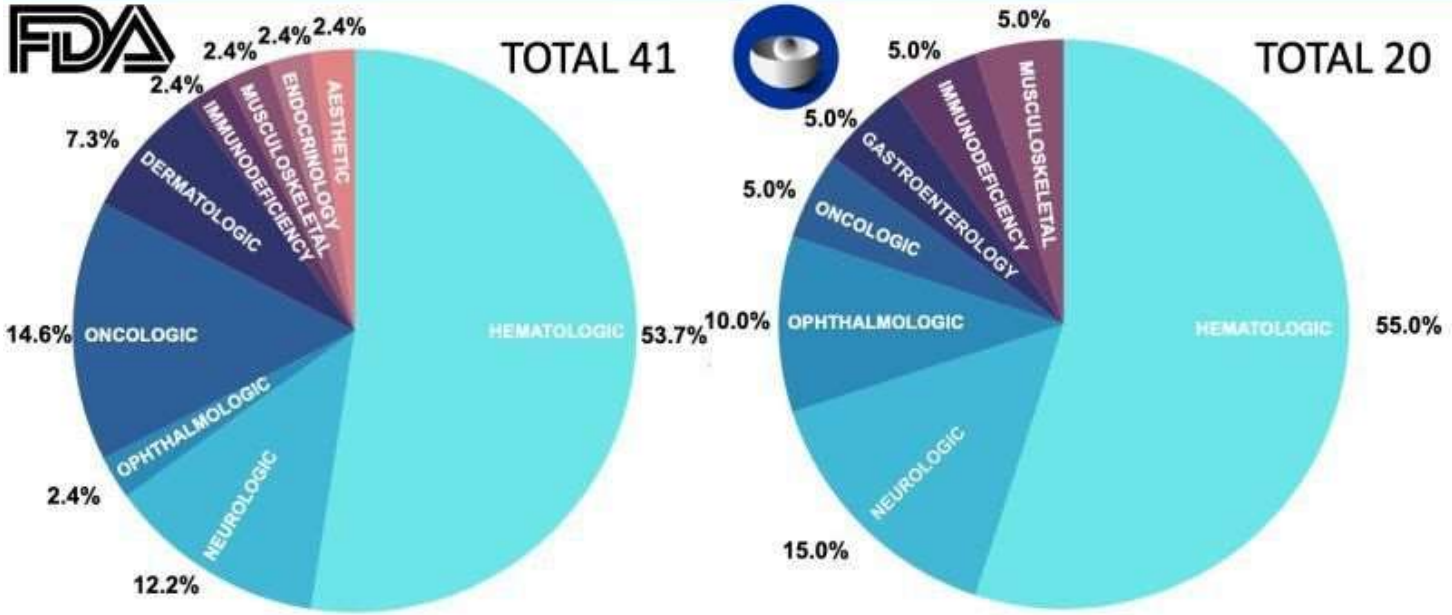
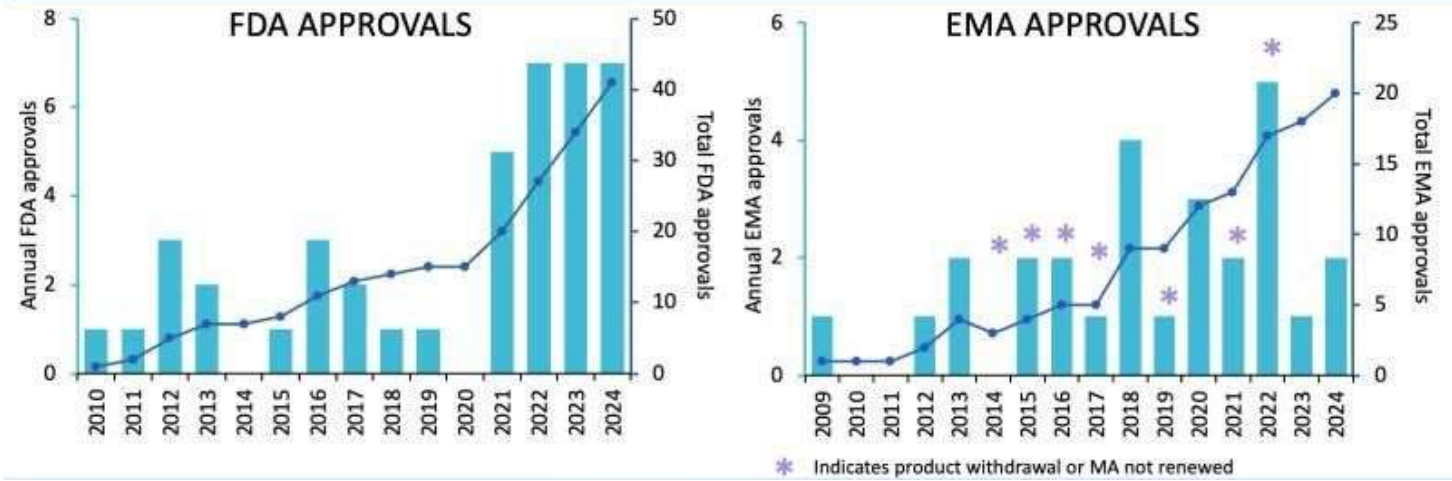
CELL AND GENE THERAPIES IN THE US AND EUROPE

joanna-sadowska-phd @jmsadowska

THERAPEUTIC AREA

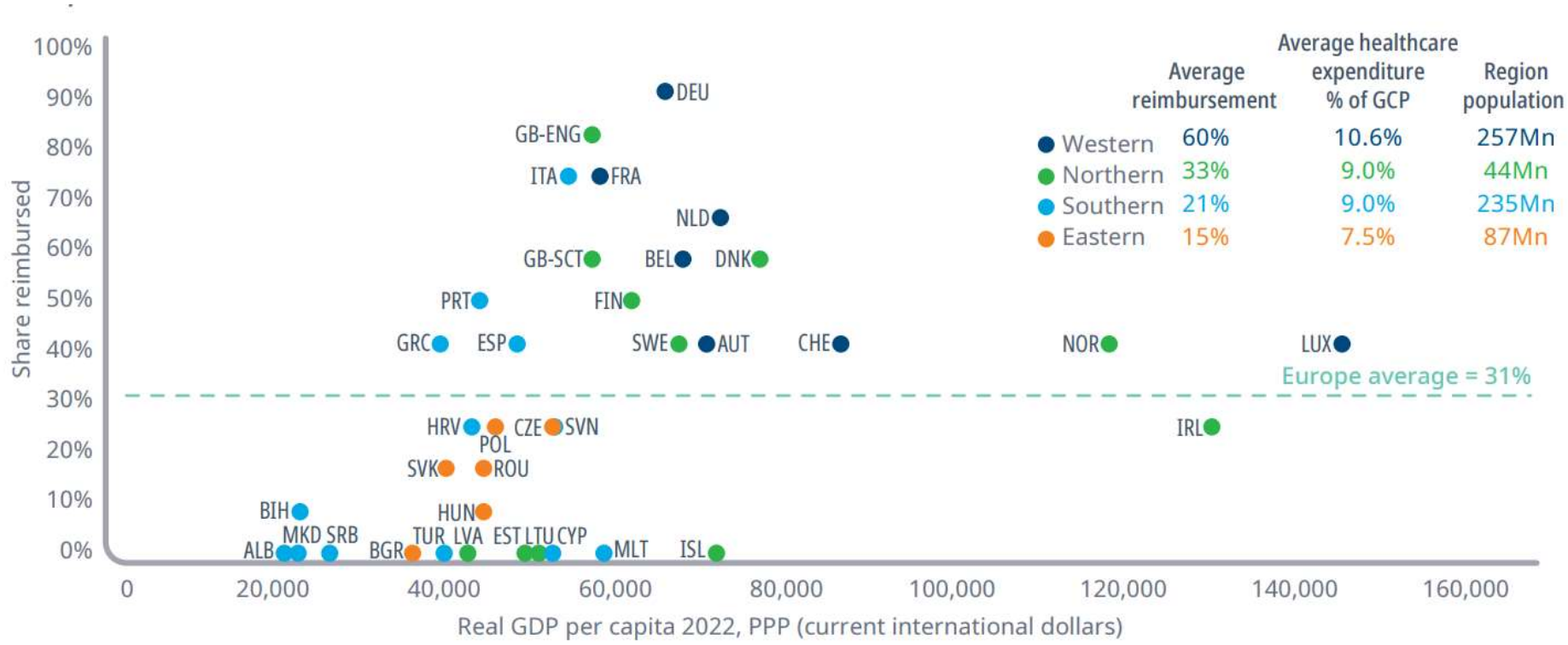


ANNUAL APPROVALS



Status of re-imbursement of gene and cell therapy in Europe

(Apr 2023)



Source: European Medicines Agency, IQVIA EFPIA Patients W.A.I.T. Indicator 2022 Survey, Apr 2023; International Monetary Fund, Oct 2023; IQVIA Institute, Jan 2024.

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Gene en cell therapy pipeline

Global Status	Q3 2023	Q4 2023	Q1 2024	Q2 2024	Q3 2024
Preclinical	1,522	1,528	1,471	1,436	1,393
Phase I	256	270	301	314	318
Phase II	267	274	282	279	289
Phase III	30	33	35	34	35
Pre-registration	7	6	4	5	6
Total	**2,082**	**2,111**	**2,093**	**2,068**	**2,041**

Gene, Cell, + RNA Therapy Landscape Report, American Society of Gene & Cell Therapy and Citeline, 2024 Q3 Original report source at https://asgct.org/publications/landscape-report

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Drug repurposing promises greater chance of clinical success

1. **Clinical benefit** has already been demonstrated
   - *in at least some patient populations*
   - *in at least some dosage forms and scheduling*

2. **Safety** has already been demonstrated
   - *in at least some patient populations*
   - *in at least some dosage forms and scheduling*

3. **Drug product** can be practically manufactured
   - *in at least some routes of administration*
   - *in at least some dosage forms*

Drug repurposing

Traditional pathway

Drug:
- withdrawn
- in development
- on market:
  - in patent
  - out of patent

Label
- extension (non/-pharma driven)
- stage of disease
- similar disease
- paediatric usage
- different disease same mode of action
- different disease different mode of action

- **Faster** Approval and Market Entry (Label extension is the best!)
- **Lower Development Costs**
- **Higher Success Rate**
- **Addresses Unmet Medical Needs Faster**
- **Lower Risk of Side Effects and Safer for Patients**
- **Environmental and Ethical Advantages**

Classical: thalidomide, senicapoc
Alternative: mitapivat, oncological product became benign hematology product

ASK FOR HELP:

- **Eurobloodnet** pierre.fenaux@aphp.fr **trial conduct /sponsorship**
- **Eurobloodnet** e.j.vanbeers-3@umcutrecht.nl **general tips directions in trialing**
- **EATRIS/ REMEDI4ALL** https://remedi4all.org/ **regulatory advice**

Experience: Satisfy trial

Registration	• NCT05935202
Setup	• Investigator initiated, prospective, multicenter, single-arm phase 2 trial.
Locations	• Denmark and The Netherlands • Sibling study in Toronto, Canada
Sponsor	• Non-profit EuroBloodNet *Association*
Funding	• Agios Pharmaceuticals

Key Inclusion Criteria

Membranopathy or CDAII

Genetically confirmed
ACMG class 3, 4, or 5

Age ≥18 years

Hb concentration:
<13.0 g/dL for males
<11.0 g/dL for females

Adequate organ function

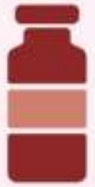
Key Exclusion Criteria

PKLR

Pyruvate kinase deficiency
diagnosed with decreased PK activity or two pathogenic PKLR alleles

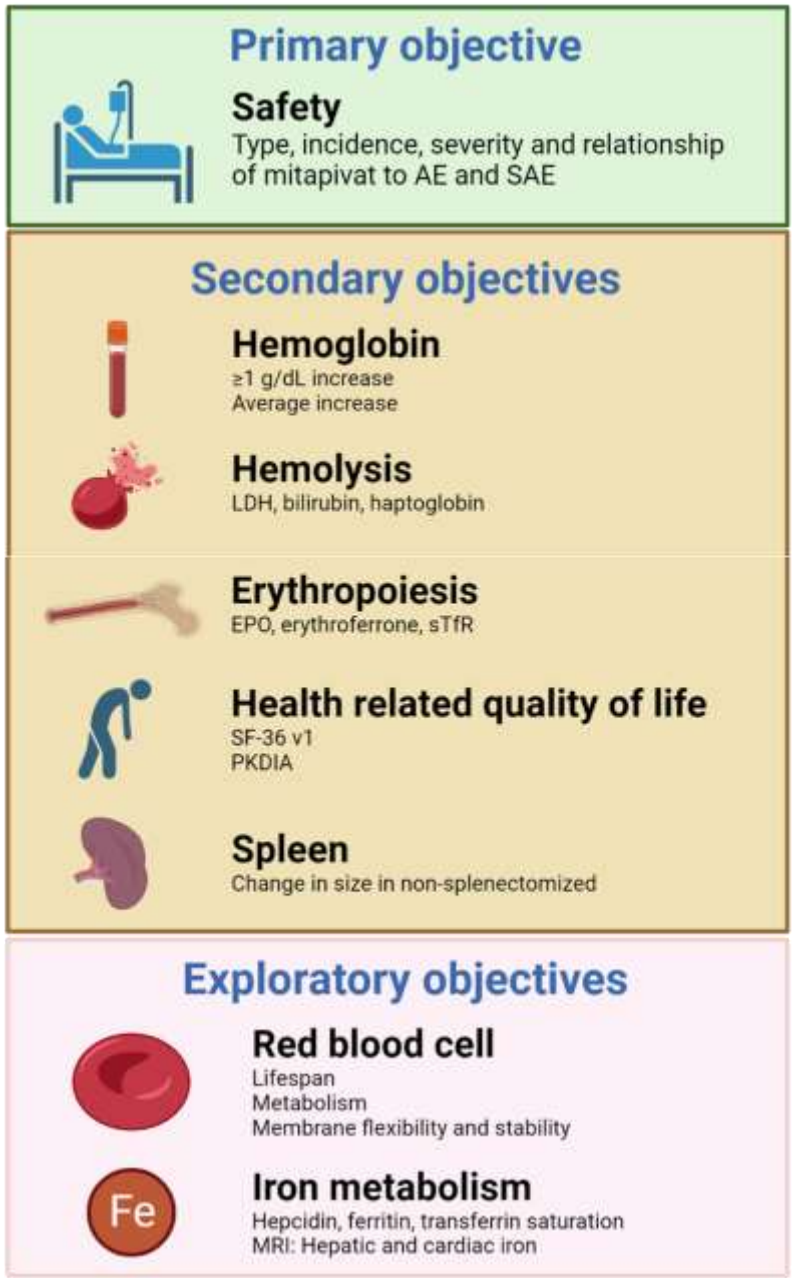
Blood transfusion within last 3 months or >5 units the last year

Significant medical comorbidity



Receiving hematopoietic stimulating agents

Intervention



Experience

Eurobloodnet as sponsor:

Excellent solution to avoid institutional restrictions on non-marketed drug research, data transfer, contracting etc

Excellent support CTIS submission and clinical trial oversight

Drug delivery operations and QC

Academic Pricing

You are in control!

Conclusions

1. Investigator initiated trials are ideal for drug re-purposing/label extension in hematology
2. Eurobloodnet can help
3. Satisfy is an example of such a study
4. Collaborate and …. have fun!

THANK YOU!

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