European & National Medicines Regulatory Systems: challenges for an equitable, timely and suitable access to innovation – 10th May 2017

Round table 3rd sessions
Patient Associations from countries with an official patients' committee in the National Medicines Agency

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Ministry of Health

PARLIAMENT
Votes laws and oversees implementation

Advice/public health policy

Pricing Committee (CEPS)

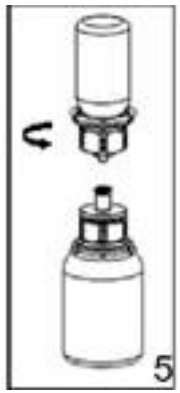
Advice/price

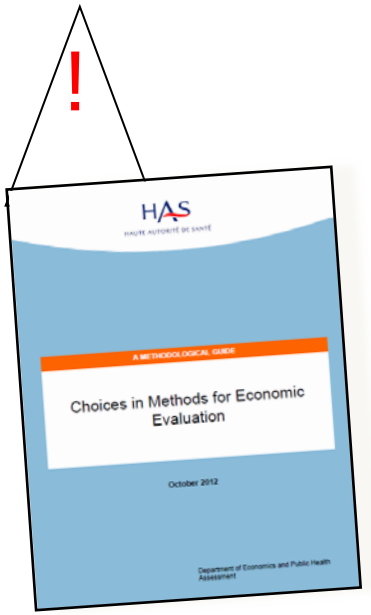
Haute Autorité de Sante

ANSM

Maximum spending targets

Advice/reimbursement



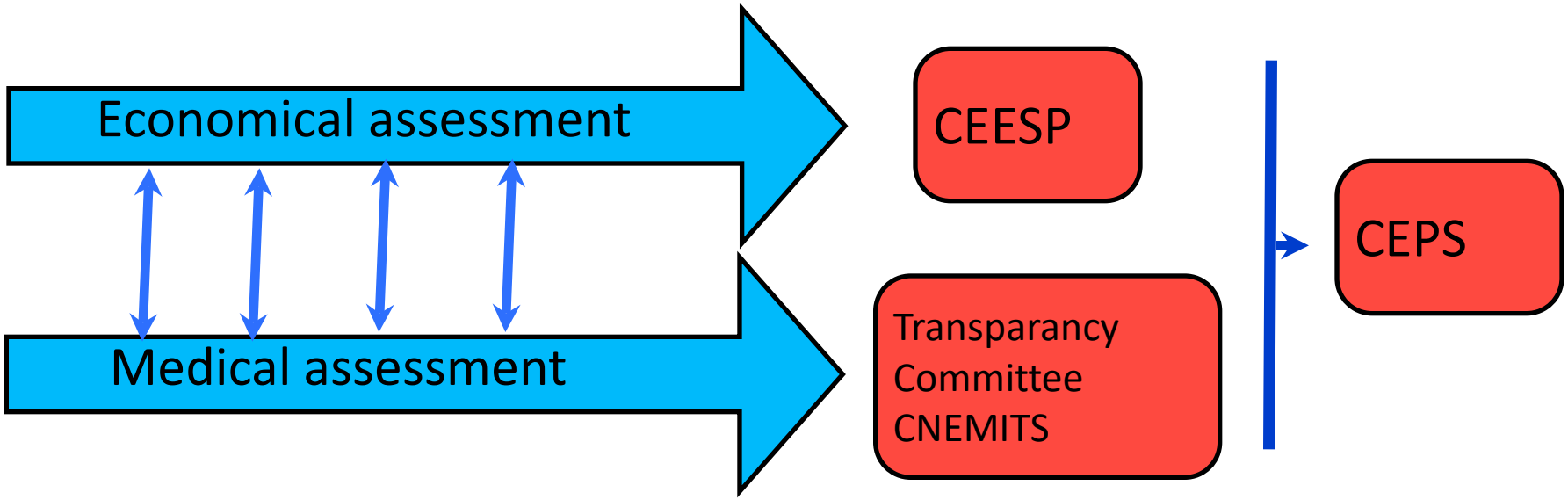


National health insurance funds (compulsory and voluntary)

Negotiation with

Health Professional Unions

Source: HAS 2013



Source: Haute Autorité de Santé 2013

Involvement?

User/patient representative in ANSM and HAS as member of board/collège (ANSM and HAS) or working groups or Committees (ANSM and HAS) :

ANSM in charge of the marketing authorization (benefit and risk),

HAS in charge of

- **Medical benefit, therapeutic added value and efficacy and safety (CT)**
- **Efficiency (CEESP)**

New opportunity for patient's organisations: during the assessment of a medicine or a medical device.

Since November 2016, HAS has asked to patient's organisations to write contributions and answer questionnaire:

- impact of the disease or the state of health of the patients/family caregivers, their perceptions, their quality of life and families ones,
- experience of patients treated with other therapies already avaible,
- experience of patients with the product assessed

Evaluation in september 2017.

CEPS : no user/patient representative member of the committee. Until now, only hearings.

Evaluation patients' involvement in terms of impact and efficacy? What are its strengths, weaknesses and challenges?

- Challenges : take into account the patient's objectives and preferences (cultural thing for Health agencies, methodology), management of potential conflict of interest, competition between disease, fair pricing (heterogeneity across Europe of methodologies to fix the price), access to care (weight of the cost of drugs).

- Patients organization, particularly in rare diseases, must provide/must be asked

1. Their analyses about the real burden of a disease (QoL) and real efficacy actual treatment (among them adverse events) and the role of patients' education (experience)
2. evidence from scientific articles, patients registries, patients testimonies (experience about illness and treatment and real case example), own survey questionnaires

- It requires an access to data from specific registry/National Health Insurance Funds and knowledge of the price of medication
- Drug analysis and Health economic analysis require expertise but also involvement of individuals («lay experts») in the collective decision making process impacting individuals and public health