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Federaal Kenniscentrum voor de Gezondheidszorg
Centre Fédéral d'Expertise des Soins de Santé
Belgian Health Care Knowledge Centre

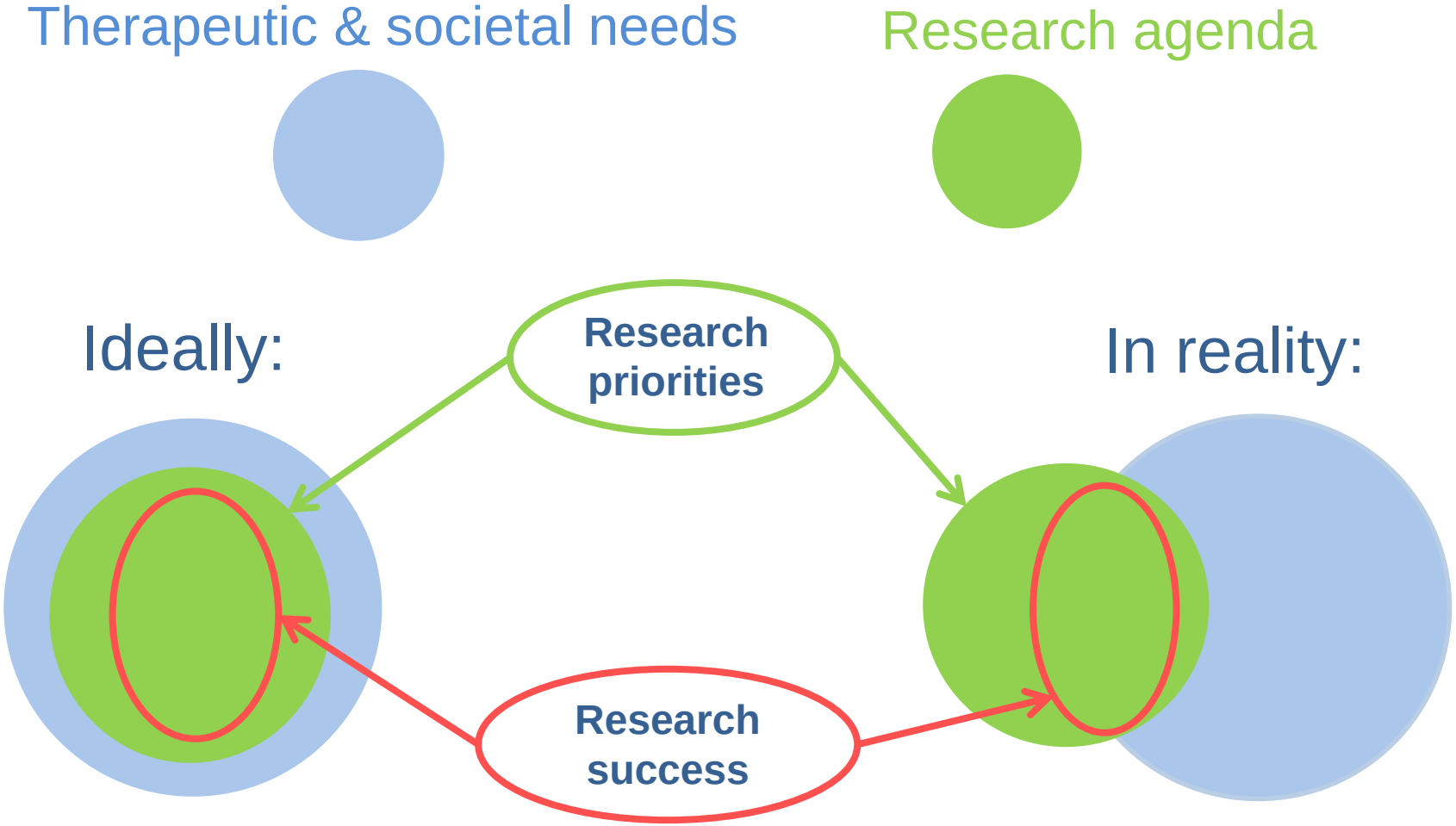
# Prioritisation of medical needs and applicability for trial selection in Belgium

Rene Westhovens, Rheumatologist, Emeritus Prof KU Leuven, Belgium.
President Orphan Drug Colleges and Advisory Commission Unmet Medical Need at RIZIV/INAMI

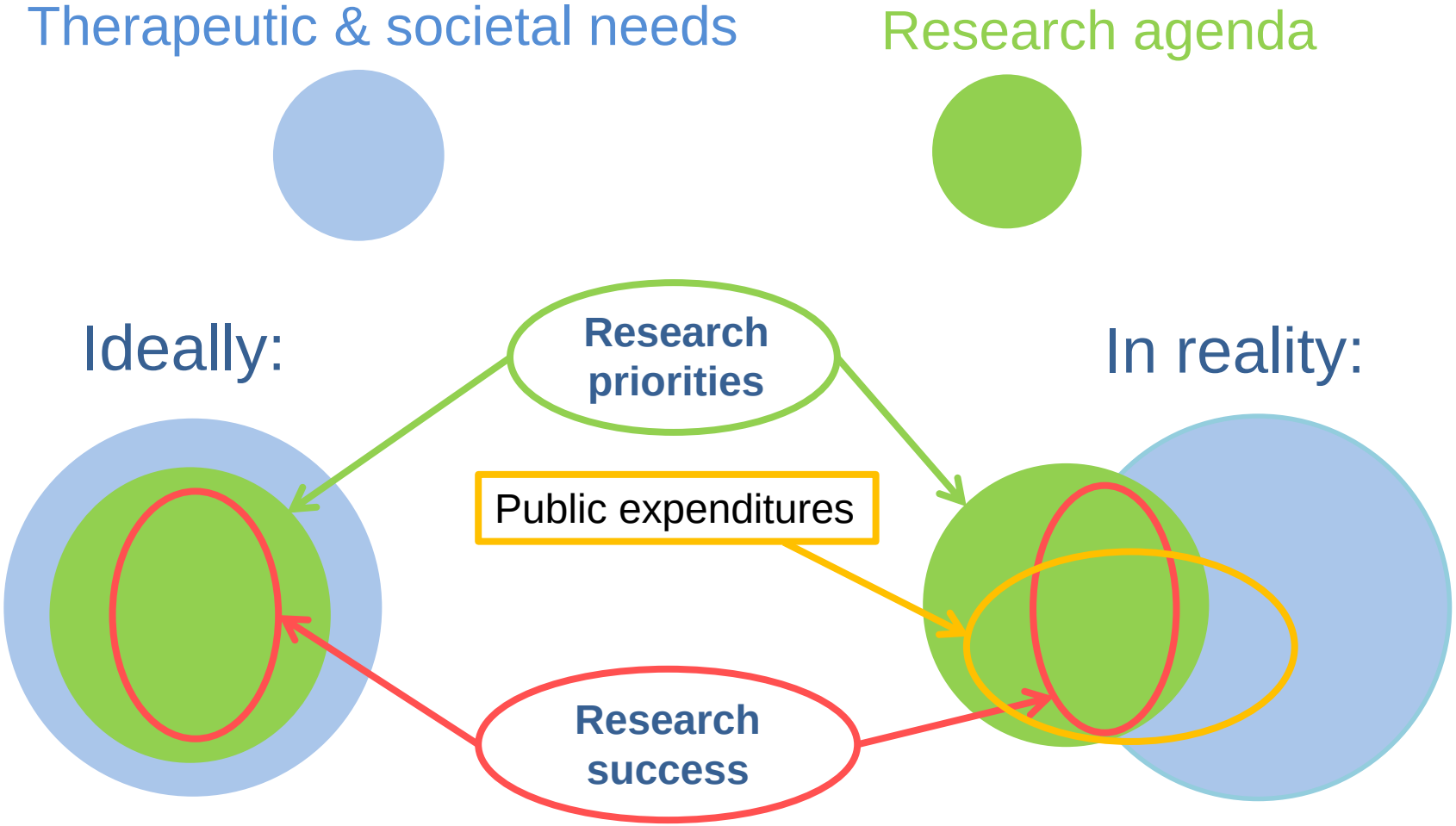
Irina Cleemput, Senior Health Economist, KCE

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# What is the issue?



# What are we paying for?



# How to solve the issue

**Step 1: Identification of unmet needs**

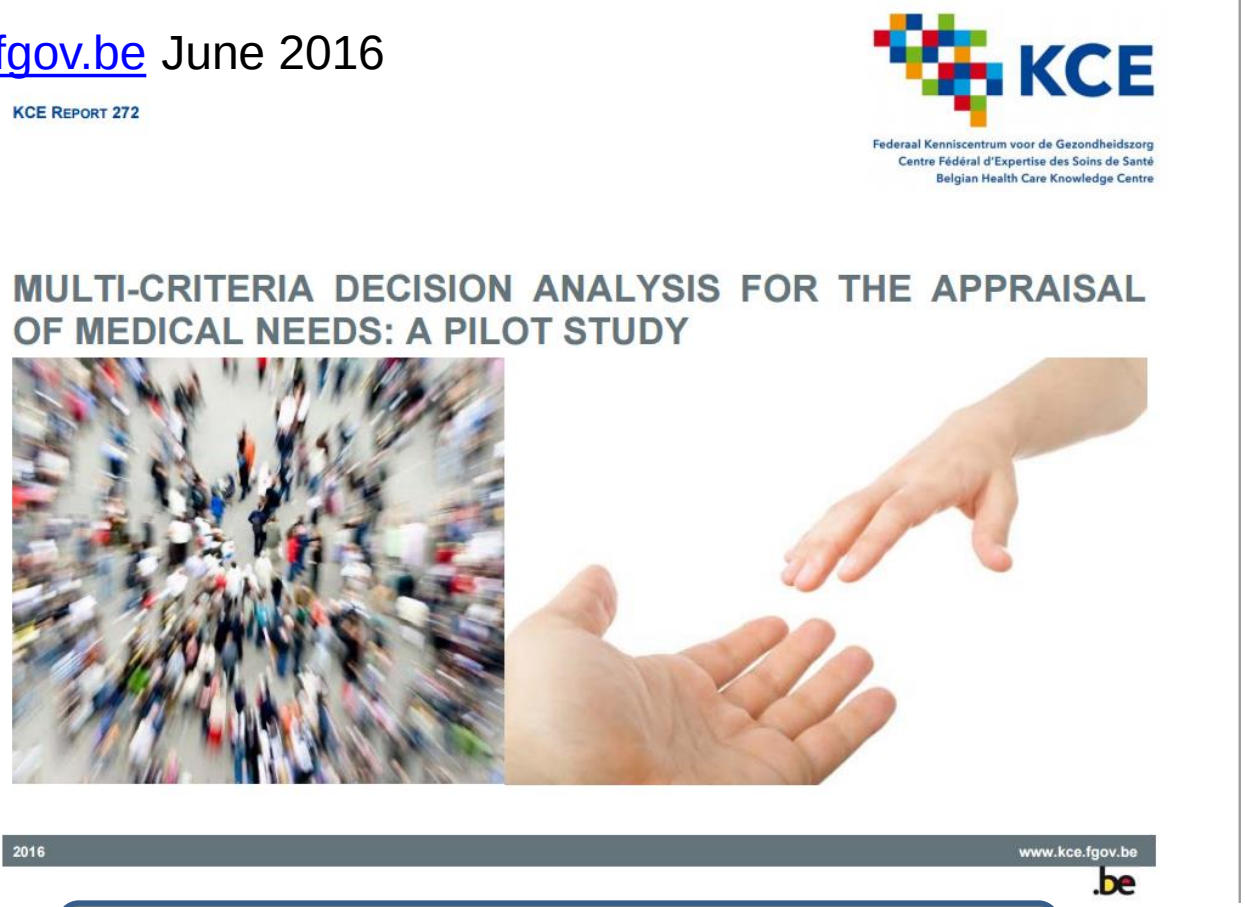
**Step 2: Prioritization**

**Step 3: Communication**

**Step 4: Support, e.g. early dialogues, public funding**

# How to prioritize needs?

www.kce.fgov.be June 2016




Cleemput I et al
Health Policy. 2018 Aug;122(8):878-884.

# Therapeutic vs societal need

## Therapeutic need

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

## Societal need

- Disease-related public expenditure
- Prevalence

*given current treatment*

# Weights of “needs” criteria

## Therapeutic need

- **Inconvenience** of current treatment — 0,43
- **Quality of life** with current treatment — 0,43
- **Life expectancy** with current treatment — 0,14

## Societal need

- **Prevalence** — 0,35
- **Disease-related societal cost** — 0,65

# How to apply the method?







Definition of the condition

Evidence assessment

Therapeutic need

Quality of Life

Life expectancy

Inconvenience curr treatment

Societal need

Public expenditures

Disease frequency

Appraisal committee

Score

Score

Score

Score

Score

Public / committee

Weight

Weight

Weight

Weight

Weight

Weighted score

Weighted score

Weighted score

Weighted score

Weighted score

Total weighted score

Total weighted score

Ranking Therapeutic need

Ranking Societal need

KBF Criteria

KBF Criteria

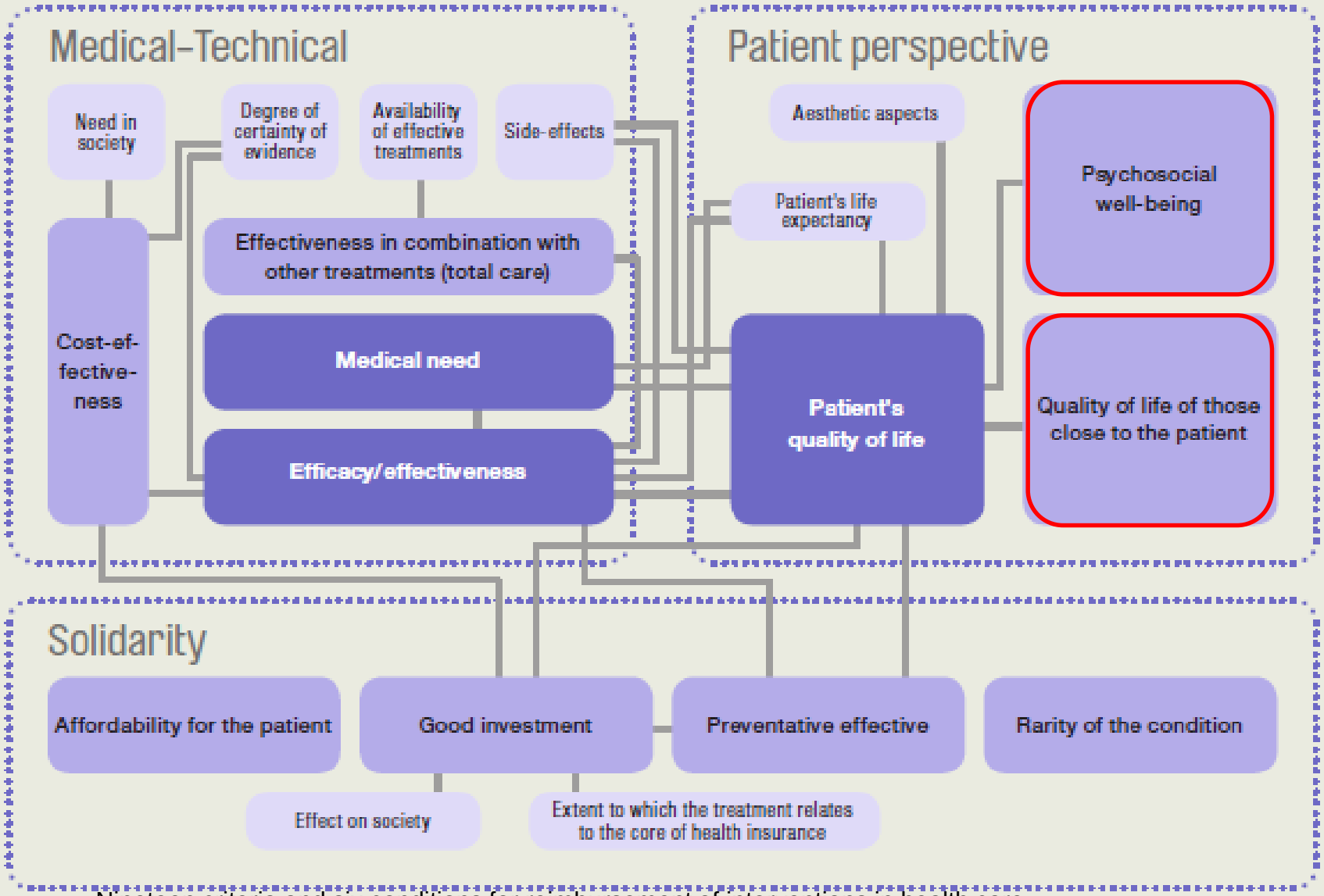
Final ranking Therapeutic need

Final ranking Societal need

Patient input

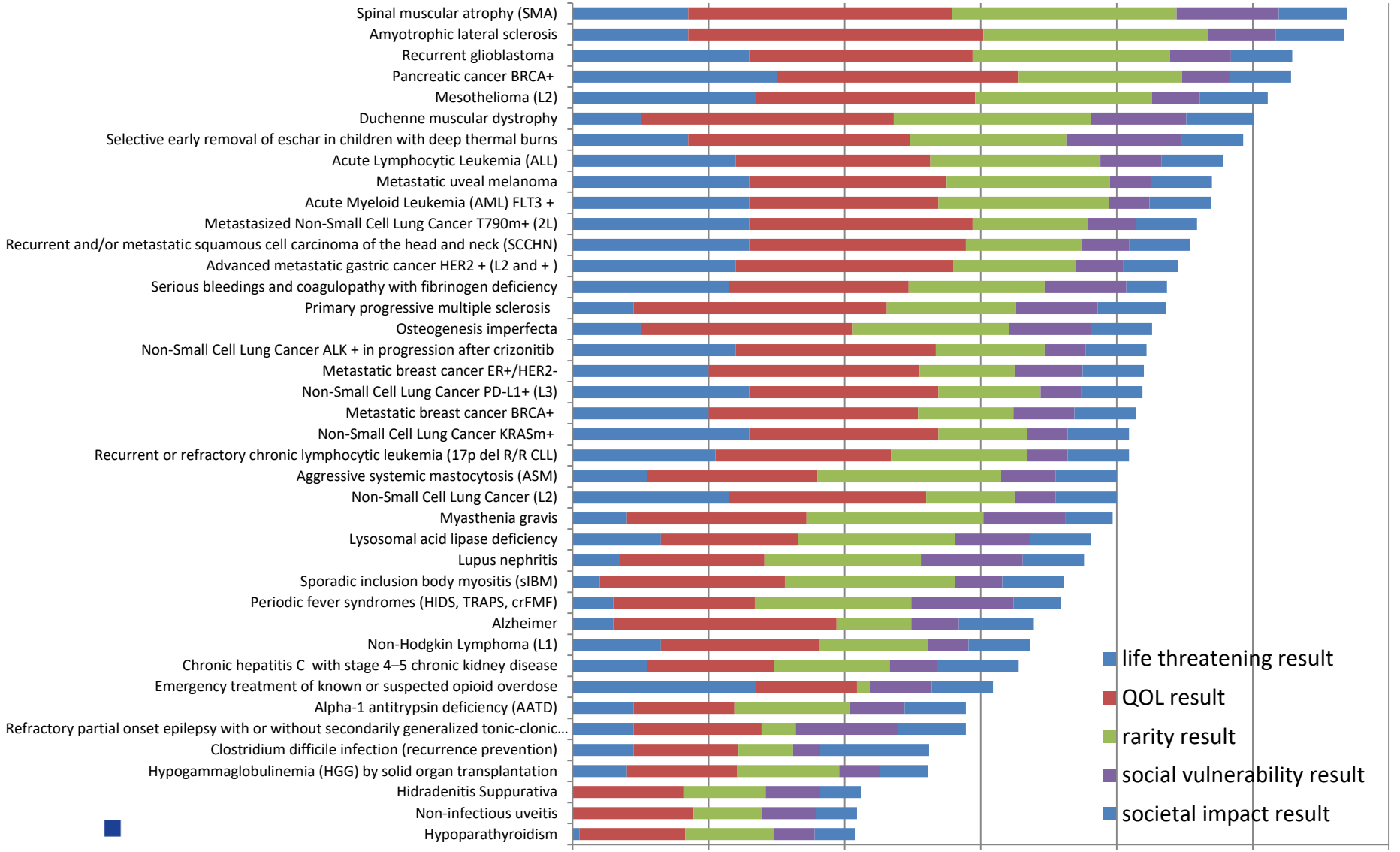
Discussion

# Criteria




Nineteen criteria and six conditions for reimbursement of interventions in health care

# UMN list 2016: Applications appraisal results



# Lessons learned + personal thoughts for a future research agenda

- Aspects of unmet need within a specific disease
- Aspects of uncertainty of an available treatment in a process of early access/early reimbursement
- Drug treatment versus disease management
- Differences for acute and chronic medical situations

# The Orphans within the Orphan diseases

- Very rare diseases or rare situations in common diseases
- Complex multi-organ diseases
- Less interest from the industry
- Neglected diseases in the Third World
- ….

*Auto-inflammatory diseases*

*MAS (macrophage activation syndrome)*

# Larotrectinib

(Drilon et al. N Engl J Med. 2018 Feb 22;378(8):731-739.)

- Targets several cancers that have NTRK gene fusion proteins in common
- Different unmet needs from children to elderly
- Lack of direct comparability data (Basket trials)
- “Few side effects” but “15% dose reduction because side effects with maintenance of efficacy”
- Implementation issues: screening for NTRK fusion

# Eculizumab

(Legendre CM et al. N Engl J Med. 2013 Jun 6;368(23):2169-81)

- A terminal complement inhibitor (C5a, C5b)
- aHUS but probably also useful in other “neighbouring” diseases that are not always studied
- Acute treatment versus prevention in high risk kidney transplants
- Dose and treatment duration?
- In our prioritisation tool acute situations difficult to judge !

# Caplacizumab

(Scully M et al. N Engl J Med. 2019 Jan 24;380(4):335-346)

- Acquired thrombotic thrombocytopenic purpura; immune mediated deficiency of ADAMTS13. Caplacizumab as an anti-von Willebrand factor
- Quid retreatment?
- How to associate with Plasmapheresis / Rituximab?
- Rituximab highlights the re-use of and the need for appropriate trialing of old drugs

# Zolgensma (AVXS-101)

- For SMA
- Onasemnogene abeparvovec-xioi
  One shot gene therapy?
- Long term outcome? QOL?
- What about the need of specific care i.e. specialised physiotherapy

# Conclusion

- Unmet needs can be prioritised
- In promising treatments, many uncertainties might remain – to be included in research prioritisation process as well

To be discussed:

When?

How?

By whom?

Ownership of data?

Who is paying?

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# Thank you!

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European Observatory on Health Systems and Policies 1998-2008

INAHTA