A Multi-Stakeholder Multicriteria Decision Analysis in Diabetic Macular Edema. MULTIDEX-EMD Study

Casado MÁ[1], de Andrés-Nogales F[1], Ignacio E[2], Martinez M[3], Ortiz P[4], Peralta G[5], Poveda JL[6], Ruiz Moreno JM[7], Sabater E[1], Trillo JL[8], Zarranz-Ventura J[9], MULTIDEX-EMD Group

[1] Pharmacoeconomics & Outcomes Research Iberia (Porib), Pozuelo de Alarcón, Spain; [2] Universidad de Cádiz, Cádiz, Spain; [3] Ministerio de Sanidad, Consumo y Bienestar Social, Madrid, Spain; [4] Consorci MAR Parc de Salut de Barcelona, Barcelona, Spain; [5] Fundació Rossend Carrasco i Formiguera, Barcelona, Spain; [6] Hospital Universitario y Politécnico La Fe, Valencia, Spain; [7] Hospital Universitario Puerta de Hierro Majadahonda, Majadahonda, Spain; [8] Departamento de Salud Clínico Malvarrosa, Valencia, Spain; [9] Instituto Clinic de Oftalmología, Hospital Clinic, Barcelona, Spain

INTRODUCTION

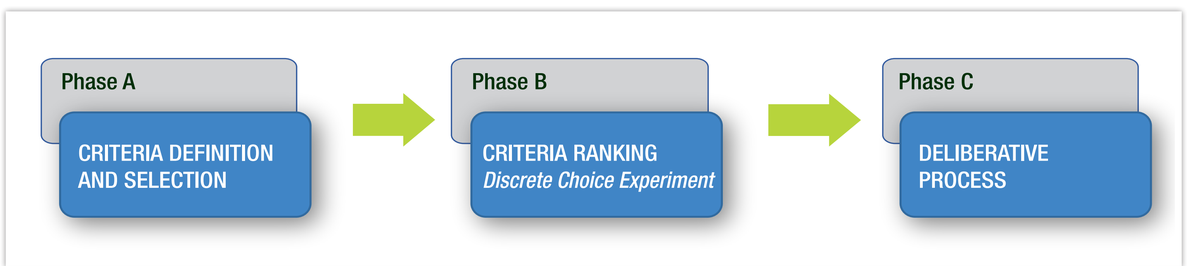
- Diabetic macular edema (DME) is the most common cause of loss of vision and blindness in diabetic patients, with a high and increasing prevalence[1].
- Multicriteria Decision Analysis (MCDA), which has been applied to a broad range of areas in health care, are a set of techniques that provides a rigorous approach for decision making and helps increase the consistency and transparency of these decisions[2,3].
- MCDA offers the potential to overcome the challenges of traditional decision-making tools especially when making complex decisions that include multiple criteria, simultaneously consider quantitative and qualitative data and involve multiple stakeholders[2].

OBJECTIVE

To determine the most relevant criteria in decision-making for the management of diabetic macular edema (DME) from the perspective of several stakeholders from different settings (clinical, pharmaceutical, health authorities, health management, psychological and patient association) in Spain.

METHODS

- A MCDA for the treatment of DME patients was carried out, following the ISPOR MCDA Emerging Good Practice Task Force recommedations[4].
- Twenty stakeholders participated in the project:
  - 7 physicians (6 ophthalmologists and 1 endocrinologist)
  - 4 hospital pharmacists
  - 3 national and regional health authorities
  - 3 health management experts (hospital general manager, medical director, and healthcare quality and management professor)
  - 2 patients
  - 1 clinical psychologist
- The study was developed in three phases:



PHASE A:

- An Advisory Board of 14 of the experts defined all the possible criteria (and the levels/characteristics that defined them) that could influence the decision-making in the treatment of DME patients (Performance Matrix).

PHASE B:

- The previous selected criteria were screened, prioritized and weighted for the treatment of a 50-65 year-old diabetic patient with DME. This analysis was conducted by using a Discrete Choice Experiment (DCE).
- From the Performance Matrix, criteria levels were combined to generate a set of hypothetical DME treatments, which guaranteed enough statistical significance to reveal the preferences of the participants and to establish the relevant criteria for decision-making. The 20 participants received an electronic questionnaire (DCE), where they chose the best option from several pairs of hypothetical treatments.
- A multinomial logit model was fitted to analyse the questionnaire responses applying the backforward algorithm, considering as relevant the criteria with p-values <0.05. The best model to predict the decision-making was estimated after selecting the relevant criteria. In this model, each criteria was weighted based on the choice preference of the participants.

PHASE C:

- Deliberative process with the Advisory Board to discuss the results and conclusions of the DCE.

RESULTS

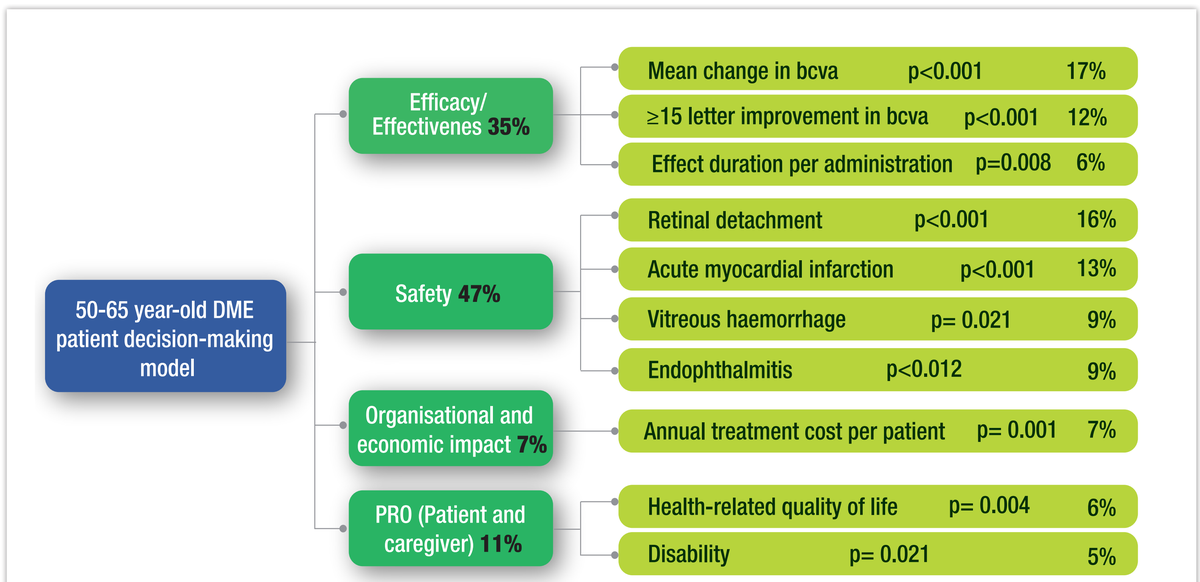
- A total of 31 criteria were initially defined in phase A (Table 1) and classified into several categories (for presentation purpose only).
- From the combination of levels from the 31 selected criteria, a set of 120 pairs of hypothetical treatments were obtained following an orthogonal design.
- The DCE results (phase B) established 10 out of 31 criteria as relevant in decision-making for a 50-65 year old diabetic patient with DME (Figure 1).
- Safety criteria had the greatest weight in the decision (47%), followed by efficacy/effectiveness (35%).
- The most relevant criteria for the decision-making in the treatment of DME patients were mean change in BVCA (17%) and the presence of adverse events such as retinal detachment (16%) or acute myocardial infarction (13%).

Table 1. Selected criteria and levels for decision-making in DME

	CRITERIA	LEVELS
EFFICACY/ EFFECTIVENESS	Mean change in BCVA	0-5 letters // 6-10 letters // 11-15 letters // >15 letters
	≥15 letter improvement in BCVA	0-15% patients // 16-30% patients // >30% patients
	Reduction in central retinal thickness	≤20% reduction // >20% reduction
	Speed of action: visual acuity improvement	<1 month // 1-3 months // >3 months
	Effect duration per administration	≤1 month // > 1-4 months // >4-12 months // >12 months
	Response in prior treatment refractory patients	After change of treatment due to lack of response: Response is maintained // Response is improved // Response is reduced
	Reduction in the need of long-term treatment (3 years)	Yes // No
SAFETY	Ocular adverse events: increased intraocular pressure	Occurrence: controlled with medical treatment // Ocurrence: controlled with surgical treatment // Non-ocurrence
	Ocular adverse events: endophthalmitis	Occurrence // Non-occurrence
	Ocular adverse events: retinal detachment	Occurrence // Non-occurrence
	Ocular adverse events: vitreous haemorrhage	Occurrence // Non-occurrence
	Ocular adverse events: cataract	Occurrence // Non-occurrence // Progression
	Systemic adverse events: acute myocardial infarction	Occurrence // Non-occurrence
	Systemic adverse events: cerebrovascular accident	Occurrence // Non-occurrence
	Immunogenicity	Occurrence // Non-occurrence
ORGANISATIONAL AND ECONOMIC IMPACT	Budget impact	Positive (increase of incremental costs) // Neutral // Negative (decrement of incremental costs)
	Annual pharmaceutical cost per patient	< €500 // €500-1500 // > €1500-3000 // > €3000
	Number of intravitreal injections (first year)	≤3 // >3
	Minimum required facilities	Clean room // Surgery room
	Healthcare burden	The treatment implies an increase in the healthcare burden // The treatment does not modify the healthcare burden // The treatment implies a reduction in the healthcare burden
	Need of Pharmacy handling	Pharmacy handling // No Pharmacy handling
PRO (PATIENT & CAREGIVER)	Disability	Improvement of functional capacity and performance of activities of daily living // No effect in functional capacity and performance of activities of daily living // Worsening of functional capacity and performance of activities of daily living
	Quality of life	Improvement of quality of life (social/occupational) // No effect in quality of life (social/occupational) // Worsening of quality of life (social/occupational)
	Affectation of emotional state	Anxiety and depression treated pharmacologically // Anxiety and depression treated non-pharmacologically //No anxiety or depression
	Treatment satisfaction	Improvement // No effect // Worsening
	Caregiver burden	No increase of the caregiver burden // Moderate increase of the caregiver burden* // High increase of the caregiver burden**
PERSISTENCE AND OTHERS	Treatment persistence	Persistent patient // Non-persistent patient
	Pharmaceutical form	Modified- or delayed-release // No modified release
	Available presentations	Vial // Syringe/Injector // Vial and Syringe/injector
	Therapeutic innovation: new mechanism of action	Yes // No
	Therapeutic innovation: new therapeutic target	Yes // No

*it requires the caregiver to occasionally accompany the patient to the treatment-related visits
**it requires the caregiver to frequently or continuously accompany the patient to the treatment-related visits

Figure 1. Relevant criteria for decision-making in DME



BCVA: best-corrected visual acuity, PRO: patient-reported outcomes

CONCLUSIONS

- From a multi-stakeholder perspective and considering the revealed preferences of the participants:
  - The selection of an appropriate treatment for DME patients should guarantee the patient safety while maximizing the improvements in visual acuity with the longest treatment effect.
  - Furthermore, it should contribute to the system sustainability with an affordable treatment cost.
  - Finally, it should assure a positive impact in health-related quality of life and prevent from disability.

REFERENCES

1. Romero-Aroca P, et al. Br J Ophthalmol. 2016;100(10):1366-71.
2. Adunlin G, et al. Health Expect. 2015;18(6):1894-905.
3. Thokala P, et al. Value Health. 2016;19(1):1-13.
4. Marsh K, et al.; ISPOR Task Force. Value Health. 2016;19(2):125-37.

MULTIDEX-EMD Group

- Pedro Acosta Robles (Agencia Pública Sanitaria Poniente, Almería, Spain)
- Eloísa Álvarez Giménez (Hospital Clínico San Carlos, Madrid, Spain)
- Miguel Ángel Casado Gómez (Pharmacoeconomics & Outcomes Research Iberia-PORIB, Pozuelo de Alarcón, Spain)
- Enrique Cervera Taulet (Hospital Universitari i Politècnic La Fe, Valencia, Spain)
- Fernando de Andrés Nogales (Pharmacoeconomics & Outcomes Research Iberia-PORIB, Pozuelo de Alarcón, Spain)
- Alfredo García Layana (Clínica Universidad de Navarra, Pamplona, Spain)
- Emilio Ignacio García (Universidad de Cádiz, Cádiz, Spain)
- Iñaki Llorente Gómez (Hospital Universitario Nuestra Señora de la Candelaria, Santa Cruz de Tenerife, Spain)
- José Martínez Olmos (Comisión de Sanidad, Senado de España)
- José Manuel Martínez Sesmero (Hospital Clínico San Carlos, Madrid, Spain)
- Mercedes Martínez Vallejo (Ministerio de Sanidad, Consumo y Bienestar Social, Madrid, Spain)
- José Mayorga Fabián (Asociación Mácula-Retina, Sevilla, Spain)
- Carlos Mur de Viu (Hospital Universitario de Fuenlabrada, Fuenlabrada, Spain)
- Pere Ortiz Sagrista (Parc de Salut Mar, Barcelona, Spain)
- Gemma Peralta Pérez (Fundació Rossend Carrasco i Formiguera. MentBarcelona, Barcelona, Spain)
- José Luis Poveda Andrés (Hospital Universitari i Politècnic La Fe, Valencia, Spain)
- Mariano Rodríguez Maqueda (Hospital Universitario Virgen del Rocío, Sevilla, Spain)
- José María Ruiz Moreno (Hospital Universitario Puerta de Hierro Majadahonda, Majadahonda, Spain)
- Eliazar Sabater Cabrera (Pharmacoeconomics & Outcomes Research Iberia-PORIB, Pozuelo de Alarcón, Spain)
- José Luis Trillo Mata (Departamento de Salud Clínico Malvarrosa, Valencia, Spain)
- Patricia Udaondo Mirete (Hospital Universitari i Politècnic La Fe, Valencia, Spain)
- Javier Zarranz Ventura (Instituto Clinic de Oftalmología, Hospital Clinic, Barcelona, Spain)
- Jacinto Zulueta Menchaca (Asociación Mácula Retina, Sevilla, Spain)

Designed by [porib] pharmacoeconomics & outcomes research iberia

Presented at ISPOR Europe 2018 10-14 November 2018 | Barcelona, Spain

This work was carried out with an unrestricted grant from Allergan.