Unmet medical need

An industry perspective

Rick Vreman, patient access & policy lead, Roche

Disclaimer

- I work for Roche. No other conflicts of interest apply.

- The views in this presentation are my own and do not necessarily reflect the positions of the organization(s) I am affiliated with

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UMN: what is it?

UMN is intended as a prioritization mechanism

- In EC proposal: YES or NO

- CMA
- PRIME
- RDP

Article 83 (Directive)

Medicinal products addressing an **unmet medical need**

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   a) there is no medicinal product authorised in the Union for such disease, or, where despite medicinal products being authorised for such disease in the Union, the disease is associated with a remaining high morbidity or mortality;
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Lijst van onbeantwoorde medische behoeften 2024	Prioriteitsindicator
Nieuw gediagnosticeerde glioblastoom	11,40
Amyotrofe Lateraal Sclerose (ALS)	11,34
Acute graft versus hostziekte (aGVHZ)	11,25
Recidiverend glioblastoom	10,58
Zure sfingomyelinasedeficiëntie	10,54
Metachromatische leukodystrofie	10,40
Neuronale ceroïdlipofuscinose type 2	10,40
Amyloïde lichte keten (AL) amyloïdose	10,14
Duchenne spierdystrofie	10,02
≥2e lijnsbehandeling van HER2-positief vergevorderde maagkanker of gastro-oesofageale junctie kanker na progressie op trastuzumab	9,96
Cholestase ten gevolge van Biliaire Atresie (BA)	9,93
Vroegtijdig en selectieve wondverzorging diepe brandwonden bij kinderen	9,86
Lokaal gevorderd of gemetastaseerd plaveiselcel anaal carcinoom na progressie op of intolerantie voor platinum- gebaseerde chemotherapie	9,84
Voorbehandeld Epstein barr virus positief post-transplantatie lymfoproliferatieve ziekte (EBV-PTLD)	9,50
≥3e lijnsbehandeling van HER2-positief gemetastaseerde borstkanker (HER2+ mBC) na falen op trastuzumab emtansine (T-DM1)	9,37
Vergevorderd gededifferentieerd liposarcoom	9,35
Fibrodysplasia ossificans progressiva (FOP)	9,28
HR +, HER2 - borstkanker (stadium IV) met een PIK3CA mutatie	9,2
Chronische graft versus hostziekte (cGVHZ)	9,2
Niet-reseceerbaar of gemetastaseerd lokaal gevorderd adenocarcinoom van de maag of de gastro-oesofageale overgang die richtbare biomarkers tot expressie brengt	9,15
MET-gedreven niet-resecteerbaar en lokaal gevorderd of gemetastaseerd papillaire niercelcarcinoom	9,09
Alfa-Mannosidose	9,06
Neurofibromatose type 1 (NF1)	8,94
Recidiverend of refractair (R/R) folliculair lymfoom (FL) dat ten minste twee eerdere systemische therapieën heeft ondergaan (3L+)	8,83
Gemetastaseerde niet-kleincellige longkanker die reeds met immunotherapie behandeld werd (2L+)	8,8
Progressieve supranucleaire paralyse (PSP)	8,8
Kleincellige longkanker (Small Cell Lung Cancer)	8,8
De ziekte van Huntington	8,78
Neuromyelitis optica spectrum disorder (NMOSD)	8,75
Non-GCB diffuus grootcellig B-cel lymfoom	8,74
Systemische sclerodermie en interstitiële longziekte (longfibrose)	8,60
Osteogenesis imperfecta	8,52
Pneumonie bij geïntubeerde en mechanisch geventileerde patiënten	8,51
Transfusie afhankelijke β-Thalassemie	8,5
Alagille Syndroom (ALGS) met pruritus en/of cholestase	8,48
Eerstelijnsbehandeling van gemetastaseerde urotheliale blaaskanker	8,37
Niet-kleincellige longkanker	8,32
Transplantatieontvangers met CMV-infectie of -ziekte, waaronder refractaire/resistente (R/R) CMV	8,28
PIK3CA-gerelateerd overgroeisyndroom (PROS)	8,23
Gemetastaseerd colorectaal carcinoom	8,03
Gevorderd plaveiselcelcarcinoom (aCSCC)	8,0
Gevorderde systemische Mastocytose (ASM)	8,0
C3-Glomerulopathie (C3G)	7,83
Sikkelcelziekte	7,8
Onvoldoende gecontroleerd carcinoïdsyndroom	7,77
Focale segmentale glomerulosclerose	7,73
Lysosomale zure lipase deficiëntie	7,62
Endogeen syndroom van Cushing	7,6
Gemetastaseerde borstkanker (mBC) met lage expressie van de HER2 receptor (HER2-low), na het falen van minimaal 1 eerdere lijn chemotherapie	7,58
Lupus nephritis	7,52
Gegeneraliseerde pustuleuze psoriasis (psoriasis pustulosa generalisata)	7,52
Gevorderd niercelcarcinoom (RCC) L2	7,33
Respiratoir syncytieel virus (RSV) infecties bij kinderen.	7,28
Sporadic inclusion-body myositis (sIBM)	7,22
Myelofibrose	7,18
Familiaal chylomicronemiesyndroom	7,12
Anti-neutrofiel cytoplasmatische auto-antilichamen (ANCA) geassocieerde vasculitis (AAV)	6,98
Trombotische trombocytopenische purpura	6,87
Alzheimer	6,78
Adjuvante behandeling bij patiënten met gBRCAm, hoog-risico HER2-negatieve borstkanker in een vroeg stadium (TNM I-III)	6,72
Reusceltumor van de weke delen	6,57
Hartfalen met behouden ejectiefractie (HFpEF)	6,52
IgA Nefropathie	6,48

https://www.riziv.fgov.be/

What do patients think?

EPF proposal for a patient-centred framework for defining Unmet Medical Needs

October 2023

- "The term "unmet medical need" itself seems inappropriate, when in fact "unmet patients' needs" – defined by and with patients – should be the main driver of pharmaceutical R&D."

- ***EPF proposal, unmet medical needs should be defined by:***
- Apply to medicinal products intended to treat, but also medicinal products intended to prevent or diagnose a disease […]
- Address patients' immediate therapeutic needs, defined as needs perceived by the patients that are not met by currently available treatments, and longer-term societal needs, including impacts on carers and future public health threats.
- Be inclusive – many lifelong, chronic diseases are not or no longer considered as "life-threatening" but still represent a significant burden on patients, carers, and healthcare systems. […]
- +Specific criteria for assessing patients' therapeutic needs

EPF proposal for a patient-centred framework for defining Unmet Medical Needs

October 2023

- ***Specifically, when assessing patients' therapeutic needs, the following criteria should be considered:***
- Impact of the condition on life expectancy after providing standard of care treatment
- Appropriateness of the current standard of care for the patient, including considerations of:
  - Impact of the new product on one or several serious outcome(s) of the condition that is/are not or insufficiently addressed by the current standard of care
  - Impact of the new product on disease onset, progression, and duration, as well as disease severity, compared with the current standard of care
  - Impact of the new product on a specific patient subpopulation who fails to tolerate or to respond to the current standard of care
  - Inconvenience of current standard of care and major improvements to patient care, including when the new product addresses treatment compliance issues, leading in turn to significantly improved outcomes
  - Lack of availability of the current standard of care in one or several EU member states. […]
- Impact of the condition on patients as it relates to their experience and everyday lives after receiving standard of care treatment. This includes considerations of quality of life, recognising the multifactorial nature and current evolutions of the concept (e.g., mental health impacts)

How does it relate?

Article 83 (Directive)

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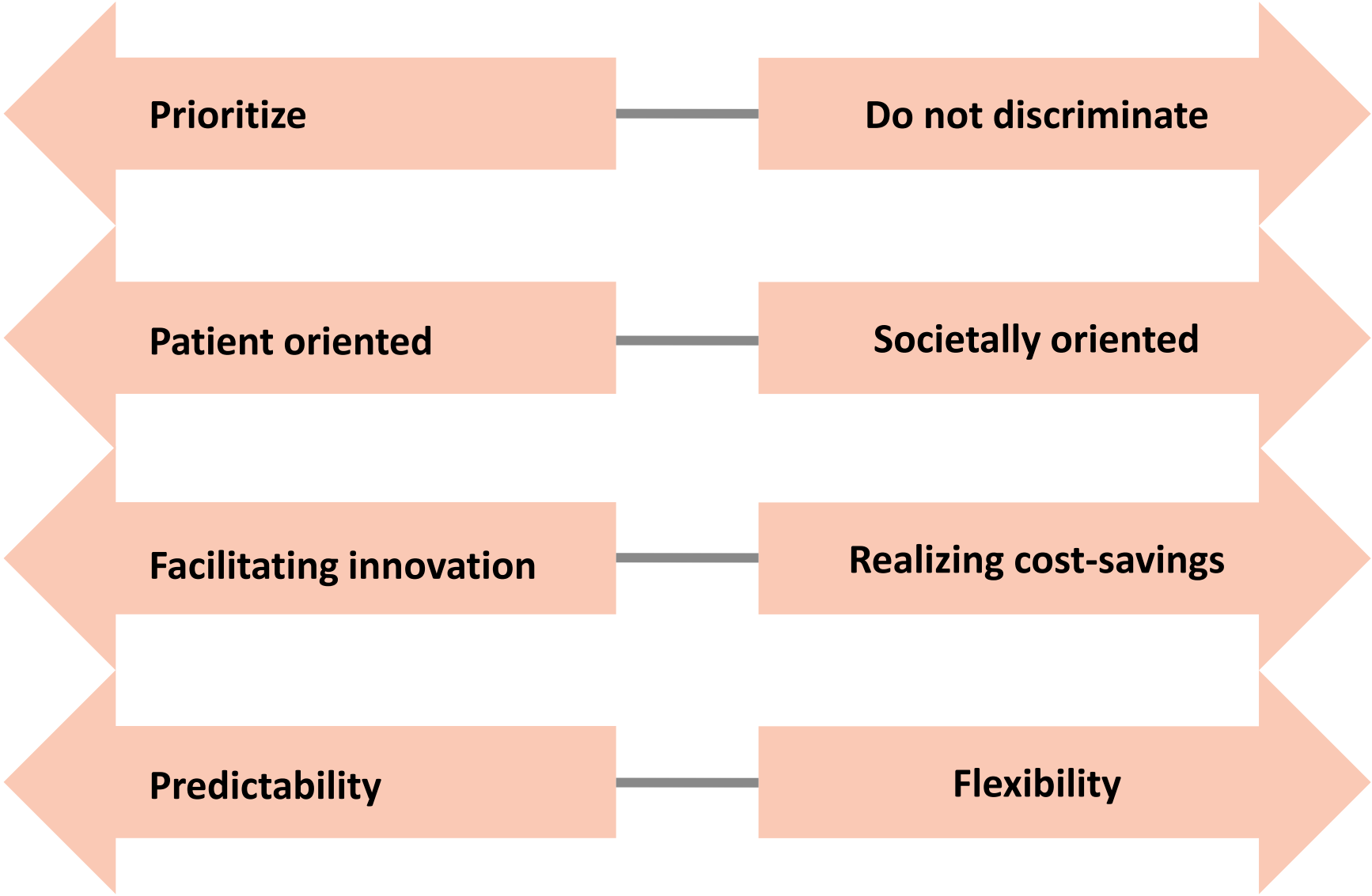
What are the principles?

What are the underlying (sometimes conflicting) principles?



How about incentives?

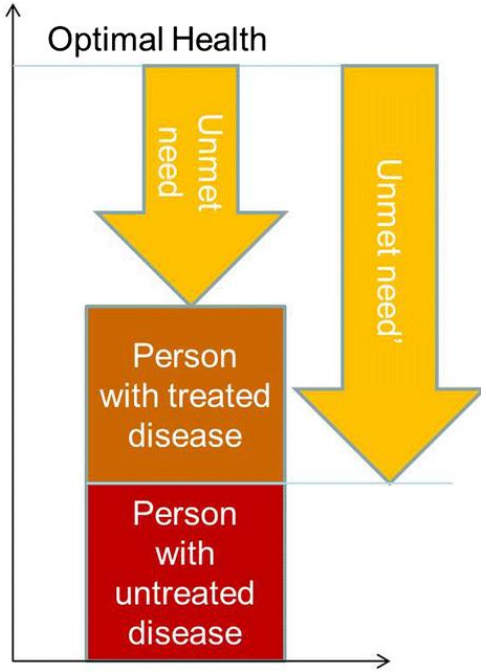
- In the EC proposal:
  - Eligibility CMA
  - Eligibility for PRIME
  - RDP +6mo

  Note:
  - No use of market exclusivity (as for orphan medicinal products 10y)

  - The EC/EU has no remit to direct pricing and reimbursement decisions (individual country mandate)

Industry perspective

Unmet Medical Need: It all starts with patients and science



- Lack of adequate authorised prevention, treatment or cure
- Need is evolving and is not a binary

Availability of
- Scientific clues
- Technology
- Data
- Infrastructure
- Collaboration

- **Incentives should be fit-for-purpose and predictable**

How does it relate?

- Fit-for-purpose
- Non-discriminatory
- Predictable

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EFPIA recommendations

1. A patient-centric definition inclusive of different perspectives
   - No discrimination of patient groups (positive incentive)
   - Processes should include all stakeholders, especially patients
   - A broad understanding of UMN, giving room for more stakeholder perspectives (patient, physician, health system, society)

https://www.efpia.eu/about-medicines/development-of-medicines/unmet-medical-need/. Addressing unmet medical need. October 2023

EFPIA recommendations

2. UMN as a means to incentivize should reflect the reality of science and R&D
   - R&D is usually in waves, incremental innovation should be incentivized
   - Incentive framework should be predictable
   - Incentives should be meaningful and not be counteracting good R&D

https://www.efpia.eu/about-medicines/development-of-medicines/unmet-medical-need/. Addressing unmet medical need. October 2023

EFPIA recommendations

3. The EU should be an innovation leader, while respecting member state competencies
   - An EU-level UMN definition is not an appropriate tool to fix access & affordability concerns (UMN definition should maintain the distinction between EU and member state remits)
   - A restrictive UMN definition combined with shortened RDP for all products, erodes Europe's competitiveness

https://www.efpia.eu/about-medicines/development-of-medicines/unmet-medical-need/. Addressing unmet medical need. October 2023

EFPIA Key recommendations

Taking the evolution of science and patient needs as a starting point, we understand an UMN as a condition that is not adequately prevented, treated or diagnosed by authorized interventions