Eligibility of Orphan Drugs for Preferential Reimbursement in Egypt

Ahmad Fasseeh[1,2], Baher Elezbawy[1], Nada Korra[1], Mary Gamal[3], Ahmad Seyam[4],Noha Hayek[5], Nagwan Abdel Rahman[6], Sohir Abdelhamid[7], Nader Fasseeh[8], Amr Shafik[9], Ahmed Agamy[3], Akram Mahmoud[6], Amal Samir Sedrak[3,10], Khaled Elshazly[11], Mariam Eldebeiky[3], Monica Talaat[12], Noura Maher Mohamed[4], Rania Ahmed Abdelaziz[4], Remonda Refaat[3], Shaimaa Akeel[3], Randa Adel[13], Samy Khalil[13], Sherif Abaza[14], Zoltán Kaló[15,16]

1. *Syreon Middle East, Alexandria, Egypt*
2. *Eötvös Loránd University, Budapest, Hungary*
3. *Egyptian Authority for Unified Procurement, Medical Supply and Technology Management, Cairo, Egypt*
4. *Universal Health Insurance Authority, Cairo, Egypt*
5. *Theodor Bilharz Research Institute, Giza, Egypt*
6. *Health Insurance Organization, Cairo, Egypt*
7. *Egyptian Parliament Health Sector, Cairo, Egypt*
8. *Faculty of Medicine, Alexandria University, Alexandria, Egypt*
9. *Faculty of Medicine, Ain Shams University, Cairo, Egypt*
10. *Cairo University, Cairo, Egypt*
11. *Presidential Program for Women's Health, Cairo, Egypt*
12. *Egyptian Drug Authority, Cairo, Egypt*
13. *Takeda Pharmaceuticals, Cairo, Egypt*
14. *Syreon Middle East, Cairo, Egypt*
15. *Semmelweis University, Budapest, Hungary*
16. *Syreon Research Institute, Budapest, Hungary*

INTRODUCTION & OBJECTIVE

Despite the recent success in orphan drug development, patient access to orphan drugs remains limited. This might be attributed to the high cost of orphan drugs resulting in denied reimbursement decisions when compared against conventional cost effectiveness thresholds (CETs). The CET framework of Egypt allowed the introduction of a differential threshold to assess orphan drugs. The value of such a threshold is determined by a multiplier of the CET. This study aims to develop a multi-criteria decision analysis (MCDA) tool to determine the multiplier value for each orphan drug and assess its eligibility for preferential reimbursement.

METHODS

To develop the MCDA tool, a scoping review was first conducted to identify relevant criteria. Followed by, a workshop attended by diversified stakeholders from governmental bodies in Egypt. The workshop was held to choose, rank, weigh the possible decision criteria and assign scoring functions for the chosen criteria. The developed tool is then utilized to provide a single score which will be used as the proxy multiplier in the CET framework as shown in figure 1.

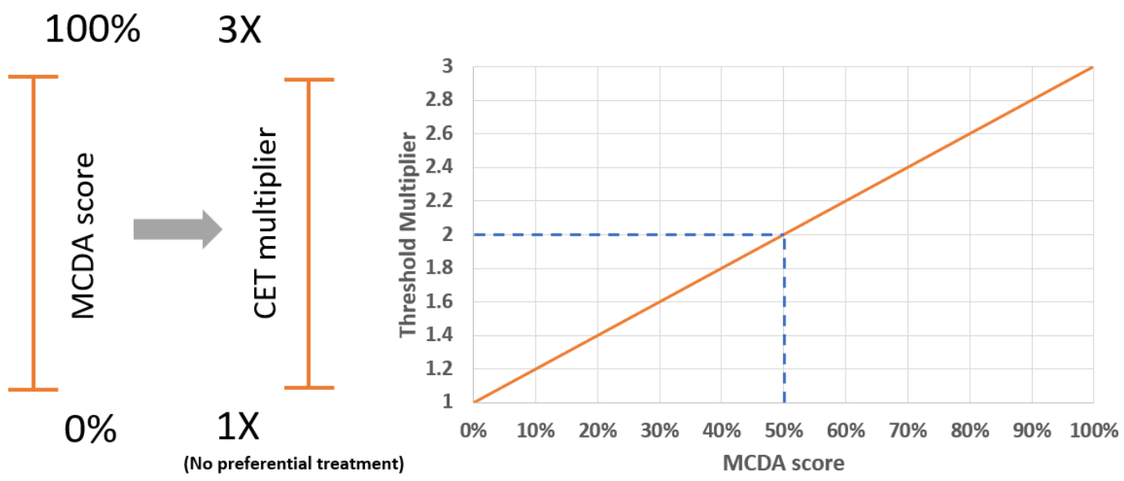

Figure 1: Orphan drug multiplier.

A drug that scores 0% in the MCDA tool is not eligible for any multiplier compared to the conventional CET. A score of 50% allows the drug to be compared to a 2x the conventional threshold, and 100% score allows for a 3 times multiplier.

RESULTS

Scoping review findings

One hundred and fourteen (114) criteria were identified and further consolidated, deduplicated, and refined by excluding non-relevant criteria to yield an initial list of nine criteria to be proposed during the workshop.

Workshop

Ranking

The final list of criteria chosen by the participants included six criteria. Three criteria were excluded by participants due to their negligible impact on the MCDA tool. The final list of criteria ranked according to importance is shown in Table 1.

Weighting

The “severity of the condition” criterion had the highest weight of 38.6%, while the “societal impact of the treatment” criterion came at the end with a weight of 4.6%. Table 1 shows the weights of the included criteria in the MCDA tool.

Table 1: Ranks & weights of the included criteria

Rank	Criterion	Weight
1	Severity of the condition	38.6%
2	Rarity of the disease	24.2%
3	Budget impact	16.1%
4	Credibility and robustness of clinical evidence	10.2%
5	Average age of patients in clinical trials or in real world	6.4%
6	Societal impact of the treatment (indirect cost)	4.6%

Scoring functions

Each criterion had a scoring function that ranges from 0% to 100% depending on the performance of the drug. The following table shows the scoring functions of each of the six included criteria.

Table 2: Scoring functions of the criteria

Criterion	Achieved outcome	Score
Severity of the condition	Chronic life threatening	100%
	Acute life threatening	80%
	Chronic with severe invalidity	60%
	Acute with severe invalidity	40%
	Other chronic diseases	20%
	Other acute diseases	0%
Rarity of the disease	Ultra-rare disease	100%
	Rare disease	75%
	Rare subgroup of a common disease	0%
Budget impact	Below 0.01% of annual drug budget	100%
	Between 0.01-0.05% of annual drug budget	75%
	Between 0.05-0.10% of annual drug budget	50%
	Between 0.1-0.3% of annual drug budget	25%
	Above 0.3% of annual drug budget	0%
Credibility and robustness of clinical evidence	Supportive RCT and real-world evidence	100%
	Supportive RCT with at least 1 year follow-up	75%
	Supportive RCT with <1 year follow-up	50%
	Single arm phase 2 study	0%
Average age of patients in clinical trials or in real world	Pediatrics (0-16 years)	100%
	Young adults (17-30 years)	60%
	Middle aged adults (31-65 years)	30%
	Old age adults (above 65 years)	0%
Societal impact of the treatment (indirect cost)	There is evidence that the societal burden on patients or caregivers is greater than direct medical cost	100%
	There is evidence on significant societal burden on patients or caregivers	75%
	There is no evidence of societal burden	0%

RCT: Randomized Clinical Trial

CONCLUSION

In Egypt, common technologies are assessed for reimbursement according to a CET of 1-3 times the GDP per capita. The developed MCDA tool offers a fair chance for orphan drugs to be reimbursed according to a relevant threshold and acceptable criteria. The MCDA score is used to determine the CET multiplier each drug deserves based on its performance which in turn potentially contributes to better patient access without compromising the budget.

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