**CLINICAL INNOVATION:** Fair & Effective Incentives for New Uses of Established Drugs

# The Politics of Incentivising SMUs

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EURORDIS
RARE DISEASES EUROPE

# THE POLITICS OF INCENTIVISING SMUs

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François Houÿez

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## EURORDIS, the European Organisation for Rare Diseases

- Founded in 1997 to support the adoption of the Orphan Medicinal Products Regulation
- As a patient organisation, societal responsibility to ensure the success of the regulation
  - By participating to the orphan drug designation process (COMP)
  - By engaging our members in scientific advice, protocol assistance, CHMP opinions
  - By contributing to policy review
  - Through a permanent dialogue with the EMA, European Commission, European Parliament, industry and our members
- E.g. 21 February 2018 workshop, Brussels: ***Rare Disease Therapies: do we get what we incentivise?***

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## January 2004: pegylated interferon and Vaquez disease, France

- Polycythemia vera is an acquired myeloproliferative disorder, life limiting
- Treated by phlebotomy
- Or Interferon but with long lasting flu-like syndrome (1 week)
- Young woman diagnosed in 1994 requested Pegylated interferon alpha-2b
- Peg-Interferon authorised for hepatitis C
- Flu-like syndrome effects last for just 1 day

**The initiation of discussions for reimbursement helped investors to decide: early dialogue with payers? Cf MOCA**

- Initial request for Peg-Inf was rejected due to its cost (16 000 € /month)
- And the absence of evidence
- Eurordis helped discussions with MAH and authorities
  - Manufacturer was asked to conduct pilot studies to generate the required evidence
  - Discussions started on conditions for reimbursement as Off-label use
- 2011: a different company obtained orphan drug designation for Peg-Inf for Vaquez
- 2016: Clinical trials are in progress

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## May 2012: anakinra for amyloidosis in a patient with multiple myeloma

- Belgian MM patient group contacted EURORDIS for a negative decision to reimburse anakinra to treat amyloidosis following paroxysmal peritonitis
- Anakinra (IL2) authorised in 2002 for Arthritis, Rheumatoid
- Belgian Special solidarity fund had refused to cover the off-label use

**Another incentive could be the provision of expert advice to authorities and/or investors (as for scientific advice and/or protocol assistance for orphan medicinal products)**

- Anakinra was tested in clinical trials for different types of amyloidosis
- Clinical research was conducted in a specialised centre in Italy
- Eurordis made contact between Belgian doctor and Italian team
- Scientific rational to use anakinra to treat amyloidosis was presented to Belgian authorities in an appeal
- Patient could start treatment again 2 weeks after its interruption
- And could save his kidney function



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## EURORDIS survey to 1,350 patients: some 300 conditions / off-label use pairs. A few examples:

| Rare Condition | Product | Authorised Indication | Off-label use |
|---|---|---|---|
| Sotos syndrome + congenital heart disease + heart failure as a baby | IV furosemide | congestive heart failure and oedema | As a baby used IV furosemide orally to reduce fluid load as there was no suitable per os dose |
| Ocular melanoma | pembrolizumab | Carcinoma, Non-Small-Cell Lung, Hodgkin Disease, skin melanoma | |
| Narcolepsy cataplexy | mazindol | Short-term (i.e. a few weeks) treatment of exogenous obesity (amphetamine like) | 1 dose per day, lifelong, with Modiodal and Effexor |

## Dilemma in the patients' community

- General interest: need for evidence based medicine and full assessment of benefits, risks and quality
- Individual's interest: access to the treatment they need
  - The public in general and patients in particular have sympathy for measures that can enlarge access
  - And consider advocating for the general interest is in fact advocating for ther financial interest of industry only



## Proposals

- Off-Label use in the absence of full evidence
  - To generate benefit/risk information for each off-label use: feasibility? Value of information?
- Therefore:
  - Scientific Advisory Group (at EMA) for a case by case scientific opinion
  - With the participation of experts in rare diseases if needed
  - When evidence missing: evidence generation plan
  - With measures to prevent using this opinion as a substitute for a marketing authorisation
    - for an off-label use that represents a small fraction of the product sales
- Fair price : discussions with MOCA, objective appreciation of fair price (discounted cash flow method)