

October

DAY 1 SCHEDULE

Wednesday

REGISTRATION

8:00 AM – 4:00 PM

Registration & Information

The registration desk is open for check-in, material collection, and event information

BREAKFAST

8:30 AM – 9:05 AM

Welcome Breakfast

OPENING ADDRESS

9:00 AM – 9:05 AM

ASCAT 2024: Opening Address

Official start of ASCAT 2024 with an address highlighting conference goals and upcoming sessions
Biree Andemariam (US)

LECTURE

9:05 AM – 9:45 AM

The Pathophysiology

- a. Molecular and cellular mechanisms of Sickle Cell Disease
Michael DeBaun (US)
- b. Genetic and physiological aspects of Thalassemia
Ali Taher (Lebanon)

10:05 AM – 10:25 AM

Growth & Development

- a. Impact of Sickle Cell Disease on growth and development
- b. Analysis of growth and development challenges in Thalassemia
Maddalena Casale (Italy)

10:25 AM – 11:05 AM

Fertility (Male & Female)

- a. Female Preservation: Strategies for preserving fertility in females with Sickle Cell Disease and Thalassemia
Sheila Lane (UK)
- b. Male: Overview of fertility issues in males with Sickle Cell Disease and Thalassemia
Pippa Sangster (UK)

BREAK

11:05 AM – 11:20 AM

Tea & Coffee Break

SYMPOSIUM

11:20 AM – 12:20 PM

Pfizer Symposium

From Experience to Evidence: Redefining SCD Treatment Goals
Speakers selected by Pfizer

BREAK

12:20 AM – 1:20 PM

Lunch Break

Food is provided. A time for delegates to eat, relax, and network

LECTURE

1:20 PM – 2:00 PM

Aging- Organ Impairment & Emerging Morbidities – Sickle Cell Disease

Examination of aging effects in Sickle Cell Disease, focusing on organ impairment and new health challenges

- a. Retinopathy
Christiana Dinah (UK)
- b. Spleen
Valentine Brousse (France)

PARALLEL SESSIONS

2:00 PM – 3:45 PM

Aging- Organ Impairment & Emerging Morbidities – Thalassemia

Discussion on aging impact in Thalassemia, including organ dysfunction and new morbidities:

- a. Thrombosis
Rayan Bou-Fakhredin (Italy) & Maria Domenica Cappellini (Italy)
- b. Malignancies
Rayan Bou-Fakhredin (Italy) & Ali Taher (Lebanon)
- c. Renal Disease
Antonis Kattamis (Greece)
- d. Bone Disease
Chloe Merrion (UK) & Julian Waung (UK)
- e. Cardiac Rhythm
Amna Abdel-Gadir (UK) & Malcolm Walker (UK)

PARALLEL SESSIONS

2:00 PM – 3:45 PM

Global Approaches to Newborn and Population-based Screening

- a. Saudi Arabia
Hatoon Ezzat (Saudi Arabia)
- b. Lebanon
Michele Abi Saad (Lebanon)
- c. Cyprus
Michael Angastiniotis (Cyprus)
- d. Nigeria
Norah Akinola (Nigeria)
- e. Zambia
Catherine Chunda (Zambia) & Josh Wright (UK)

BREAK

3:45 PM – 4:00 PM

Tea & Coffee Break

LECTURE

4:00 PM – 5:00 PM

Treatment (excl. Gene Therapy)

- a. Comprehensive overview of treatment strategies for Sickle Cell Disease
Rachel Kesse-Adu (UK)
- b. Detailed review of treatment approaches for Thalassemia
Irene Motta (Italy)
- c. Examination of standard care practices for TDT and NTD, and new pharmacological treatments
Khaled Musallam (UAE)

5:00 PM – 6:00 PM

Haematopoietic Stem Cell Transplantation

- a. Insights into the role and outcomes of transplantation as treatment for Sickle Cell Disease
Josu De la Fuente (UK)
- b. Overview of transplantation in Thalassemia, including success rates and challenges
Emanuele Angelucci (Italy)
- c. Analysis of secondary malignancies risk post-transplantation
Adetola Kassim (US)

October

3

DAY 2 SCHEDULE

Thursday

REGISTRATION

8:00 AM - 4:00 PM

Registration & Information

The registration desk is open for check-in, material collection, and event information

PARALLEL SESSIONS

8:15 AM - 9:15 AM

Meet The Expert

Informal & interactive sessions with leading professionals. Breakfast is served in the room.

a. Bone Marrow Transplant in Children

Jean Hugues Dalle (France)

b. Writing a Manuscript

Lan Lan Smith (UK)

c. How to Design a Pre - Clinical Study

Thiago Trovati (France)

d. Alternatives to Pain Management

Natasha Archer (US)

e. Strategies adopted for Sickle Cell Mission in India - Prevention, Management & Research

Vinita Srivastava (India)

BREAK

9:15 AM - 9:30 AM

Tea & Coffee Break

PARALLEL SESSIONS

9:30 AM - 11:30 AM

Oral Abstract Presentations (Part 1)

Formal presentations of peer-reviewed research on Haemoglobinopathies

Various speakers

LECTURE

11:30 AM - 11:50 AM

Differential Diagnosis of Rare Inherited Anaemia

Case studies on diagnosing Rare Inherited Anaemia

Noemi Roy (UK)

BREAK

11:50 AM - 1:00 PM

Lunch Break

Food is provided. A time for delegates to eat, relax, and network

SYMPOSIUM

1:00 PM - 2:00 PM

Agios Symposium

Session Sponsored by Agios:

Activation of Pyruvate Kinase with Mitapivat - Latest Data in Thalassaemia and Sickle Cell Disease

Speakers selected by Agios

PARALLEL SESSIONS

2:00 PM - 5:30 PM

Helios Symposium

Session Sponsored by Helios:

Fostering Collaboration and Harmonisation in Haemoglobinopathy Research and Training through Synergistic Networking

Speakers selected by Helios

LECTURE

2:00 PM - 3:15 PM

Oral Abstract - Top 5

Formal presentation of the top scoring abstracts

Mary Akua Ampomah (Ghana)

Ibrahim Idris (Nigeria)

Duah Dwomoh (Ghana)

Margherita Di Mauro (Italy)

Justin Yoo (US)

PATIENT SESSION

2:30 PM - 5:30 PM

ASCAT x Eurobloodnet Patient Session - Closed (Parallel)

ERN-EuroBloodNet Patients Educational Session:

Adult SCD Patients Mentoring Teenagers

Speakers selected by ERN - Eurobloodnet

BREAK

3:15 PM - 3:30 PM

Tea & Coffee Break

LECTURE

3:45 PM - 4:45 PM

Gene Therapy

a. Mechanistic Approaches to Gene Therapy for Sickle Cell Disease and Thalassaemia

b. Clinical Data for Gene Therapy in Sickle Cell Disease: An Update

c. Clinical Data for Gene Therapy in Thalassaemia: An Update

Alexis Thompson (US)

Julie Kanter (US)

4:45 PM - 6:00 PM

Treatment in Sickle Cell Disease & Thalassaemia: Decision Making

a. Role of Psychologists in shared decision making.

Lori Crosby (US)

b. MDT approach for Curative Therapy.

Janet Kwiatkowski (US)

c. Timing and reasons for choosing Haplo Donor HSCT.

Adetola Kassim (US)

October

DAY 3 SCHEDULE

Friday

REGISTRATION

8:00 AM – 4:00 PM

Registration & Information

The registration desk is open for check-in, material collection, and event information

PARALLEL SESSIONS

8:15 AM – 9:15 AM

Meet The Expert

Informal & interactive sessions with leading professionals. Breakfast is served in the room

- Hydroxyurea use in Sub-Saharan Africa

Leon Tshilolo (DRC)

- Incorporating patient perspectives into research/trial design

Leon Tshilolo (DRC)

- Adult stroke in SCD – (case and discussion)

Paul Holmes (UK)

- Stroke (incidence and treatment and prevention) in Sub-Saharan Africa

Shehu Abdulla (Nigeria) & Michael DeBaun (US)

- A Focus on Red Cell Research : How to Construct a Research Project

Sara El Hoss (France) & David Rees (UK)

BREAK

9:15 AM – 9:30 AM

Tea & Coffee Break

PARALLEL SESSIONS

9:30 AM – 10:45 AM

PARALLEL A

Development & Implementation of Multi-National Networks

This session explores various global initiatives aimed at improving the management and treatment of Sickle Cell Disease and Thalassaemia through Multi-National Collaborations.

- ARISE (Global)

Duccio Bonifazi (Italy)

- Sickle in Africa (Sadaac/SPARCO)

Vicky Newambare (South Africa)

- CONSA

Enrico Novelli (US)

- HELIOS (Sickle & Thalassaemia)

Petros Kountouris (Cyprus)

- TIF

Androulla Eleftheriou (Cyprus)

9:30 AM – 10:45 AM

PARALLEL B

Basic Research Session (Part 1)

Dedicated to discussing fundamental research topics and breakthroughs:

- What is Basic Science Research – From Bed to Bench and from Bench to Bed

Sara El Hoss (France)

- Red Cell Biology – From the Bone Marrow to the Circulation

Wassim El Nemer (France)

- Iron Metabolism in Thalassemia

Stefano Rivella (US)

- Clonal Hematopoiesis in Hemoglobinopathies

Olivier Hermine

- Gene Therapy in Haemoglobinopathies

Annarita Miccio (France)

9:30 AM – 10:45 AM

PARALLEL C

Pfizer Sponsored Session

Empowering change: Addressing the unmet needs in SCD

Prof. Lori Crosby

Dr. Abdullah Al Zayed

BREAK

10:45 AM – 11:00 AM

Tea & Coffee Break

SYMPOSIUM

11:00 AM – 12:00 PM

EHA Partner Symposium

Evolution of Therapies in Hemoglobinopathies:

This presentation will delve into the latest advancements in treating hemoglobinopathies, focusing on three therapeutic approaches. We will explore the potential of voxelator and CRS-601 in inhibiting polymerization, the role of PK-R activation in sickle cell disease and thalassemia, and new data on transfusion in managing these debilitating disorders.

- Inhibition of polymerization: Voxelator and -601

Modupe Idowu (US)

- PK-R activation in Sickle Cell Disease and Thalassemia

DeBeers E (Netherlands)

- Assessing red cell concentrate quality using in vitro markers predictive of transfusion efficacy

Amireault Pascal (France)

BREAK

12:00 PM – 1:00 PM

Lunch Break

Food is provided. A time for delegates to eat, relax, and network

SYMPOSIUM

1:00 PM – 2:00 PM

Vertex Symposium

Session Sponsored by Vertex:

Transforming the treatment landscape for patients with haemoglobinopathies:

Perspectives from the multidisciplinary team

Speakers selected by Vertex

PATIENT SESSION

2:00 PM – 5:00 PM

ASCAT x Eurobloodnet Patient Session – (Closed)

ERN-EuroBloodNet Patients Educational Session:

Communication Between Parents and Teenagers for Building Autonomy (Using PROMs)

Speakers selected by ERN – Eurobloodnet

PARALLEL SESSIONS

2:00 PM – 3:00 PM

PARALLEL A

Basic Research (Part 2)

Dedicated to discussing fundamental research topics and breakthroughs:

- Inflammation in SCD and Thalassemia

Nicola Conran (Brazil)

- MAST Cells in Sickle Cell Disease

Slimane Allali (France)

- ADAMTS13 in hemoglobinopathies

Lucia De Francesci (Italy)

- Sickle Cell Disease Research in Tanzania

Florence Urio (Tanzania)

- Role and Mechanisms of the Haem-Haemopexin Axis in Organ Dysfunctions in Sickle Cell Disease

Solomon Ofori-Acquah (Ghana)

2:00 PM – 3:00 PM

PARALLEL B

Pain Management : Models of Care – A Global View

Presents different models of care for Pain Management from a global perspective

Wally Smith (US)

Sanne Lugthart (UK)

Rana Yamout (Lebanon)

Mona El Ghamrawy (Egypt)

2:00 PM – 3:00 PM

PARALLEL C

Xcene Research Symposium

Conducting SCD Trials in Africa – Success Stories and Lessons Learned:

This workshop is to showcase the achievements, best practices, and key learnings from successful Sickle Cell Disease (SCD) trials conducted in Africa in collaboration with Xcene Research, a leading

Contract Research Organization (CRO) with expertise in the African region.

Speakers selected by Xcene Research

BREAK

3:00 PM – 3:15 PM

Tea & Coffee Break

PARALLEL SESSIONS

3:15 PM – 4:30 PM

PARALLEL A

Enhancing Clinical Research in Countries with Limited Resources

- Thailand/Indonesia

Vip Viprasit (Thailand)

- Tanzania

Julie Makani (Tanzania)

- Kenya

Bernards Ogutu (Kenya)

- Nigeria

Obiageli Nnodu (Nigeria)

3:15 PM – 4:30 PM

PARALLEL B

Psychosocial Perspective on Care in Sickle Disease & Thalassaemia – Current Status Across the World and Impact of Social Determinants

- Africa

Daima Bukini (Tanzania)

- Middle East

Maya Bizri (Lebanon)

- US

Andrew Heitzer (US)

3:15 PM – 4:30 PM

PARALLEL C

Collaborative Research Catalyst Session – Closed (Parallel)

Offering three ASCAT attendees the unique chance to present their research ideas to a panel of experts

and receive valuable feedback in a supportive, interactive environment

Participants selected by The Collaborative Research Catalyst.

POSTER WALK

4:30 PM – 6:00 PM

Poster Walk

A session dedicated to viewing and discussing research posters.

GALA NIGHT

7:00 PM – 12:00 AM

Gala Night (Additional Registration Required)

An evening event featuring dinner, entertainment, and a charity fundraiser.

Organised by ASCAT Trustess

October

DAY 4 SCHEDULE

Saturday

REGISTRATION

8:00 AM – 11:00 AM ———— **Registration & Information**

LECTURE

9:30 AM – 10:15 AM ———— **Algorithm for Selecting the Best Candidates for Gene Therapy**
Discusses the criteria and decision-making processes for identifying the best candidates for gene therapy in haemoglobinopathies.
Marsha Treadwell (US)
Sickle Cell Disease - **Mariane De Montalambert (France)**
Thalassemia - **Maria Domenica Cappellini (Italy)**

PANEL DISCUSSION

10:15 AM – 11:00 PM ———— **Discuss Different Therapeutic Approaches in SCD (Basic Care vs Curative Options): A Physician Perspective**
The priority for care in 2024 should be:
i. Getting basic care (Hydroxy etc) right in the majority
ii. Establishing gene therapy as a basic care right
Explores the balance between basic care and curative options for Sickle Cell Disease from a physician's point of view, emphasizing the priorities for 2024
Julie Makani (Tanzania)
Leon Tshilolo (DRC)
Hatoon Ezzat (Saudi Arabia)
Raffaella Colombatti (Italy)

BREAK

11:00 AM – 11:15 AM ———— **Tea & Coffee Break**

PATIENT SESSION

11:15 AM – 1:15 PM ———— **ASCAT x Eurobloodnet Patient Session - Closed (Parallel)**
ERN-EuroBloodNet Patients Educational Session:
Workshop on Patient Storytelling: Transitioning Through Linguistic and Cultural Differences
Speakers selected by ERN - Eurobloodnet

PARALLEL SESSION

11:15 AM – 1:15 PM ———— **Oral Abstract Presentations**
Formal presentations of peer-reviewed research on Haemoglobinopathies
Various speakers
See Timetable

BREAK

1:15 PM – 2:15 PM ———— **Lunch Break**
Food is provided. A time for delegates to eat, relax, and network

LECTURE

2:15 PM – 3:15 PM ———— **Impact of a Patient and Clinician Survey – the UK output after the APPG report**
Discusses the results and implications of a survey involving patients and clinicians in the UK, following the APPG (All-Party Parliamentary Group) report
a. NHP/HCC/NHR network arrangement
John Porter (UK)
b. Hyperacute units
Dianne Addei/Joseph Sharif (UK)
c. Universal Care Plan
Banu Kaya/Subarna Chakravorty (UK)
d. ACTNOW – development and implementation
Sara Stuart Smith (UK)
e. The London Community bid – how funding becomes care
Ella Wilson (UK)

PATIENT SESSION

3:15 PM – 3:30 PM ———— **ERN - Eurobloodnet Patient Educational Session Summary**
Presentation of ERN-EuroBloodNet Patient Educational Sessions

SURVEY SESSION

3:30 PM – 4:00 PM ———— **ASCAT 2024: Closing Address**
ASCAT Conference Recap
ASCAT Closing Remarks
Andrew Campbell (US)
Biree Andemariam (US) & Wale Atoyebi (UK)