

Synthesis of the Global Congress & Reflections

Coming together

- Last global congress 5 years ago
- Pandemic impact
- Great to see each other in-person
- We lost a leader, colleague, friend, mentor and champion

Some progress made

- Newborn screening initiatives
- Establishment of SCD treatment programs
- New disease-modifying therapies
 - Wider use of hydroxyurea
 - From 1 to 4 approved drugs
 - 30+ in development pipeline
- Curative therapies
 - BMT strategies
 - Emerging gene therapy and gene editing approaches

Areas covered in this congress

- Patients' perspectives
 - Opening and closing ceremonies
- State of SCD around the world
- Basic/Translational/Clinical Science
 - Frontiers of science
 - Global research efforts

Robust program

- Basic/translational/ clinical science
 - Advances in SCD pathobiology
 - Evolutional genetics of SCD
 - Organ dysfunction
 - Clinical variability in SCD
 - Neonatal Screening
 - Transfusion therapy
 - COVID-19
 - Pregnancy
 - Psychosocial
 - Pain and integrative health
 - Neurological complications

Robust program

- How to enhance teaching of SCD
 - State of the art including digital strategies
- Curative therapies
 - Hematopoietic stem cell transplantation
 - Gene therapy and gene editing

Focus on LMICs

- Research in Africa
- International networks
- Session in French

State of SCD around the world

- Full circle
 - Africa
 - India
 - West Indies
 - Brazil
 - Europe
 - USA
- Different challenges
- Common themes
 - Social/economic determinants

Patients' participation

- Opening ceremony
- Dedicated patients' session
 - Fatigue
 - Experience of pain and BMT
 - Living, growing and aging with SCD
- Patient's testimony on curative treatment
- Patients' declaration

Reflections

- Patients and caregivers at the centre of what we do
- More treatment centres needed
- Diagnose SCD at every level
 - Newborns
 - Neonates
 - Infants
 - Health system encounter

Reflections

- We need a global solution to make hydroxyurea available and affordable
- Remove barriers to HU access
 - Health care providers
 - Patients and families
 - Health systems
- Multi-sector, multi-stakeholder partnerships are crucial
 - Global health funders
 - Academic societies: ASH, EHA and others
 - Academic and healthcare institutions
 - Industry
 - NGOs
 - Governments
 - World Health Organization
 - Advocacy groups

Reflections

- Let's emphasize the burden of SCD
 - Physical
 - Psychosocial and mental health
 - Socioeconomic determinants
- Patients' voice

Emerging therapies and equity

- New therapies should build on hydroxyurea as a foundation
- More research needed to advance their use in low-resource settings
 - BMT protocols that can be delivered at low-cost
 - Gene delivery approaches
 - In vivo
 - Not myeloablative
 - Stem cell mobilization- safe and effective
 - Appropriate vectors
 - Genome editing innovations
 - Base editing
 - Prime editing

Thank you