HEMATOPOIETIC STEM CELL TRANSPLANTATION AND NEWBORN SCREENING: MENA update

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Introduction

• HSCT is a highly specialized and unique medical procedure. (autologous, allogeneic)

• The first allogeneic transplantation was performed by E. Donnall Thomas in 1957.

• Since then the field has evolved and expanded worldwide. New indications beside acute leukemia and aplastic anemia have been constantly explored and now include congenital disorders of the hematopoietic system, metabolic disorders, and autoimmune disease.
Introduction

• (HSCT) is the only therapeutic approach that can arrest cerebral demyelination of X-linked adrenoleukodystrophy (ALD) in boys.
• The MPS diseases benefiting most significantly from HSCT include MPS I (Hurler), MPS VI (Maroteaux-Lamy), and MPS VII (Sly).
• Reduce morbidity and mortality for SCID
Evidence clearly shows that HSCT has the best outcome when performed on an asymptomatic individual with X-ALD. (Shapiro et al, 2000)

Long-term effect of bone-marrow transplantation for childhood-onset cerebral X-linked adrenoleukodystrophy

Hematopoietic Stem Cell Transplantation for Severe Combined Immunodeficiency Diseases

Morton J. Cowan,1 Benedicte Neven,2 M. Cavazanna-Calvo,2 A. Fischer,2 Jennifer Puck3

Biology of Blood and Marrow Transplantation 14:73-80 (2008)

Allogeneic Hematopoietic Stem Cell Transplantation in Thirty-Four Pediatric Cases of Mucopolysaccharidosis—A Ten-Year Report from the China Children Transplant Group

Biol Blood Marrow Transplant 22 (2016) 2100–2108
Wilson & Jungner criteria Revisited

- Knowledge of the disorder and its natural history: **High mortality and morbidity**
- Availability of, access to treatment: **Ability to be cured if it is recognized early**
- Availability “simple” test,
- **inexpensive test**
- Follow up of “screen positive” newborns for confirmatory testing, introduction and monitoring of treatment
- Prevalence in the “target population”: **High consanguinity rate**
- Cost benefit Screening: **“Governments are paying for the treatment with or without treatment”**
- The principles as outlined earlier were acceptable.
Newborn Screening Grows Up

It started with one test in one state. But technological advancements, increased awareness, and federal action in recent years have spurred sharp growth in the average number of conditions states include in their newborn screening programs.
More than a decade ago Screening trials started:

- Mei Baker et al in 2009 (SCID)

CDC free solutions for LSD started in 2007-2008
In February 2016, The Secretary of the US department of Health and Human Services recommended the Addition of X-ALD to the standard neonatal Screening panel.
“Simple test : Ready to use kits”

• For X-ALD : Measuring C26-LPC

• For SCID : TREC measurement

• For LSD : MSMS Screening have the possibility of multiplex and second tiers markers or other
MENA Region

22 countries
60% of the world's oil reserves
6% of the world's population,
45% of the world's natural gas reserves

Northern Africa and the Middle East
• Region of the Middle East and North Africa (MENA) consists of more than twenty countries
• Population of the region is about 400 million, 
• An estimated 10 million newborns per year
• Significant diversity between the countries in relation to population size, per capita income, health system, insurance coverage and differences in the stages of epidemiological transition.
• High rates of consanguinity and first cousin marriages, genetic disorders are relatively common.
Newborn Screening MENA history

• Newborn screening started in the region in the 80’s.

• All Governments Health policies since and still cover the treatment and follow up: Milk formula, ERT, Hematopoietic stem cell transplantation (HSCT), etc,....
Efforts in the region

Newborn Screening Quality Assurance Program

Conference on Strengthening Newborn Screening in the Middle East and North Africa

Japan International Cooperation Agency

International Atomic Energy Agency

CDC

ISNS

International Society for Neonatal Screening
MENA Newborn Screening Overview: National programs (Therell et al, 2015)

- Many countries have national program for at least one disease:
  - Kuwait
  - Egypt,
  - Jordan
  - Palestinian Territories (Occupied),
  - Bahrain
  - Qatar
  - Saudi Arabia
  - United Arab Emirates
  - Iran
  - Lebanon
  - Morocco
No National program Yet

• Algeria
• Tunisia
• Syria
• Oman
• Lybia
• Yemen
• Sudan
SAUDI ARABIA

• Pioneer In Newborn screening in the Region.


KSA had participated in the CDC-LSD free solution newborn screening
High Incidence of Severe Combined Immunodeficiency Disease in Saudi Arabia Detected Through Combined T Cell Receptor Excision Circle and Next Generation Sequencing of Newborn Dried Blood Spots

Hamoud Al-Mousa¹,²,³*, Ghadah Al-Dakheel², Amal Jabr², Fahd Elbadaoui², Mohamed Abouelhoda²,⁴, Mansoor Baig⁵, Dorota Monies²,⁴, Brian Meyer²,⁴, Abbas Hawwari²,⁶ and Majed Dasouki²*
Qatar

"World Allergy International Scientific Congress: Novel Approaches to the world Allergy Epidemic"
4-6 April 2019, Beirut, Lebanon
Lebanon

- National SCID program in 2018: NASPID by Ifight PID

www.ifightpid.org
Conclusion

- In this Area of man made calamities, improvement is remarkable since the Marrakech declaration in 2006 (Saadallah et al, in 2007).
- Newborn screening for X-ALD, LSD and SCID may result in a higher incidence of these disease.
- NBS is not only a test but also a comprehensive system of education, screening, follow-up, diagnosis, treatment or management and evaluation that must be institutionalized and sustained within public health systems.
Parents, pediatric health-care providers and policy makers should be educated about available NBS services in a nation. Cost-effective NBS studies should be conducted; example:

Every newborn deserves access to NBS, diagnosis, treatment and the best quality of life.
SAVE the date APRIL 2020,
4th MENA-ISNS meeting for Newborn Screening,
in Limasol Cyprus

International Society for Neonatal Screening

THANK YOU