EDITSCD-webinar program on gene editing

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European Reference Network | Hematological Diseases (ERN EuroBloodNet)

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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)

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

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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. »

A bridge between complex science and patient-friendly communication

STEPS

For each webinar:

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.

Newsletter

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.*

ERN | EuroBloodNet

EDU | BloodAcademy

European Reference Network for rare or low prevalence complex diseases

Network Hematological Diseases (ERN EuroBloodNet)

EDU BloodAcademy

ACTIVITIES FOR **SEPTEMBER 2025**

HIGHLIGHTS

GENE THERAPY | THURSDAYS WEBINARS | TOPIC ON FOCUS

SPEAKERS of the month:

JOSE CARLOS SEGOVIA SANZ | MERCEDES DESSY RODRIGUEZ | OSCAR QUINTANA BUSTAMANTE | ISABEL OJEDA-PÉREZ | LOREDANA BURY | PIERRE FENAUX | ANNARITA MICCIO | MICHAELA SEMERARO

Patients Webinars

News on the ERN-EuroBloodNet website

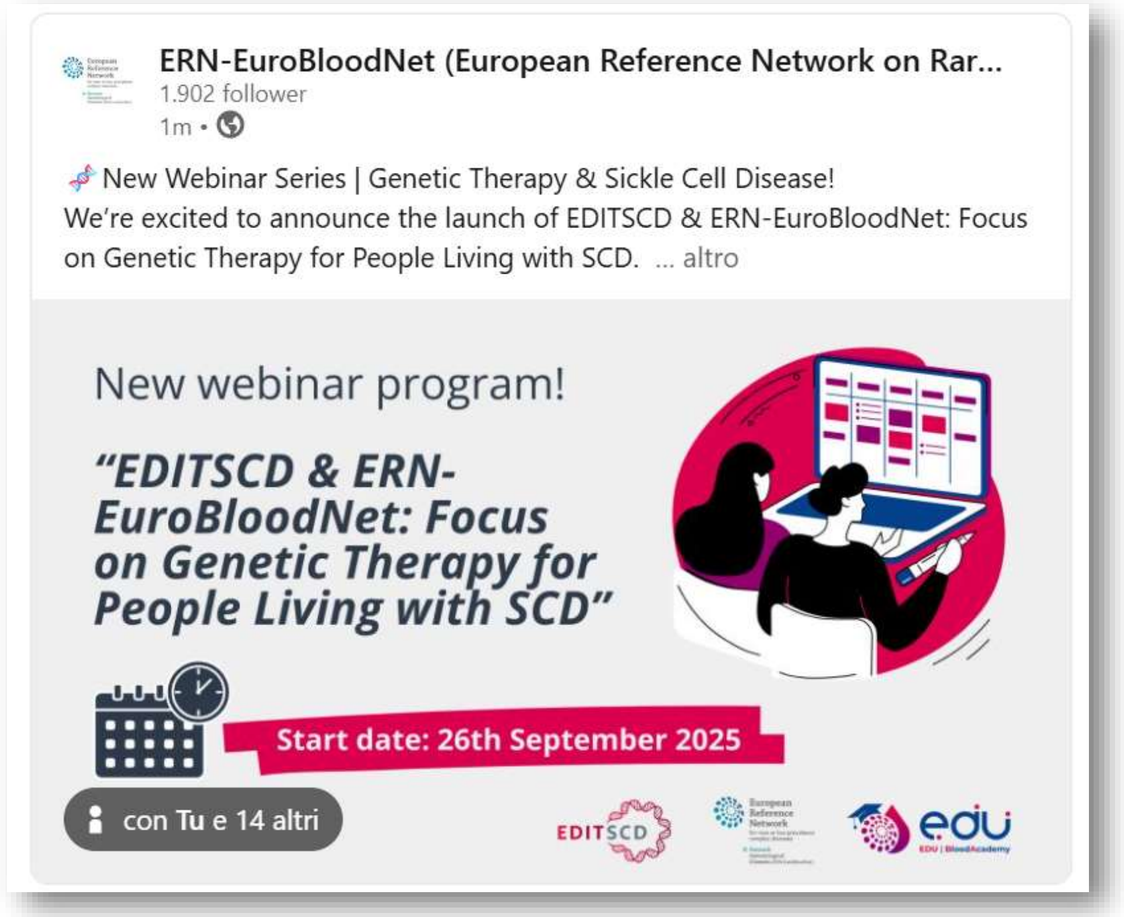
New webinar program! EDITSCD & ERN-EuroBloodNet: Explaining Genetic Therapy for People living with Sickle Cell Disease

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 →

Social media



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

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

WE DO CRISPR

Martina Franchini • Antonio Carusillo • Manel Llado-Santaeularia

webinar

www.eurobloodnet.eu

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Eurobloodnet - European Reference Network on Rare Hematological Diseases

ERN-EuroBloodNet's EDUcational Youtube channel

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Thank you!

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