



**AIM**

Healthcare and  
social benefits  
for all

# Defining unmet medical need

EMA Payers Community Meeting

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# Unmet Medical Need according to EC

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Article 4 paragraph 2 of Commission Regulation (EC) No. 507/2006 (about conditional marketing authorization):

*“Unmet medical needs means a condition for which there exists no satisfactory method of diagnosis, prevention or treatment in the Union or, even if such a method exists, in relation to which the medicinal product concerned will be of major therapeutic advantage to those affected”*

**Very general, not enough guidance.**

# Case study : Belgium – used definitions

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- Chronically or seriously debilitating diseases or diseases considered to be life threatening and that cannot be treated satisfactorily by **an authorised medicinal product** (Belgian Federal Agency of Medicines and Health Products for **compassionate use and medical needs programs**)
- Pharmaceutical product for the treatment of a severe or life threatening condition for which **no reimbursed alternative treatment** is available (law of 7 February 2014 and the Royal Decree of 12 May 2014 used by NIHDI for **reimbursement in Unmet Medical Need Procedure - UMNP**)

# UMNP : Prioritization - MCDA (1)

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## Therapeutic need:

- Impact of disease on quality of life, given current treatment
- Impact of disease on life expectancy, given current treatment
- Inconvenience of current treatment

## Societal need:

- Disease-related public expenditures
- Frequency of the disease (prevalence or incidence)

**Additional criteria** (after the MCDA results have been produced) are:

- Psychosocial well-being / patient frailty/impact on carer's quality of life/autonomy

Source: [https://kce.fgov.be/sites/default/files/atoms/files/KCE\\_272\\_Unmet\\_needs\\_Report2.pdf](https://kce.fgov.be/sites/default/files/atoms/files/KCE_272_Unmet_needs_Report2.pdf)

# UMNP : Prioritization - MCDA (2)

Involvement of citizens in :

- defining criteria
- **weights of criteria**

Therapeutic need	Weight
• Impact of the condition on quality of life, given current treatments available	0.43
• Impact of the condition on life expectancy, given current treatments available	0.14
• Inconvenience of current treatment	0.43
Societal need	Weight
• Condition-related public expenditures per patient	0.65
• Frequency of the condition (prevalence or incidence)	0.35

Source: Cleemput et al. 2014<sup>4</sup>

Role of commission (CAIT):

- analyzing evidence
- scoring
- ranking

Source: [https://kce.fgov.be/sites/default/files/atoms/files/KCE\\_272\\_Unmet\\_needs\\_Report2.pdf](https://kce.fgov.be/sites/default/files/atoms/files/KCE_272_Unmet_needs_Report2.pdf)

# UMNP : limitative list

List unmet medical needs 2019 (Top 10)	Priority indicator
Amyotrophic Lateral Sclerosis (ALS)	11,34
Recurrent glioblastoma	10,58
Pancreatic cancer	10,56
Neuronal ceroid lipofuscinosis	10,40
Malignant pleural mesothelioma	10,34
Mesothelioma	10,22
Duchenne muscular dystrophy	10,02
Early and selective wound care for deep burns in children	9,86
Acute lymphoblastic leukemia	9,56
First line treatment for metastatic Non Small Cell Lung Cancer) with high, low or no expression of PD-L1	9,49

**Driven by what companies have in the pipeline**

# Payers: discussion (1)

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## Why do we need a definition?

- To make it clear for all stakeholders (benefits for patients/payers/industry)
- To help using limited HC means according to real needs
- To orientate the funds given to research towards real needs

## What is it used for today (and should not be anymore)?

- An instrument to market medicines in an immature stage (limited evidence => benefit unsure)
- A reason for exuberant pricing



# Payers : discussion (2)

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A definition of UMN should also include notions about :

- severity (seriously debilitating or life-threatening condition)
- impact on Quality of life
- burden of illness
- a guarantee of safety for the patient (?)

**Who should define unmet medical needs?**

- Societies with an involvement of patients and their representatives (demand driven) and citizens

**There are so many unmet medical needs!**

- How to prioritize?

**Setting a limit**

- Answering unmet need can not threaten accessibility to other therapies