INESSS
LE SAVOIR PREND FORME

MANAGING REAL WORLD SAFETY AND EFFECTIVENESS

INESSS – POINTS FOR CONSIDERATION
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Institut national d'excellence en santé et en services sociaux
Québec

ONE QUESTION

Sustainability of the healthcare system and equitable and timely access to new medications ;

Attainable goals ?? Yes but…

DEFINING INNOVATION

Innovative versus promising (from INESSS's perspective)

A drug may be considered promising if:

- it provides a measurable benefit for the patient compared to the other existing therapeutic options;
- the drug is prescribed for an illness for which there is currently no treatment;
- the drug has a great potential of improving the treatment of an illness for which the current treatment is unsatisfactory;
- the drug has the potential to improve the organization or the cost effectiveness of the health system.

THE CHALLENGES OF EVALUATION AND UNCERTAINTY

The word ‘promising’ implies a certain degree of uncertainty about a drug’s safety and effectiveness as well as its ability to efficiently fill a health need, whether that need is already filled or not.

The uncertainty may involve:

- the specific methodological limitations of the clinical evidence that is being submitted for registration (methodological study design);
- the disease’s characteristics that limit study designs (especially in the case of low prevalence diseases, rapidly progressing and dramatic diseases or diseases with a variable progression);
- the data and models submitted for a cost/effectiveness analysis of the molecule or its impact on the health system.

UNCERTAINTY AND THE RISK NOTION

The possible use of a product not supported by all the required evidence of its therapeutic value in a population of individuals for which the product is recommended, or for a population of individuals for which it is not recommended, but where there is a risk of use, brings us to the notion of clinical risk.

This risk may be pharmacoeconomic, economic or systemic in nature:

- When the evaluated product has not demonstrated an acceptable cost/effectiveness ratio.
- The budgetary impact is so great that it prohibits the product's use to treat people for which it is recommended, or for which there is a greater risk of the product's use than initially expected.

MEANS

In light of Canadian and international experiences,

To deal with the clinical uncertainty of determining the cost/benefit ratio, we need:

- A mechanism to develop evidence.

To deal with pharmacoeconomic uncertainty, we need:

- Mechanisms for partnership agreements with variable objectives that are adapted to the situation.
- Agreements on risk sharing.

RISK SHARING AGREEMENTS

The goal of a risk sharing agreement is to mitigate the uncertainty that the funder may have about the results, a population sub-group, the product's cost effectiveness (that is to say a cost-effectiveness that is unfavorable), the patient volume (that is to say the number of patients being treated) or the budgetary impact [Towse and Garrison, 2010].

In the scientific literature, several terms are used to refer to what are basically similar concepts: outcomes-based schemes, risk-sharing agreements, coverage with evidence development, access with evidence development, patient access schemes, conditional listing, managed entry schemes, pay-for-performance programs, performance-based agreements, etc.

EVIDENCE DEVELOPMENT

In a context of uncertainty about the product's therapeutic value, a takeover could be contemplated to promote access to a promising product, conditional to the production of additional data.

Concepts: coverage with evidence development or treatment continuation in order to achieve short-term therapeutic goals.

SITUATION EXAMPLES

Example of cases where additional data is needed to be able to submit a recommendation:

- The situation involves a small group of patients (suffering from a rare oncological illness, for example).
- The uncertainty is related to the study design (e.g.: the aggregate survival data is not reliable) the control group patients are transferred to the active group at the moment of crossover).
- The aggregate survival data or the long term safety data is not available.
- Data impacting the pharmaeconomic analysis (e.g.: data on the quality of life) is missing.
- Uncertainties about the drug's optimal use may have important repercussions on pharmaeconomic analysis.

IN WHAT PARTICULAR CASES?

Drugs are deemed promising when:

- They have significant clinical benefits for the patient.
- There is no available alternative.
- They show great potential to improve disease management (fills a need!).
- They can significantly improve the organization of health services.
- They help to achieve an overall cost-effective use of resources in the short, medium and long term.

EDUCATION ON THE CONDITIONS FOR SUCCESS

- Access to the drug must be fair and reasonable.
- The process must be transparent.
- Doable and realistic.
- A mechanism for exceptions.
- The financial burden of developing the evidence should be shared fairly between the maker and the government.

EDUCATION ON THE CONDITIONS FOR SUCCESS (CONTINUED)

- The goals should target the problems identified during the evaluation.

- The methodology used should be rigorous and lead to the measurement of tangible and strong results.

- The adoption of terms and conditions (such as derogatory clauses) should be accepted to allow continued funding (until cessation of treatments) if the results are negative or warrant a tightening of payment criteria.

CONCLUSION

- Providing evidence should be a considered option.
- The cumbersomeness and financing of the process should be a focus.
- It requires a sharing of the burden with the industry.
- International experience suggests that evidence development benefits from the active participation of clinicians in the data's development and collection, as well as from the cooperation of research and university centres and organizations having relevant expertise in independent data evaluation.