HAUTE AUTORITÉ DE SANTÉ

# Methodological guidance for life sciences industry

**François Meyer**
**Director, HTA Division**
**Haute Autorité de Santé, Paris**

# HAS
# French National Authority for Health

- **6-year old institution**
- **8 members of the Board (Collège)**
  - 5 members out of 8 are physicians

    Chair Prof. Jean-Luc Harousseau since 2011
  - Prof. JM Dubernard
  - Prof. G. Bouvenot
  - Dr JF Thébaut
  - Dr C. Grouchka
  - JP Guérin, former director of a teaching hospital
  - Alain Cordier, former director of Paris hospitals
  - Lise Rochaix, Professor of Economics

# HAS missions

## Broad scope of missions

- **Health technology Assessment :** drugs, devices, diagnostic and interventional procedures, Public health actions and programs
- Clinical practice guidelines,
- Chronic disease management models and guidance,
- Guidance and recommendations on the most effective strategies (prescriptions, care pathways...)
- Continuous professional development,
- Hospital accreditation,
- Quality of the information provided to health professionals and patients

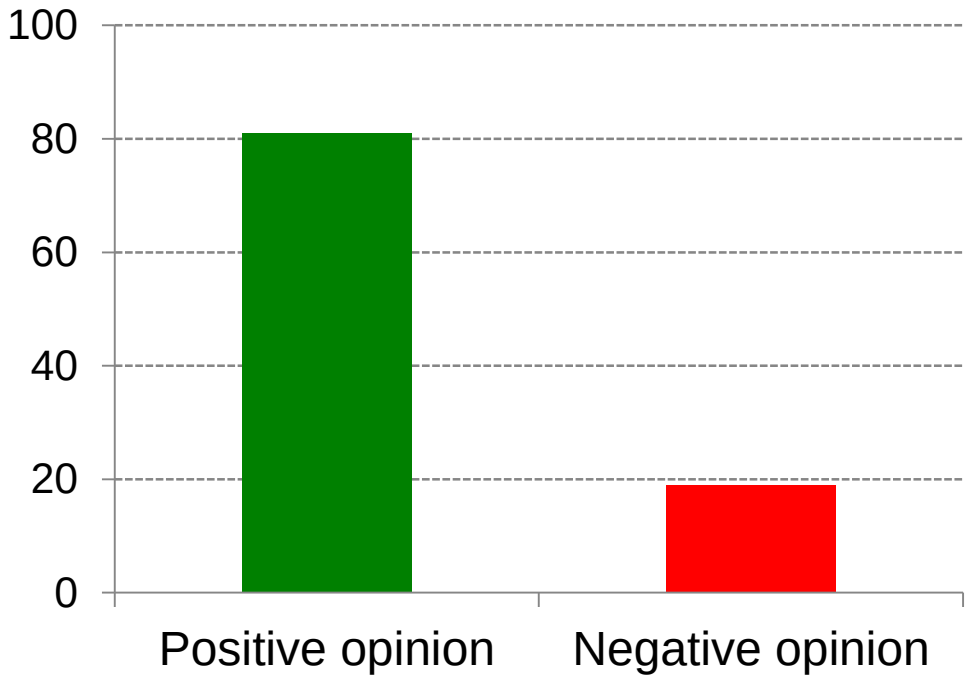
# HAS activity report for 2010

- **795** single technology assessment on medicines
- **159** single technology assessment on MDs
- **20** HTA reports on procedures (diagnostic or therarp)
- **13** health economic assessments
  public health programs recommendations
- **20** proper use leaflets (4 drugs, 5 MDs, 11 procedures)

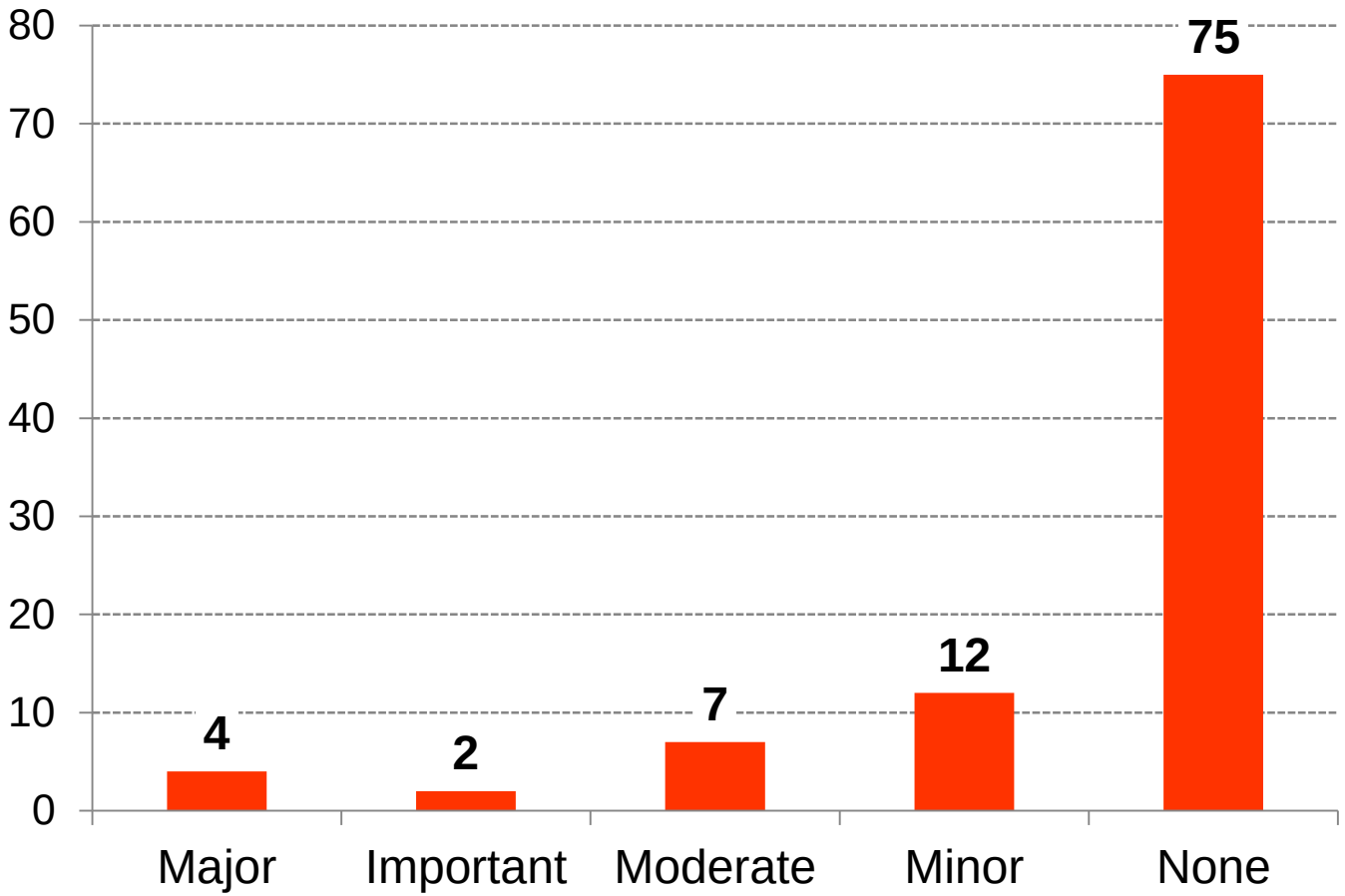
# Assessment of MDs by HAS, 2010

- **Commission nationale d'évaluation des dispositifs médicaux et technologies de santé**
  - Chair Prof. JM Dubernard
    Catherine Denis, MD, Head of MD evaluation department

- **2010 results:**



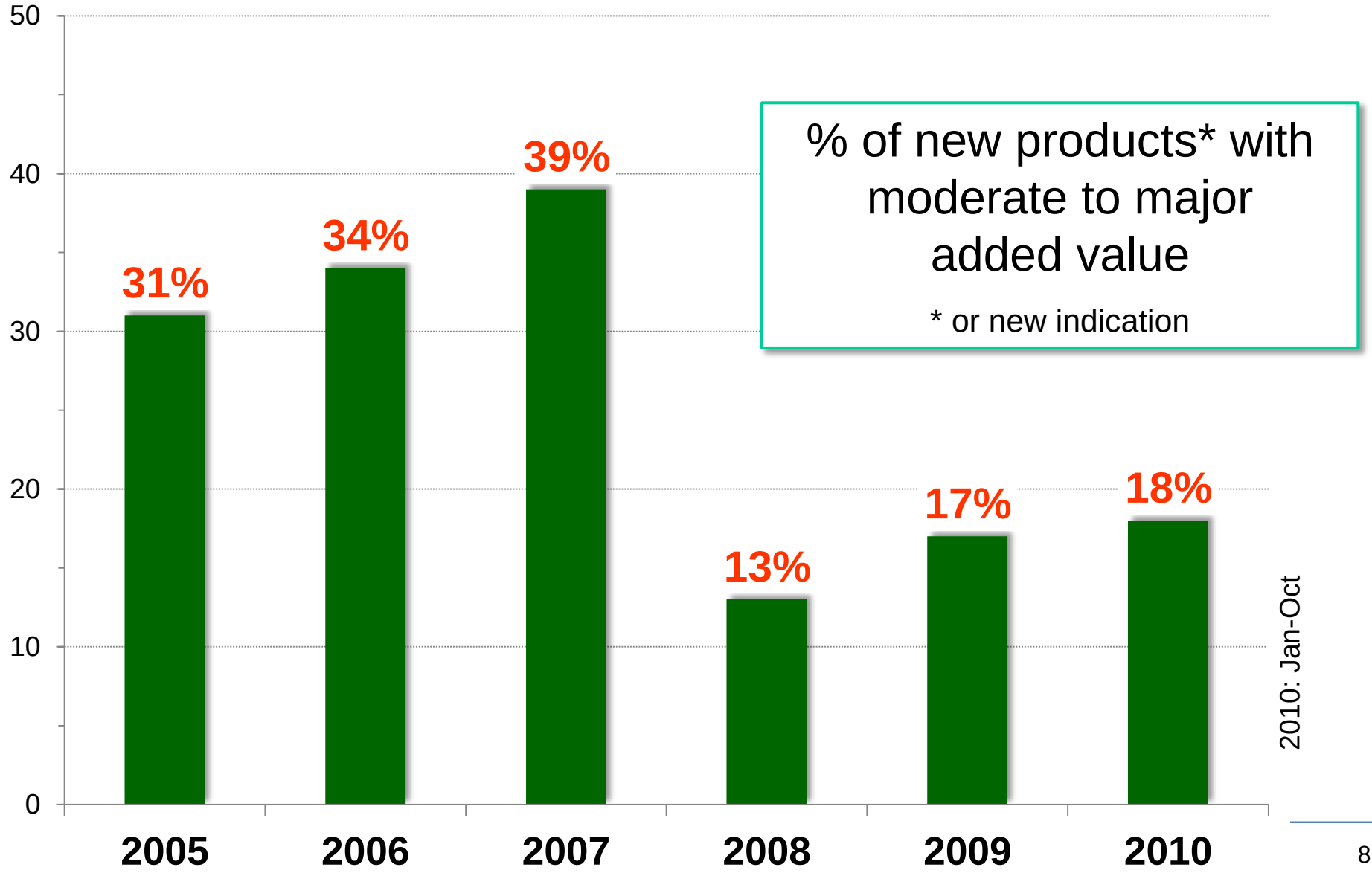
# Clinical added value for MDs (2010)



# Assessment of Drugs by HAS

- **Commission de la transparence**
  - Chair Prof. G. Bouvenot
  - Anne d'Andon, MD, Head of Pharmaceuticals evaluation department
- **Results over time**
  - As compared to MDs, more clinical data are available
  - Drugs have been granted a marketing authorisation => positive benefit/risk ratio in clinical trials (experimental context) :
  - Clinical effectiveness ? Recent doubt on the clinical effectiveness of new drugs > **Negative opinion**
  - Clinical added value over existing therapies ?

# Drugs considered as bringing clinical added value



# Request for post marketing data collection to reduce uncertainty

- **Additional evidence generation:**
  - French regulation allows HAS to make the request of 'post-listing' data collection, to be performed by the companies, to reduce uncertainty
- **From 2004 to 2010 : 346 requests made**
  - 166 for Drugs
  - 180 for Medical Devices
- **Questions raised not always appropriately answered**

# Summary of the problem

- **Limitations of various causes to the amount and adequacy of data produced**
- **Current debate on safety and effectiveness of health products**
- **How to reduce the gap between data produced by industry and expectations from the HTA world and the patient perspective?**
- **Actions needed to improve adequacy of data**
  - Early Dialogue / Scientific Advice
  - Disease specific guidelines

# Early Dialogue / Scientific advice

- Scientific advice meetings are organized in order to provide **responses to specific questions** pertaining to the development of innovative health technology to support its proposed use and reimbursement.

- **Aim:**
  - Not to substitute a company's responsibility in the development of the technology.
  - Optional, not legally binding, neither for the developers nor for HAS (advice can not be taken as indicative of any future agreed position).
  - Questions may address specific scientific issues on the clinical development; e.g. endpoints, trial duration, study population, choice of comparator(s), study design, safety, methodological

# HAS and Scientific Advice activities

- **No formal decision to be engaged in regular SA activities**

- **Some pilots conducted at national level**
  - Drugs
  - Devices
  - Procedures

- **Participation in international pilots on SA**

# Broad EMA/HTA scientific advice Tapestry network

- **Multistakeholder consultation in early stage drug development**
- **Three pilots (EMA/HTA meetings) up to now to discuss added therapeutic value of a drug in development:**
  - **Pilot 1**: new anti-DM2 drug that would treat both DM and its risk factors (obesity, hyperlipidemia and atherosclerosis)
  - **Pilot 2**: new treatment of DM2 patients with elevated CRP (2 aims: treatment of DM2 and slowing of disease progression).
  - **Pilot 3**: new treatment of breast K, 2 populations (ER+ and triple negative breast K)

# Broad EMA/HTA scientific advice
# Organisational aspects

## Procedure

- **Briefing book**
- **Broad advice: more « parallel » than joint advice**
  - EMA: gives an independent SA following the classical 70-day procedure
  - HTA: representatives from several HTA bodies, give oral recommendations during the discussion meeting with the company (no written advice)
- **Final advice:**
  - confidential, not shared between regulators and HTA bodies

# Broad EMA/HTA scientific advice
# What may be improved

- **Briefing book !!! (Content, timing)**
- **« Parallel » EMA/HTA advice:**
  - Each organisation (EMA, HTA) independently assess the SA request
    - EMA - targeted questions? (product development)
    - treatment added value, HTA – targeted questions? (active comparisons, outcomes, pragmatic trials)
  - Discussion of the SA request by HTA representatives before the meeting with the EMA and the company:
    - may be of interest, not mandatory
  - Final written HTA recommendations to be issued after the discusison meeting
    - by each HTA body participating in the exercise ?
    - compiled document ?
- **Final advices (EMA and HTA) to share? (confidentiality agreement)**

# Disease specific guidelines

- **Various reasons to develop disease specific guidelines**
  - SA time consuming, on a voluntary basis, confidential
  - International guidelines exist for drugs licensing
  - Medical devices industry need to be stimulated and guided for the production of clinical data
- **HAS actions**
  - Ongoing development of guidelines for MDs (Wound healing)
  - International Cooperation +++
    - EUnetHTA
    - Others