

# Synthesis of the Global Congress & Reflections

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**GLOBAL SICKLE CELL  
DISEASE NETWORK**

# 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



**GLOBAL SICKLE CELL  
DISEASE NETWORK**

# 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
  - Caribbean
  - 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

