

Accessibility Challenges for Rare Diseases Treatments at Mena Region Amyloidosis & Thalassemia Cases

Abdalla Abotaleb

Department World Health Organization, Egypt

*Corresponding author: Abdalla Abotaleb, Department World Health Organization, Egypt.

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Abstract

Background

Regarding the nature of Rare Diseases like

- Nature and definitions for those diseases
- Treatment protocols.
- Targeted paints.
- Technology assessment for those treatments
- Funding and financing for reimbursement strategies.
- Regulatory pathways.

Accessibility of Rare Diseases Treatment is one of the major challenges facing Any Health System In General and Specially Systems Facing scarcity of resources due to Challenges Facing payers at Economic Evaluation fair Classification for Patients Criteria availability of medical and economic Evidence and finally Treatments availability and affordability.

The Objective of this Research is analyzing factors affecting Acceptility for the following diseases

Amyloidosis & Thalassemia in Order to Developing and enhancing Efficient accesses plans and strategies for those Disease areas

Methods

Standardized survey was developed and conducted for 150 of HCPs (healthcare professionals) at the following countries (Algeria, Morocco, Egypt, KSA, UAE, Iraq, Oman, Iran and Turkey) HCPs included (physicians, Nurses, Pharmacists and Payers)

Patient's Groups are represented at the Survey. Previous Survey with Integrated with Health systems data Bases and Nationals Health Accounts analysis for these countries for previous 12 Years. One way sensitivity analysis was conducted for robustness of data guaranteeing.

Results

Analysis for Amyloidosis founded the following findings

- Lack of Awareness for Emphasizing on the problem of Disease
- Lack of Priority Policies Towards Disease.
- Lack of Efficient Policy in Disease Management.
- Lack of Stake holders Engagement.
- Acceptility Challenges presented at Diseases Management policies.
- Lack of Clear Treatment guidelines combined with effective awareness and Specialized Clinics might have positive Impact on Policy Enhancement.

Analysis for Thalassemia founded the following findings

- Enhancement of Work Force Cabalites are remarkable.
- Awareness policies Are efficient.
- Developing Center of Excellences are facing challenges.
- Acceptility for innovative medicines Need More Enhancement
- Adult Paints Acceptility for Treatments Generally Is considering one of major challenges facing Health Systems.
- NGOs Playing Successful model for partnership with Public Sector in Enhancement
- Treatment Policies

Conclusion

For Enhancement Acceptility for Amyloidosis & Thalassemia the following Recommendations should be implemented.

Amyloidosis

- Without Efficient Policy it might represent Burden on Health System.
- Stake holders Engagement represent the corner stone for System Enhancement.
- Acceptility Challenges presented at Diseases Management policies.
- Clear Treatment guidelines combined with effective awareness and Specialized Clinics might have positive Impact on Policy Enhancement.

Thalassemia

- Center of Excellences Establishment.
- Enhancement for Adult Treatment Policies.
- Enhancement Acceptility for Innovative medicines especially for adults

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Amyloidosis

Is one of Rare Diseases is Usually fatal. increasingly recognized in clinical practice despite patients presenting with non-specific symptoms of cardiomyopathy for cardiac Amyloidosis. Amyloidosis is a systemic illness that affects multiple organ systems, including the cardiovascular, renal, gastrointestinal, and pulmonary systems. Common manifestations include restrictive cardiomyopathy, arrhythmias, nephrotic syndrome, and gastrointestinal hemorrhage.

It is unknown whether coexisting atrial fibrillation (AF). due to Amyloidosis is considered as a rare Disease according to The European Union considers a disease as rare when it affects less than 1 in 2,000 citizens. With characterization of wide diversity of symptoms and signs that vary not only from disease to disease

And some time from Patients to Patients.

Thalassemia

Thalassemia is one of the most common genetic disorders among humans. It occurs in many world regions but the highest levels

are recorded in the Mediterranean countries. And Characterized with the following

- Inherited
- passed from parents to children through genes.
- Blood disorder caused when the body doesn't make enough of a protein called hemoglobin

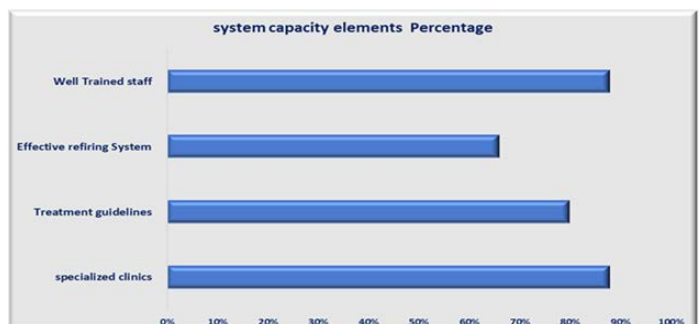
Methods

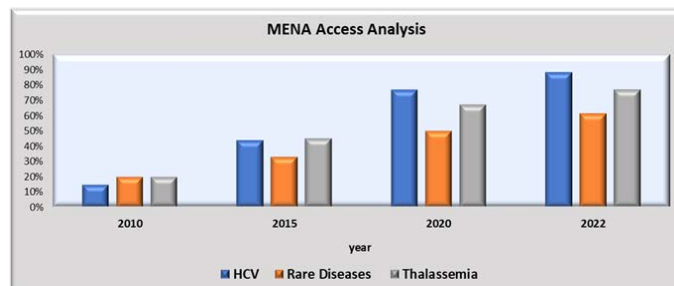
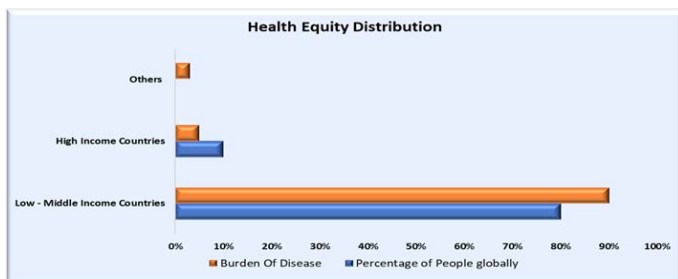
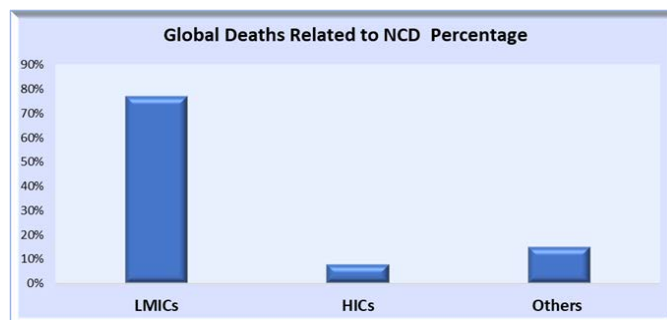
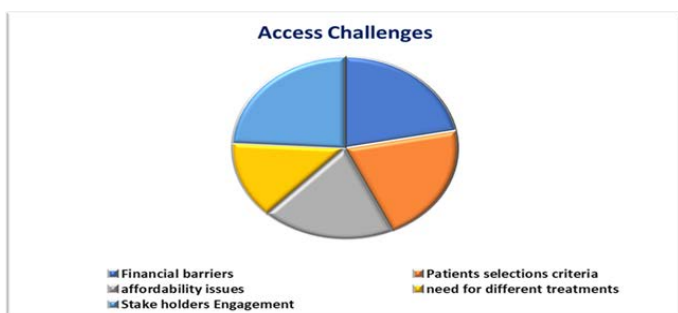
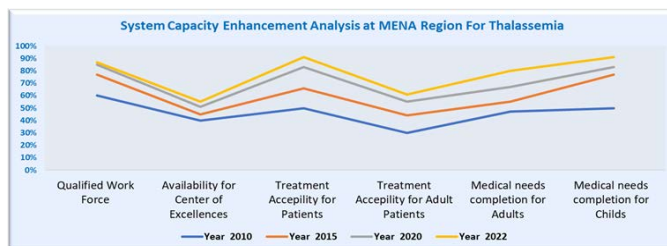
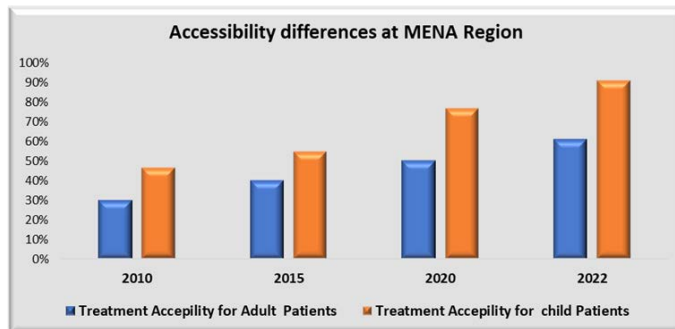
Standardized survey was developed and conducted for 150 of HCPs (healthcar professionals) at the following countries (Algeri, Morocco, Egypt, KSA, UAE Iraq, Oman, Iran and Turkey) HCPs included (physicians, Nurses, Pharmacists and Payers) [1-5].

Patient's Groups are represented at the Survey. Previous Survey with Integrated with Health systems data Bases and Nationals Health Accounts analysis for these countries for previous 12 Years. One-way sensitivity analysis was conducted for robustness of data guaranteeing [6-8].

Analysis for Amyloidosis founded the following findings

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Analysis for Thalassemia Founded the Following Findings

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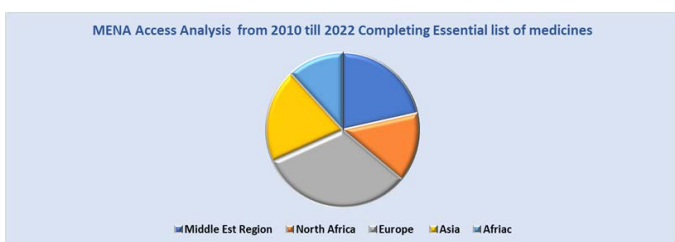
For Enhancement Acceptility for Amyloidosis & Thalassemia the following Recommendations should be implemented [9-14].

Amyloidosis

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Thalassemia

- Center of Excellences Establishment.
- Enhancement for Adult Treatment Policies.
- Enhancement Acceptility for Innovative medicines especial-ly for adults



References

1. Griffin JM, Rosenblum H, Maurer MS (2021) Pathophysiology and therapeutic approaches to cardiac amyloidosis. *Circ Res* (2021) 128: 1554-1575.
2. Nativi-Nicolau J, Maurer MS (2018) Amyloidosis cardiomyopathy : Update in the diagnosis and treatment of the most common types. *Curr Opin Cardiol* 33: 571–579.
3. Macedo AVS, Schwartzmann PV, de Gusmao BM, Melo MDT, Coelho-Filho OR (2020) Advances in the treatment of cardiac amyloidosis. *Curr Treat Options Oncol* 21: 36.
4. Knight DS, Zumbo G, Barcella W, Steeden JA, Muthurangu V, et al. (2019) Cardiac structural and functional consequences of amyloid deposition by cardiac magnetic resonance and echocardiography and their prognostic roles. *JACC Cardiovasc Imag* 12: 823-833.
5. Wechalekar AD, Gillmore JD (2016) Hawkins PN. Systemic amyloidosis. *Lancet* 387: 2641-2654.
6. WHO website (2017) World Bank and WHO: Half the world lacks access to essential health services. <https://www.who.int/news/item/>.
7. WHO (2007) Ten years in public health 2007–17 - Access to medicines: making market forces serve the poor. <https://apps.who.int/iris/bitstream/handle/10665/255355/9789241512442-eng.pdf> .
8. UN SDG web site (2016) agenda <https://sdgs.un.org/2030> .
9. WHO website. Social determinants of health. https://www.air.org/our-work/health/equity-and-social-determinants-health?gclid=CjwKCAjwx_eiBhBGEiwA15gLNyB-MJvFm2RrwCWf2wG-Qk0HGczLepJk1n7npvF3v5L-AOWFEqv1ZqYBoCXhYQAvD_BwE
10. Katherine Keisler-Starkey, Lisa N Bunch US (2019) data, people with no insurance at any point during the year. Source: Census Bureau Current Population Reports, P60-271, Health Insurance Coverage in the United States: 2019, U.S. Government Publishing Office, Washington, DC, 2020 <https://www.census.gov/content/dam/Census/library/publications/2021/demo/p60-274.pdf> .
11. Katherine Keisler-Starkey, Lisa N Bunch US (2019) Census Bureau Current Population Reports, P60-271, Health Insurance Coverage in the United States: 2019, U.S. Government Publishing Office, Washington, DC, 2020 <https://www.census.gov/content/dam/Census/library/publications/2020/demo/p60-271.pdf> .
12. OECD/European Union (2020) Health at a Glance: Europe 2020: State of Health in the EU Cycle, OECD Publishing, Paris, https://health.ec.europa.eu/system/files/2020-12/2020_healthatglance_rep_en_0.pdf .
13. PhRMA (2020) Cancer Medicines <https://www.phrma.org/-/media/Project/PhRMA/PhRMA-Org/PhRMA-Org/PDF/A-C/CANCER-CHARTPACK-12-14-2020-for-publicationv2.pdf>
14. PhRMA (2020) Cancer Medicines: Value in Context <https://www.phrma.org/-/media/Project/PhRMA/PhRMA-Org/PhRMA-Org/PDF/A-C/CANCER-CHARTPACK-12-14-2020-for-publicationv2.pdf> .
15. EFPIA (2020) The root cause of unavailability and delay to innovative medicines: Reducing the time before patients have access to innovative medicines. <https://www.efpia.eu/media/554527/root-causes-unavailability-delay-cranal-300620.pdf> .

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