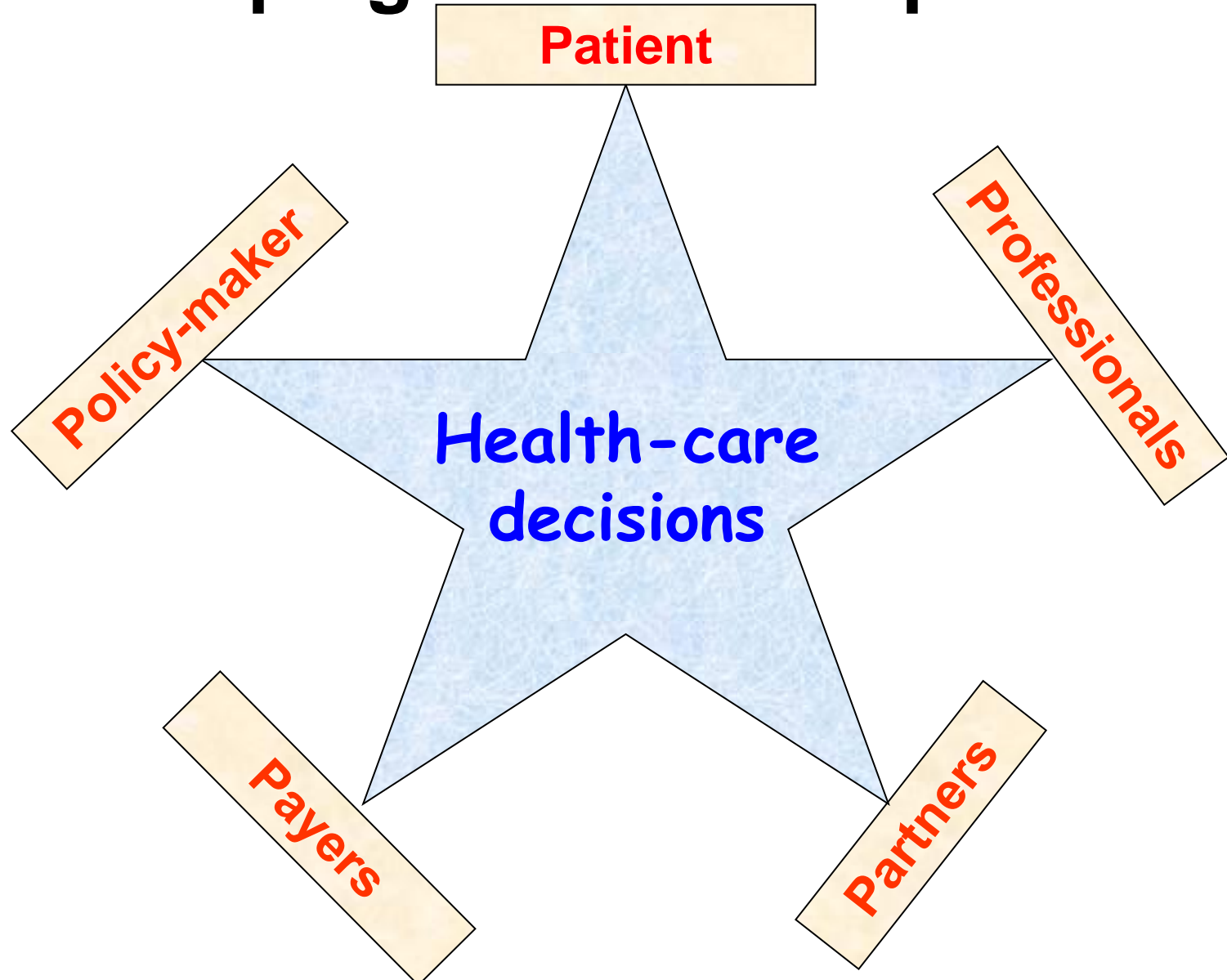# Sustainable healthcare system approaches for Rare Diseases. Options for Developing Countries
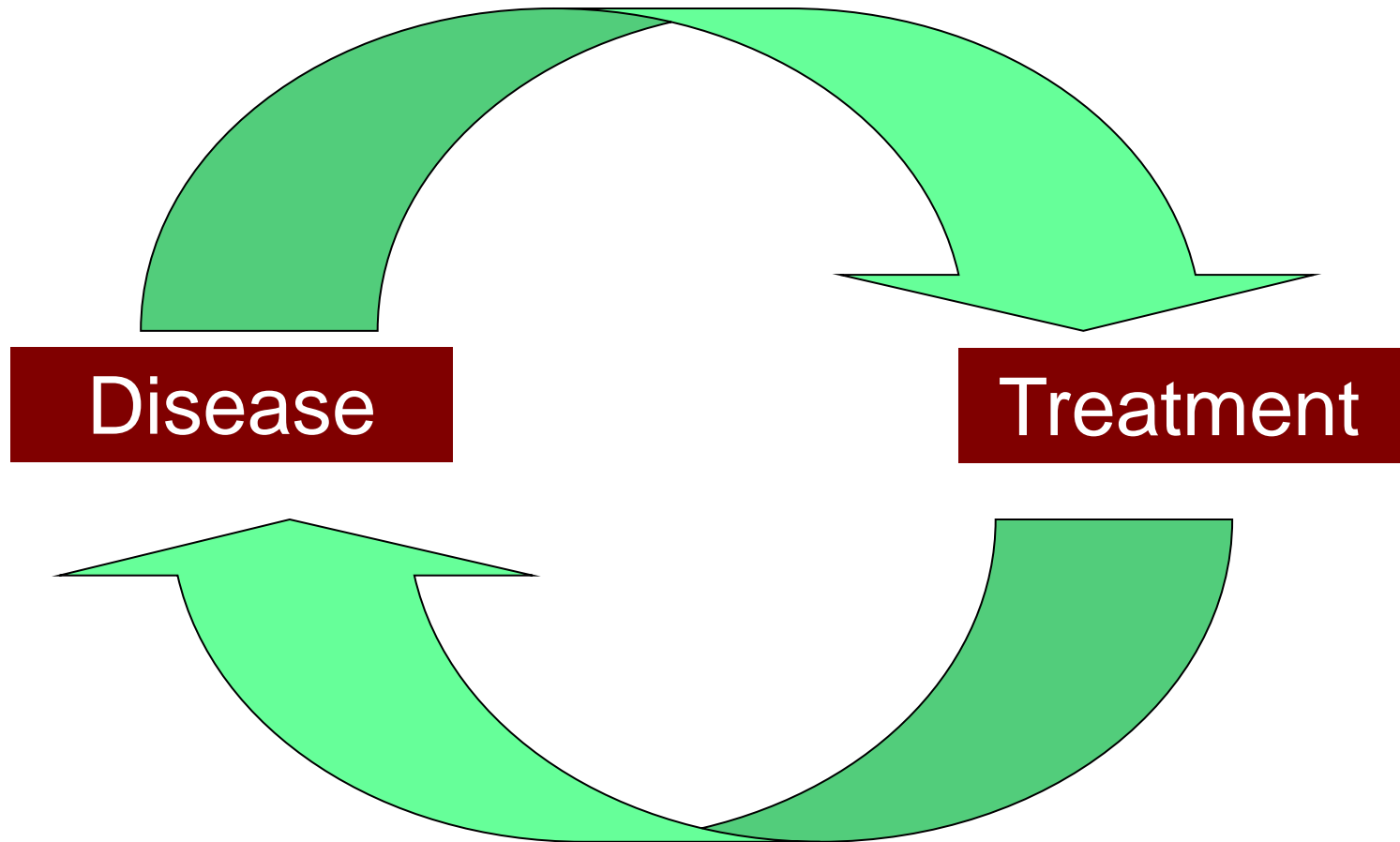
Joseph L. Mathew

What's done for rare diseases in developing countries at present?

Some exceptions to the rule…

- Brazil: Judicial process

- India: Employees State Insurance Act

- Limited social security/ Health insurance

The Paradox

Disease

Treatment

What can I do for this disease?

How can I use this treatment?

Is there another option?

KNOW ESSENTIALS

Mathew JL. IJTAHC 2011; 27: 139-150

KNOW

Knowledge of need (KN)

Outcome of interest (O)

Who is the target (W)

.

ESSENTIALS

Evidence of effectiveness/efficacy,

Safety,

Social quotient (ELSI)

Economic issues

Novelty (newness)

Time to outcome of interest,

Integration

Alternate options

Likely impact of not using this HT

Sustainability

KN = Knowledge of Need

What is the need?

- Epidemiology:

  – Burden to individual and community

  – Consequences: Complications, sequelae

  – Is there local data?

- Is this health technology needed?

O = Outcome of interest

What are the outcomes of interest (O)

Patient	Affordability, efficacy, safety
Professionals	Effectiveness, Efficacy, safety
Policy-makers	Effectiveness, Affordability, Sustainability, Coverage, Comparison with alternatives
Payers	Cost-effectiveness
Partners	Profit

W = Who is the target?

Who is the target? (W)

- Universal: All people with the disease.

- Selective: Limited subgroup only.

- Is KN described/defined for the target group?

- Is O defined for the target group?

E = Evidence of effectiveness, efficacy

Green	Robust evidence of effectiveness in the same population/ setting <u>OR</u> similar population/setting.
	No evidence of effectiveness, but robust evidence of efficacy in the same <u>OR</u> similar population/setting.
Red	Limited or no evidence of effectiveness OR efficacy in the same or similar population/setting.
Yellow	Data insufficient to categorise as Green or Red

S = Safety

Green	Proven short and long term safety profile. No serious adverse events (SAE) reported. Adverse events (AE) reported are within expected or accepted limits.
Red	Incidence and/or severity of adverse events is greater than expected or accepted limits
Yellow	Data insufficient to categorise as Green or Red

S = Social quotient

Green	No unacceptable ethical, legal and social issues (ELSI) with respect to the health-care recipient, provider and/or society.
Red	Unacceptable or unclear ethical or legal or social issues to the health-care recipient or provider or society, are involved.
Yellow	Data insufficient to categorise as Green or Red

E = Economic issues

Green	Clear benefit in terms of cost of HT, cost of providing HT and cost-effectiveness of HT. Cost-effectiveness not clearly proven, but cost of HT and cost of providing are favourable.
Red	Cost of HT and cost of providing are both high and there is low cost-effectiveness. Low cost-effectiveness, irrespective of the cost of HT and cost of providing
Yellow	Data insufficient to categorise as Green or Red

N = Novelty

| Green | HT is novel, but there is evidence of significant benefit for important outcomes such as mortality.

Existing HT in a contextually or conceptually novel setting; with evidence of effectiveness/efficacy. |
|---|---|
| Red | HT is so novel that short term and long term safety and/or effectiveness cannot be judged.

Beneficial effects in trials are not compelling enough to warrant application to individuals and/or community. |
| Yellow | Data insufficient to categorise as Green or Red |
| White | Not applicable |

T = Time factor

Green	Time to achieve outcome of interest is short.
	Time to outcome of interest is not short, but disease burden or its consequences are expected/anticipated to increase.
	Neither time to outcome of interest is short nor burden is expected to increase, but current burden/consequences are unacceptable.
Red	Time to outcome of interest unacceptably long or unpredictable.
	Irrespective of time to outcome of interest, disease burden and/or consequences are acceptable.
Yellow	Data insufficient to categorise as Green or Red
White	Not applicable

I = Integration

Green	HT can be integrated into existing health-care system with convenience and low additional cost. HT can conveniently replace an existing technology, thereby keeping additional cost(s) down. Although a separate delivery system has to be introduced, additional cost(s) is/are acceptable.
Red	Cost of separate delivery system is undesirable or unacceptable.
Yellow	Data insufficient to categorise as Green or Red
White	Not applicable

A = Alternatives

Green	No alternative option(s) is/are available.
	Alternative option(s) are not favoured/preferred based on this algorithm.
	Available alternative(s) are unaffordable or unacceptable for specific reasons.
Red	Alternative option(s) are favoured/preferred in terms of all the major and most of the minor criteria in this algorithm.
Yellow	Data insufficient to categorise as Green or Red
White	Not applicable

L = Likely impact of rejecting the HT

Green	Impact of rejecting the HT can be undesirable, unacceptable or harmful to the individual and/or society in terms of disease burden and cost of care.
Red	No serious consequences from rejecting the HT.
Yellow	Data insufficient to categorise as Green or Red
White	Not applicable

S = Sustainability

Green	Use of the HT is sustainable for an individual patient throughout the duration required. Use of the HT is sustainable for the community until the disease burden is controlled/ eliminated.
Red	Use of the HT is not sustainable for an individual patient throughout the duration required. Use of the HT is not sustainable for the community for long enough to control/ eliminate the disease burden.
Yellow	Data insufficient to categorise as Green or Red
White	Not applicable

How to apply the algorithm

Criteria	Importance/ Implication
Essential: KNOW	Do not initiate decision-making process if any of these components is unknown or unclear
Major: ESSE	Proceed if all the four criteria are green. If any are yellow, wait till more data available. Do not proceed if any is/are red
Other: NTIALS	Consider these only if above are met.
Overall decision	All Green: Proceed in favour of the technology Yellow: Wait (for more information/ data) Red: Do not proceed in favour of the HT Mixed: Check predominant colour and proceed as above

Thank you

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