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Research Article

Policy Analysis of Accessibly for Rare and Ultra-Rare Diseases at MENA Region

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Abstract

Objectives: Rare and ultra-rare diseases treatments have their differences form regular treatments for regular diseases for the following:

- Nature of definitions for those diseases
- Treatment protocol
- Targeted paints
- Technology assessment for those treatments
- Funding and financing for reimbursement strategies.

The objectives of the research are to analyze and defines factors affecting accessibly for rare and ultra-rare diseases at MENA region.

Methods: Integration between a systematic literature reviews and analysis for Local Access policies, treatment and technology assessment guidelines. Of rare and ultra-rare diseases Interviews was conducted with Key stake holders for health system in countries as (Egypt, Algeria, Turkey, Jordan, UAE, KSA, Morocco) those stock holders included physicians, clinical pharmacist, Representors of patient's groups, payers, service providers using questionnaire as a survey tool for interview. Public expenditures descriptive analysis was conducted. Plus, financing schemes for those type of diseases.

Results: The following elements of results are playing major roles for garneting accessibly for rare and ultra-rare products at MENA region.

Conclusion: Developing up capacities and capabilities projects to cover the gaps and achieving the following:

- Enhancement of the disease's awareness for different stakeholders.
- Integration between technology assessment and treatment guidelines.
- Patient's advocacy and innovative methods for financing.
- Local platforms to enhance effective communications for different stake holders.

Keywords: *Policy Analysis; Ultra-Rare Diseases; MENA Region; Abdalla Abotaleb*

Introduction and Objectives

Rare and ultra-rare diseases treatments have their differences form regular treatments for regular diseases for the following:

- Nature of definitions for those diseases
- Treatment protocol
- Targeted paints
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- Funding and financing for reimbursement strategies.

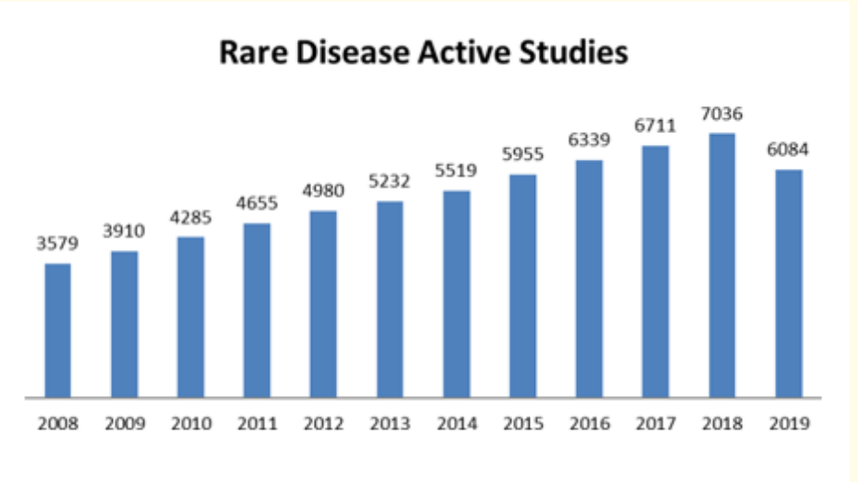
The objectives of the research is to analyze and defines factors affecting accessibly for rare and ultra-rare diseases at MENA region.

Estimated prevalence of orphan disease in the MENA Region.

Country	Population- MN	Estimated Prevalence/ 100,000	Estimated Prevalence- MN
MENA	441	0.675	2.97
Algeria	33.9	0.7	240,000
Saudi	31.5	1	320,000
UAE	9.16	0.6	50,000
Jordan	6.8	0.7	50,000
Tunisia	11.1	0.6	70,000
Lebanon	4.5	0.5	20,000
Egypt	88.4	0.7	620,000
Morocco	33.5	0.6	200,000

Registration, pricing and reimbursement of orphan drugs in the MENA Region*.

COUNTRY	REGISTRATION	PRICING	REIMBURSEMENT PRACTICES	ORPHAN DRUG PURCHASING
KSA	SFDA	SFDA	Different payer level (MoH, National Guard, MODA, etc)	Direct purchasing by institutions LPO
UAE	UAE MoH	UAE MoH	DHA, MoH and HAAD	Direct purchasing by institutions LPO
Egypt	Egyptian Drug Authority (EDA)	CAPA, Pricing & technical committee	Govt: MoH Semi-govt: Ministry of Defense	Direct purchasing by institutions
Jordan	JFDA	JFDA	Upon inclusion on Rational drug list	Direct purchasing by JPM
Lebanon	MoH Drug Registration Tech Committee (DRTC)	MoH DRTC and pricing committee	The National Social Security Fund (NSSF), CPS, Military, MoH	Direct purchasing by institutions level
Algeria	MoH & National Nomenclature Commission Future: NMA	MoH Ministry of Labor Cross-pricing committee	Ministry of Labor, CNAS (National social insurance)	Pharmacie Centrale des Hopitaux (PCH) procures and distribute on patient name basis
Tunisia	MoH DPM (Dept. of Medicine), Tech committee	Central Pharmacy of Tunisia (CPT), DPM	CNAM (Social welfare scheme)	CPT procedures high-cost drugs and distributes on patients' name basis
Morocco	MoH-Department of Drug and Pharmacy (DMP)	Pricing committee	National Agency of Insurance (ANAM), Committee for Economic Financial Evaluation, Transparency commission	Purchasing through national & institutional tenders mainly MoH



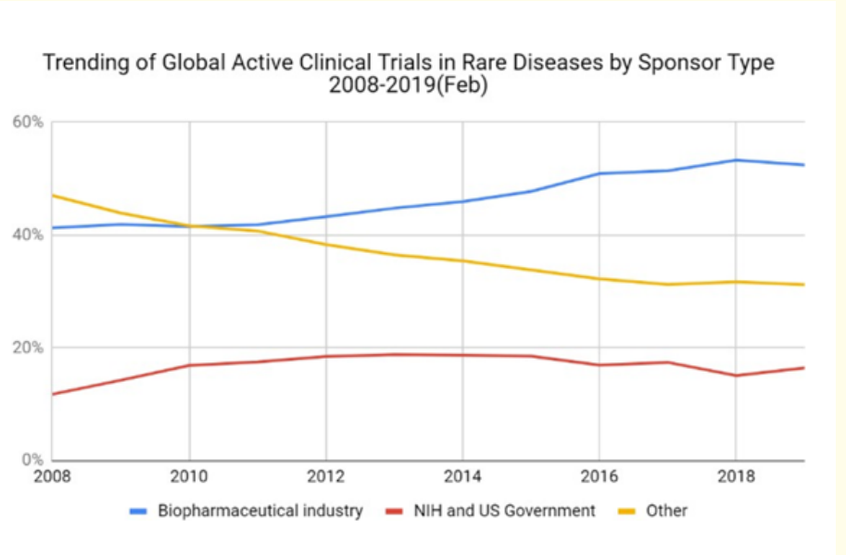


Figure 1

Cumulative number of approved orphan indications and distinct drugs with at least one orphan indication by year of marketing approval.

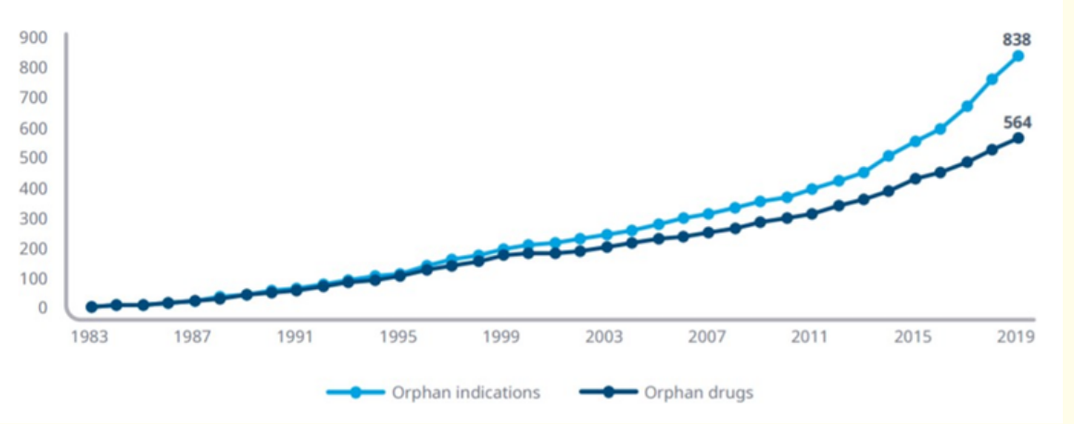


Figure 2

Orphan drug and patients treated by drugs with an orphan indication in 2019 by annual drug cost bands.

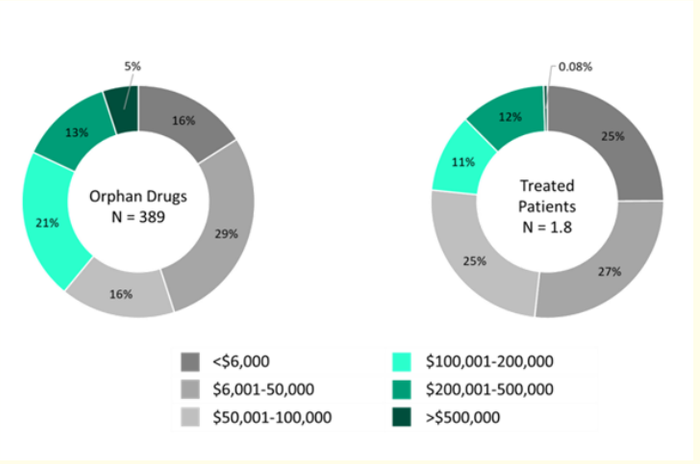


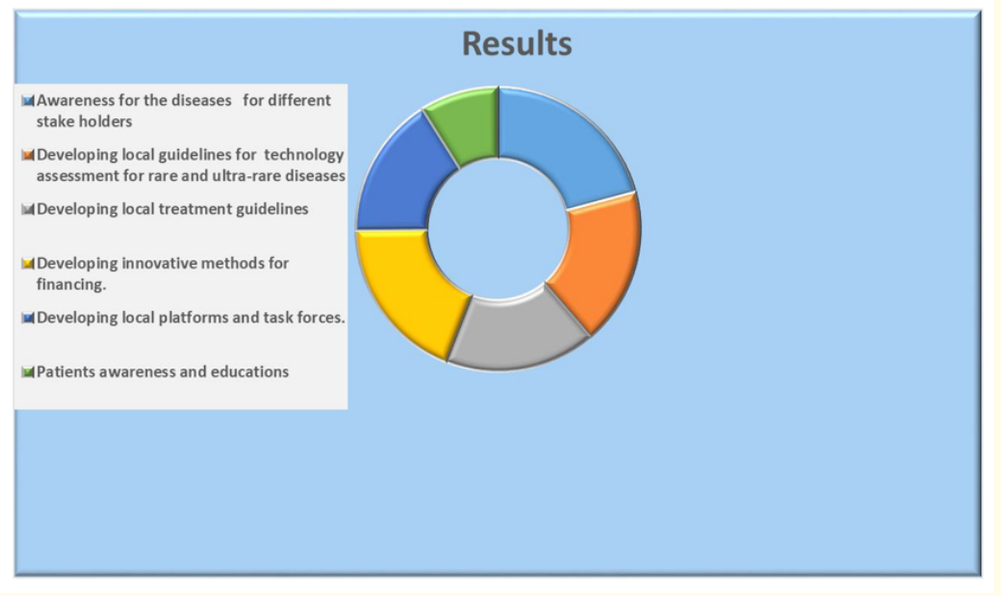
Figure 3

Methods

Integration between a systematic literature reviews and analysis for Local Access policies, treatment and technology assessment guidelines. Of rare and ultra-rare diseases Interviews was conducted with Key stake holders for health system in countries as (Egypt, Algeria, Turkey, Jordan, UAE, KSA, Morocco) those stock holders included physicians, clinical pharmacist, Representors of patient's groups, payers, service providers using questionnaire as a survey tool for interview. Public expenditures descriptive analysis was conducted. Plus, financing schemes for those type of diseases.

Results

The following elements of results are playing major roles for garneting accessibly for rare and ultra-rare products at MENA region.



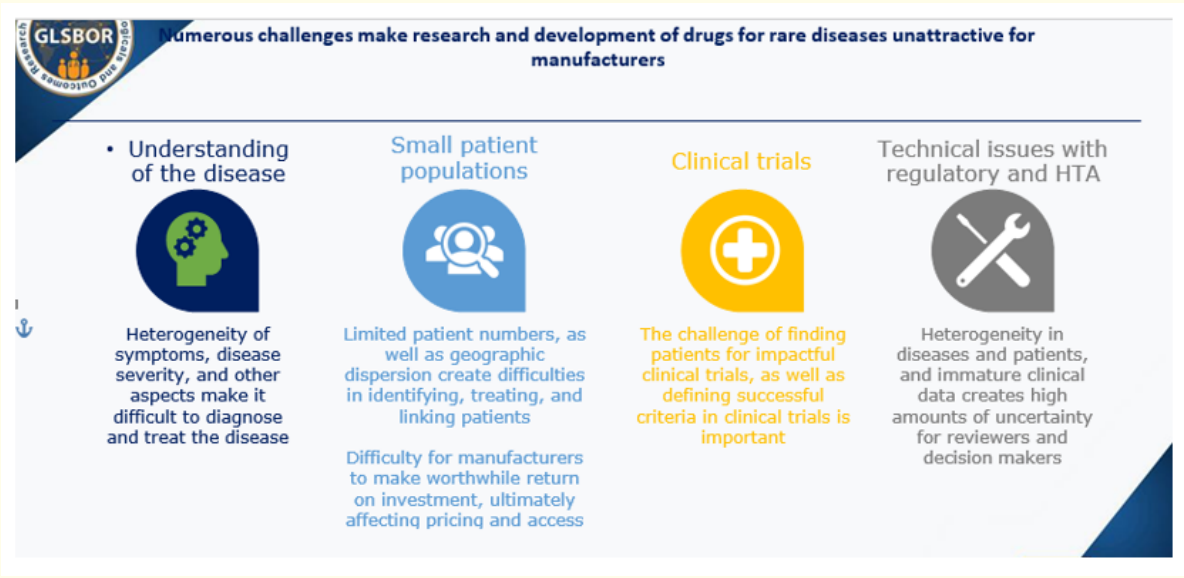


Figure 4

Discussion and Conclusion

Developing up capacities and capabilities projects to cover the gapsand achieving the following:

- Enhancement of the disease's awareness for differentstakeholders.
- Integration between technology assessment and treatmentguidelines.
- Patient's advocacy and innovative methods for financing.
- Local platforms to enhance effective communications fordifferent stake holders.

Recommendations

Accessibility recommendations

Transform health system to accessibility through:

- Developing local essential dug list for rare diseases based on international standards and references
- Enhancement HTA values
- Developing affordability programs

- Developing patient access programs
- Enhancement and reshaping of regulatory pathway
- Enhancement and facilitate clinical research for rare diseases
- Establishing local data bases.

HTA recommendations

Budling up capabilities for local HTA unites through:

- Consideration multi dominations for HTA including (Health problem and nature of different diseases, safety, clinical effectiveness, costs and economic evaluation, social aspect, technical characteristics)
- Training programs for different health care members
- Developing national guidelines based on international standards
- Developing local databases.

Health policy pathway recommendations

- Developing and enhance local treatment guidelines based on international standards (WHO, EMA, FDA).
- Enhancement patient journey through electronic patients' files and referring systems.
- Developing training programs for all medical staff.
- Developing centers of excellence for rare diseases [1-16].

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