



# ***EDITSCD-webinar program on gene editing***

**Your full name: Annarita Miccio**

**Affiliation: Institut Imagine**

**Contact: [annarita.miccio@institutimagine.org](mailto:annarita.miccio@institutimagine.org)**

**Your full name: Mariangela Pellegrini**

**Affiliation: ERN-Eurobloodnet**

**Contact: [mariangela.pellegrini@aphp.fr](mailto:mariangela.pellegrini@aphp.fr)**



# Collaboration EDITSCD & ERN-EuroBloodNet

- Dissemination of results to patients and patient associations
- Brainstorm on EDITSCD communication tools
- Organization of technical workshops for patient advocate from Europe and outside of Europe (US, Canada, Africa, Australia)

### CONSORTIUM

Coordinated by Imagine Institut, the EDITSCD Consortium is composed of 8 international partner institutes.

**IMAGINE INSTITUT DES MALADIES GÉNÉTIQUES**  
Annarita MICCIO

**OSPEDALE SAN RAFFAELE SRL**  
Giuliana FERRARI

**UNIVERSITÄTSKLINIKUM FREIBURG**  
Claudio MUSSOLINO  
Toni CATHOMEN

**BAR ILAN UNIVERSITY**  
Ayal HENDEL

**INSTITUT NATIONAL DE LA SANTÉ ET DE LA RECHERCHE MÉDICALE**  
Marco AMENDOLA

**EIDGENÖSSISCHE TECHNISCHE HOCHSCHULE ZÜRICH**  
Jacob CORN

**ASTRAZENECA AB**  
Marcello MARESCA

**EATRI ERIC**  
David MORROW

The Consortium is supported by an External Scientific Advisor and the patient-centered network ERN-EuroBloodNet.

### THE PROJECT

#### WORKPLAN

In this project, we have assembled a multidisciplinary team to understand the molecular and cellular mechanisms underlying SCD HSPC autonomous and non-cell-autonomous dysfunctions and to evaluate the impact of established and novel genome editing approaches on SCD HSPC properties and genome integrity.

**WP1** – Evaluation of the impact of different SCD genome editing approaches on SCD human HSPC cell-autonomous properties  
Annarita MICCIO

**WP2** – Evaluation of the impact of different SCD genome editing approaches on SCD human HSPC non-cell-autonomous properties  
Giuliana FERRARI

**WP3** – Establishment of safe and effective genome editing approaches for SCD  
Claudio MUSSOLINO

**WP4** – Evaluation of the outcomes and impact of different SCD genome editing approaches on genome integrity  
Ayal HENDEL

**WP5** – Comparison of the most effective editing strategies in SCD human HSPC  
Annarita MICCIO

**WP6** – Innovation management  
David MORROW

**WP7** – Dissemination and communication  
Annarita MICCIO

**WP8** – Coordination and management  
Annarita MICCIO

**WP9** – Ethics requirements  
Annarita MICCIO

### OUTPUTS

**Efficient correction of SCD HSPCs with minimal impact on their properties and genome integrity**

**Catalogue of best practice tools and protocols for genome editing-based therapeutic approaches**

### IMPACTS

**Scientific impact**  
Cutting edge methodologies in gene therapy for hematological disorders, using site-specific nucleases or base editors

**Technological impact**  
Catalyze the development of novel strategies for the treatment of other genetic and non-genetic and develop clinically relevant approaches to improve the genome editing approaches of genome editing

**Societal impact**  
Improvement of patient expectancy, quality of life, and of EDITSCD, their families, public and stakeholders

**Economic impact**  
Development of novel therapeutic approaches for the anticipation of SCD

### ABSTRACT

Sickle cell disease (SCD) is a result of a single amino acid substitution in the beta-globin chain of adult hemoglobin. The severity of SCD is alleviated by the co-inheritance of mutations driving the expression of fetal gamma-globin in adults. Transplantation of autologous, genetically modified hematopoietic stem/progenitor cells (HSPC) represents a therapeutic option for SCD. The EU-funded EDITSCD project aims to understand the molecular and cellular mechanisms underlying SCD HSPC dysfunctions and evaluate the impact of genome editing approaches on SCD HSPC. The objective of this study is to improve the SCD gene therapy strategy and evaluate the best tools and protocols for HSPC-based genome editing therapies.

### HORIZON EUROPE PROJECT

## Assessing efficacy and safety of genome EDITing approaches for Sickle Cell Disease

# EDITSCD

**Call**  
HORIZON Research and innovation Actions  
HORIZON-HLTH-2021-TOOL-06-02

**Total EC contribution**  
6.001.250,00€

**Start month**  
September 2022

**Duration**  
5 years

**Institute of genetic diseases Imagine**  
24, Boulevard du Montparnasse  
75015 Paris, France

**Coordinator**  
Annarita MICCIO, Lab director  
annarita.miccio@institutimagine.org

**Project Manager**  
Priyanka LOGANATHAN  
priyanka.loganathan@institutimagine.org

**editscd.eu**

EDITSCD project has received funding from the European Union's Horizon Europe research and innovation programme under grant agreement No 101057659



# The program

- **Session 1: Introduction to Sickle Cell Disease (SCD) and Gene Therapy**

Date: 26 September 2025

Speaker: Annarita Miccio (Imagine Institute des Maladies Génétiques) and Prof. Michaela Semeraro (INSERM CIC1419 and AP-HP.Centre - Université de Paris-Cité)

- **Session 2: Basics of Gene Therapy with Lentiviral Vectors**

Date: by the end of November

Speakers: Annarita Miccio (Imagine Institute des Maladies Génétiques) & Giuliana Ferrari (IRCCS Ospedale San Raffaele)

- **Session 3: Genome Editing: CRISPR/Cas9 and SCD - How It Works and Its Uses**

Date: 16 February 2026

Speaker: Annarita Miccio (Imagine Institute des Maladies Génétiques) & Claudio Mussolino (Universitaetsklinikum Freiburg) & Mario Amendola (Institut National de la Santé et de la Recherche Médicale - INSERM)

- **Session 4: Genome Editing: CRISPR/Cas9 Advanced Tools and SCD - Using New Methods**

Date: 15 April 2026

Speakers: Annarita Miccio (Imagine Institute des Maladies Génétiques) & Marcello Maresca (AstraZeneca AB)

- **Session 5: Safety of CRISPR/Cas9**

Date: 3 June 2026

Speakers: Ayal Hendel (Bar-Ilan University) & Toni Cathomen (Universitaetsklinikum Freiburg)

- **Session 6: Future Developments and CRISPR/Cas9 for SCD**

Date: 17 July 2026

Speaker: Annarita Miccio (Imagine Institute des Maladies Génétiques)

- **Session 7: Regulatory Path to the Clinic**

Date: 2 October 2026

Speaker: David Morrow (EATRIS)





# The model of the ERN-EuroBloodNet for Online Education:

- **Online live program:** speaker presentation & interaction with the audience
- **Recorded videos** implemented on the e-learning platforms (website, Youtube) with the PDF of the PPT presentation
- **Worldwide free open access** both for live sessions (after registration) & recorded videos
- **Dissemination** plan via mailing list, scientific society and social media
- **AI tool for interactive interpretation** of the lecture into national languages



The background is an abstract composition featuring a gradient from deep blue on the left to vibrant red on the right. Numerous semi-transparent circles of various sizes are scattered across the frame, some appearing to float or move, creating a sense of depth and motion. The text is centered in the middle of the image.

# **A SOLID METHOD FOR DEVELOPING THE PROGRAM**



## Method

### EXPERT PROFESSIONALS

- Consensus meeting for identifying contents, topics & speakers
- Individual meetings with the speakers for identifying key concepts

### PATIENT ADVOCATES

- Program validated by SCD patients advocates
- Identification of patients pedagogical needs with live council meetings

- ✓ Guidelines for delivering webinars in lay language in order to target patients audience
- ✓ A team of medical writer taking into account expert knowledge and patients needs





# Guidelines for Delivering Webinars in Lay Language to Target Patients

- Keep slides clear and concise (no more than 5 bullet points per slide).
- Avoid excessive text – prioritize visuals (diagrams, infographics, animations).
- Use plain language and define all scientific terms clearly.
- Stick to a consistent slide structure (title, key message, visual, and example).
- Use patient-friendly analogies (e.g., “CRISPR is like a molecular pair of scissors”).
- Address Common Misconceptions and Concerns



# Guidelines for Delivering Webinars in Lay Language to Target Patients

**1. Briefly introduce yourself & your expertise.**

**2. Learning Objective:** Make it clear what you want the audience to take away from the talk (e.g., understanding gene therapy and its impact on patient care).

**3. Define Scientific terms in simple language/ Avoid assuming prior knowledge**

- **Example:** "Gene therapy is a way to fix or replace faulty genes in the body. Think of it like replacing a broken part in a machine. + **Analogies can help:** "Lentiviral vectors are like delivery trucks that bring new genetic instructions to your cells."

**4. Divide complex notions step-by-step Process:**

- **Example:** «First, doctors collect blood cells. Then, they introduce the healthy gene. Finally, those cells are returned to the patient, where they start producing healthy blood cells»





# Guidelines for Delivering Webinars in Lay Language to Target Patients

## 5. Use Clear Transitions:

- **Example:** «Now that we understand the basics of gene therapy, let's talk about how it works in practice»

## 6. Show visuals, diagrams, or animations

- **Example:** « Imagine a child with sickle cell disease. Before gene therapy, they were frequently in the hospital for transfusions. After gene therapy, they may have fewer hospital visits and better quality of life. »

## 7. End with a Strong Conclusion

- **Example:** « So, to recap: gene therapy offers hope for treating sickle cell disease by correcting faulty genes and improving patients' lives. »

## For each webinar:

### STEPS

A bridge between complex science and patient-friendly communication

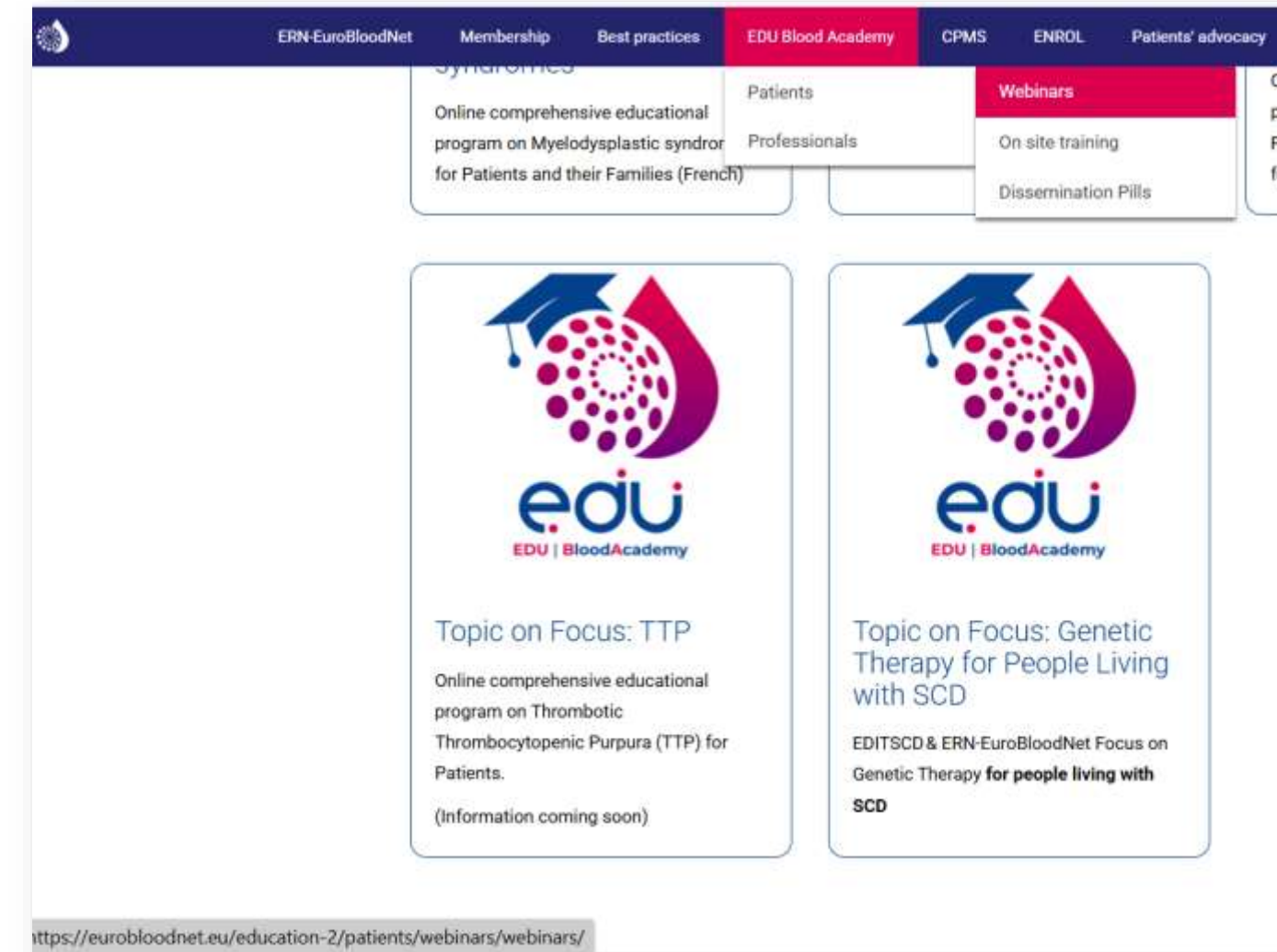
1. 1st meeting with the medical writers, session speakers, patient advocates, Annarita, and Mariangela - as coordinators of the program - to identify the key messages of the webinar
2. The group receives a narrative script of the session from the medical writers
3. The group provides feedback
4. The medical writers incorporate the feedback and prepare the first draft of the PowerPoint presentation
5. 2nd meeting to review and provide feedback on the slides
6. The speakers receive the final slides





# Dissemination Plan

- Visual Identity of the program
- A dedicated section on the e-learning environment of the ERN
- Newsletter & Social Media
- Content of the website including Registration Form
  - Registration to the session/s.
  - Distribution of satisfaction questionnaires and gathering of answers.
  - Repository of content for publicly available.
  - Automatic statistics of the participation in each webinar.



## EDITSCD & ERN-EuroBloodNet Focus on Genetic Therapy for People Living with Sickle Cell Disease (SCD)

26th September 2025 - 17:00h CET

### "Session 1: Introduction to Sickle Cell Disease (SCD) and Gene Therapy "



Annarita's main research interests are the transcriptional control of hematopoiesis, and the development of therapeutic approaches to hematologic genetic disorders. (...) **Michaela Semeraro** is a pediatrician and head of the Clinical Investigation Center at Necker Hospital, Paris, a leading hub for pediatric research. (...)

[Read more & register](#)

ERN-EuroBloodNet and the EDITSCD Consortium are launching a series of webinars entitled EDITSCD & ERN-EuroBloodNet: **Focus on Genetic Therapy for People Living with SCD**, aimed at providing an overview of gene therapy and genome editing approaches for Sickle Cell Disease.



## EDU BloodAcademy

### ACTIVITIES FOR SEPTEMBER 2025



### SPEAKERS of the month:



Patients Webinars





## News on the ERN-EuroBloodNet website



### New webinar program! EDITSCD & ERN-EuroBloodNet: Explaining Genetic Therapy for People living with Sickle Cell Disease

🕒 2025-07-22

The program starts on the 26th of September registration is open and free to all patients, families and anyone curious about the future of genetic medicine!

[READ MORE →](#)



**ERN-EuroBloodNet (European Reference Network on Rar...**  
1.902 follower  
1m •

 New Webinar Series | Genetic Therapy & Sickle Cell Disease!  
We're excited to announce the launch of EDITSCD & ERN-EuroBloodNet: Focus on Genetic Therapy for People Living with SCD. ... altro

New webinar program!

***"EDITSCD & ERN-EuroBloodNet: Focus on Genetic Therapy for People Living with SCD"***

 **Start date: 26th September 2025**

 con Tu e 14 altri

Linkdn, Imagine Institute



Each session has a QR codes for accessing to content and registration







## Engaging the audience

- One webinar every 2 months
- In the month in between, the video recording will be published and integrated into the ERN-EuroBloodNet e-learning platform
- The video will be advertised and promoted
- The use of AI tools for translating the recording into native languages will further boost accessibility and reach







## The first session

### EDITSCD & ERN-EUROBLOODNET FOCUS ON GENETIC THERAPY FOR PEOPLE LIVING WITH SCD

**SESSION 1: INTRODUCTION TO SICKLE CELL  
DISEASE (SCD) AND GENE THERAPY**



26th September 2025  
At 17:00 (CET)

Webinar provided by:



**ANNARITA MICCIO**



**MICHAELA SEMERARO**





## Ideas for the future

- Assess the program
- Evaluate if other sessions are needed
- Publish a paper on the methods
- Roundtable with Researchers-Patients



# EDITSCD



[www.editscd.eu](http://www.editscd.eu)



Martina Franchini • Antonio Carusillo • Manel Llado-Santaeularia



[www.eurobloodnet.eu](http://www.eurobloodnet.eu)



@ERNEuroBloodNet



eurobloodnet-european-reference-network-on-rare-hematological-diseases



Eurobloodnet - European Reference Network on Rare Hematological Diseases



ERN-EuroBloodNet's EDUcational Youtube channel



Funded by the European Union. Views and opinions expressed are however those of the author(s) only and do not necessarily reflect those of the European Union or European Health and Digital Executive Agency (HaDEA). Neither the European Union nor the granting authority can be held responsible for them.



Funded by  
the European Union



# Thank you!

