

# **The orphans need FOSTER: Towards a sustainable model for reimbursement of orphan drugs**



# Who we are

**KU LEUVEN**

Department of Pharmaceutical and  
Pharmacological Sciences

Clinical Pharmacology &  
Pharmacotherapy

Prof. Steven Simoens  
Dr. Eline Picavet  
Thomas Morel



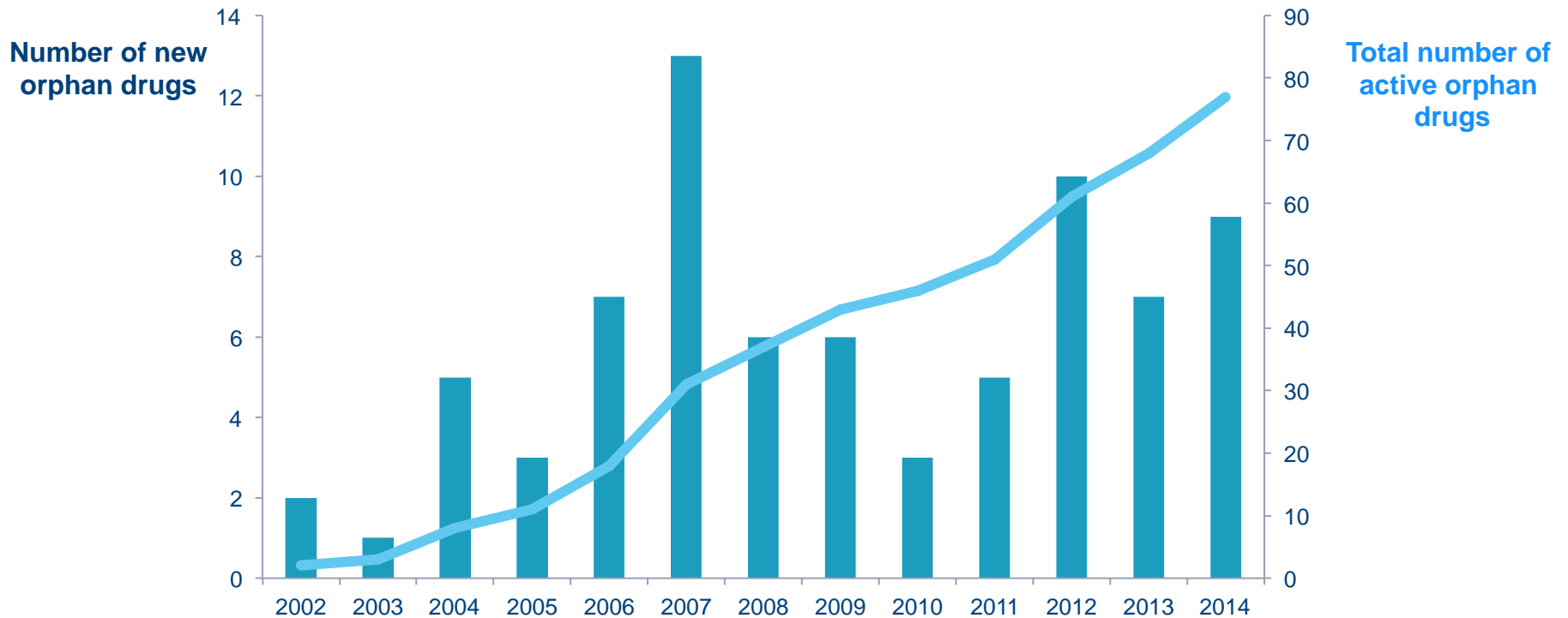
Metabolic Centre

Prof. David Cassiman

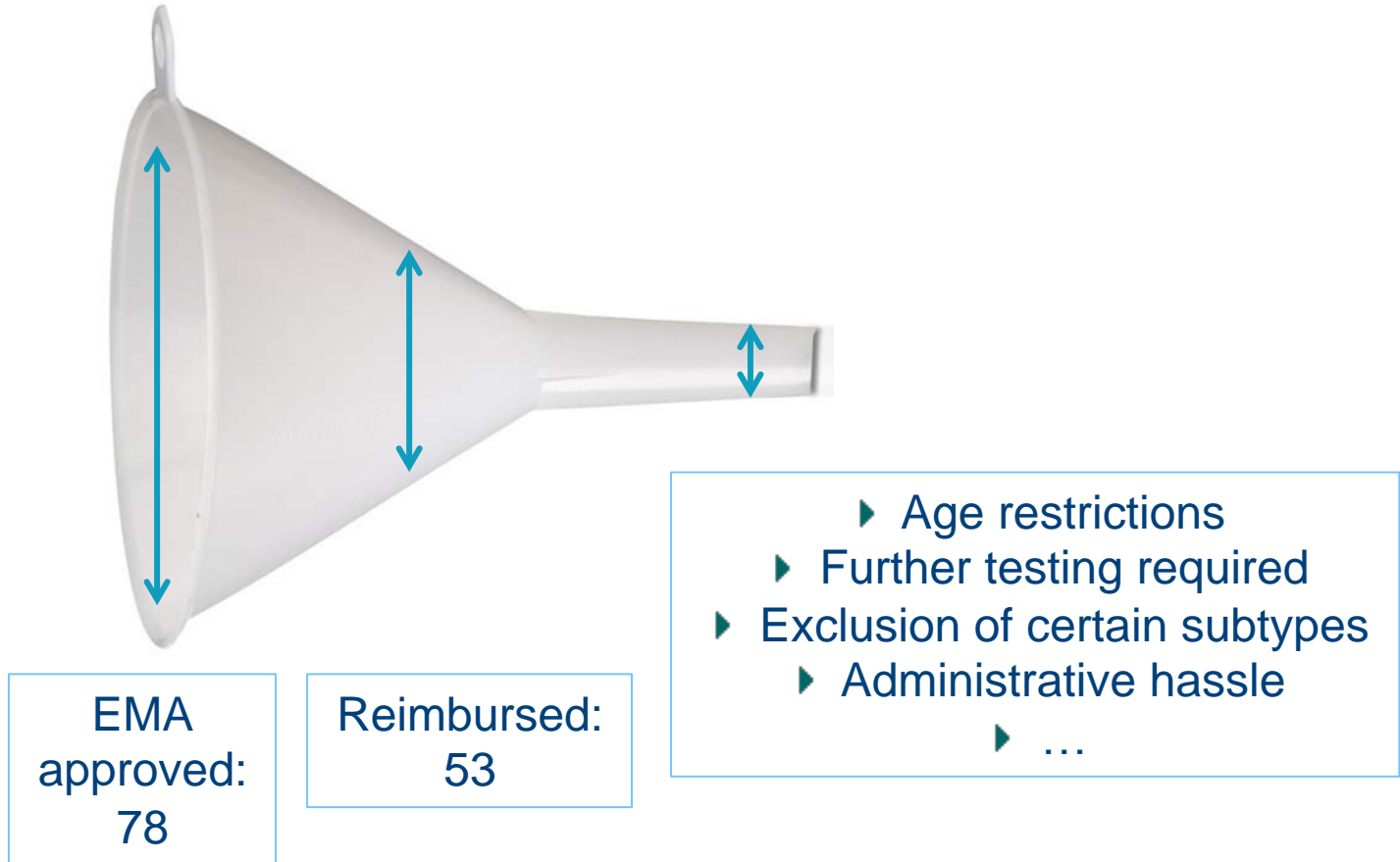
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# Background: rising number of orphan drugs

REGULATION (EC) No 141/2000 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL  
of 16 December 1999  
on orphan medicinal products



# Current situation



# Current situation

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- ▶ **EU principles: Charter of Fundamental Rights**
  - ▶ Society has a moral obligation to provide a minimum of health care to help the less fortunate and not to abandon those in need.

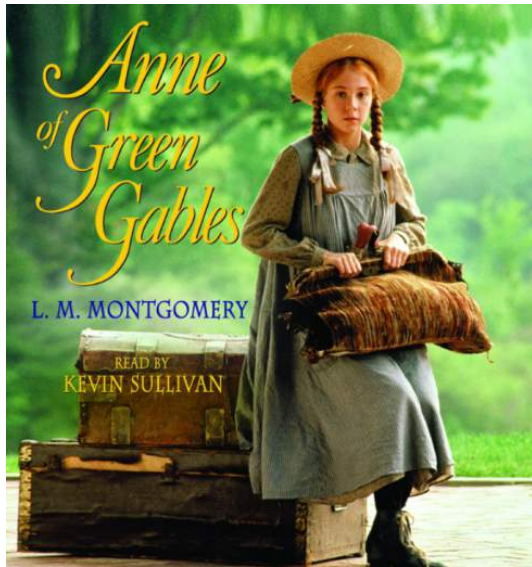
# Current situation

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- ▶ **Advances in scientific knowledge and innovation**
  - ▶ most orphan drugs are *innovative* drugs: gene therapy, enzyme replacement therapy, biologicals & cell therapy
  - ▶ half of the R&D investments in e.g. Belgium originate from biopharmaceutical companies

# A sustainable model: A fair share for all - “Anne of Green Gables”



## Principles

- 1) budgetary insulation of a guaranteed, but limited share of resources dedicated to orphan drugs
- 2) guaranteed access for some according to rational priorities
- 3) possible access for all (at random): “Anne of Green Gables principle”

**Pinxten W**, Denier Y, Dooms M, Cassiman J-J, Dierickx K. A fair share for the orphans: ethical guidelines for a fair distribution of resources within the bounds of the 10-year-old European Orphan Drug Regulation

**Picavet, E., Cassiman, D., Pinxten, W.,** Simoens, S. (2013). Ethical, legal and social implications of rare diseases and orphan drugs in Europe: meeting report of a Brocher symposium. *Expert Review of Pharmacoeconomics and Outcomes Research*, 13 (5), 571-573.

# FOSTER: Fund for Orphan drugS Towards Ethical Reimbursement

## 1) Budgetary insulation: **FOSTER**

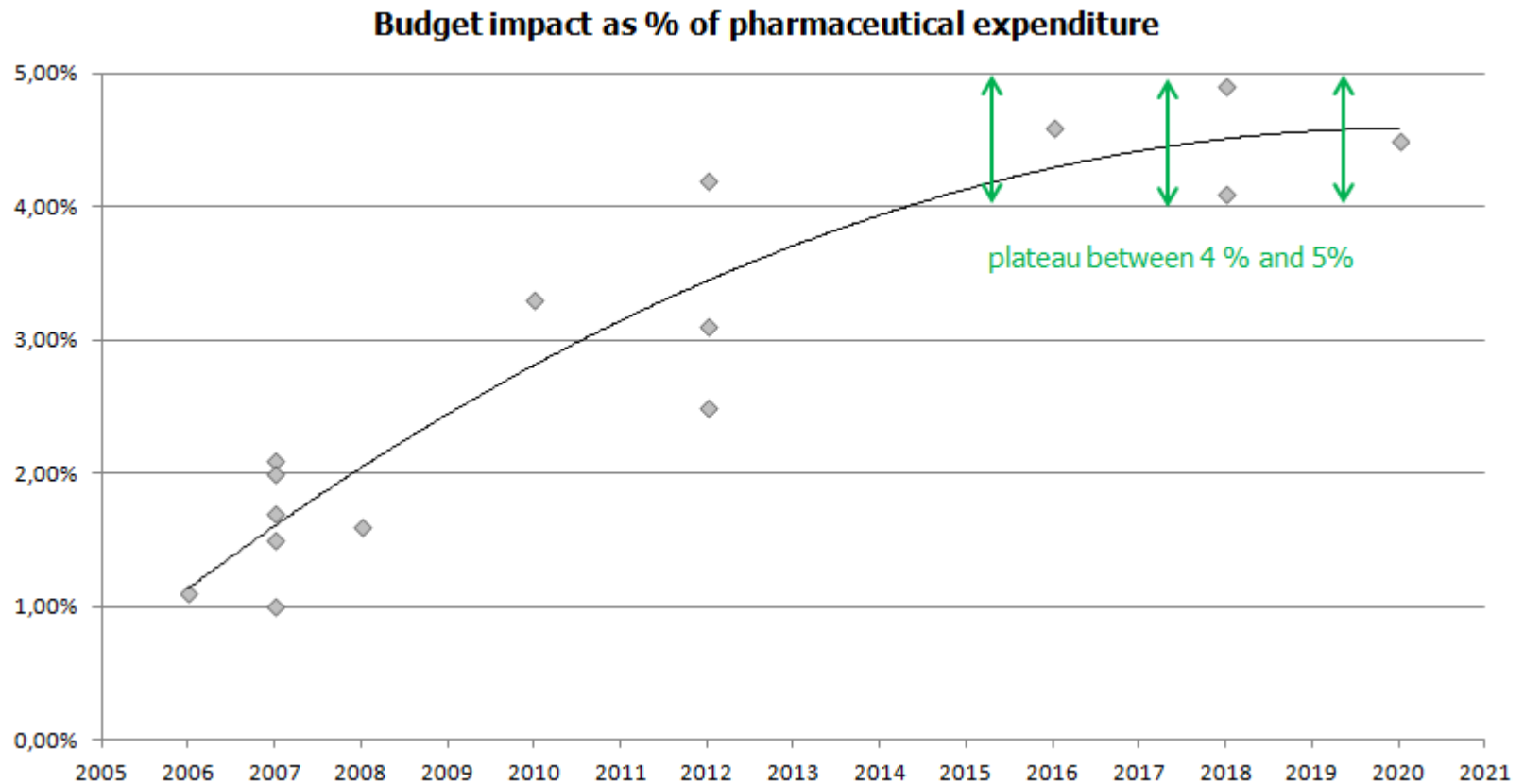
- ▶ to fund **non-oncology drugs** immediately after EMA approval

- ▶ drugs for unique indications
- ▶ no alternatives

- ▶ estimated budget: ?

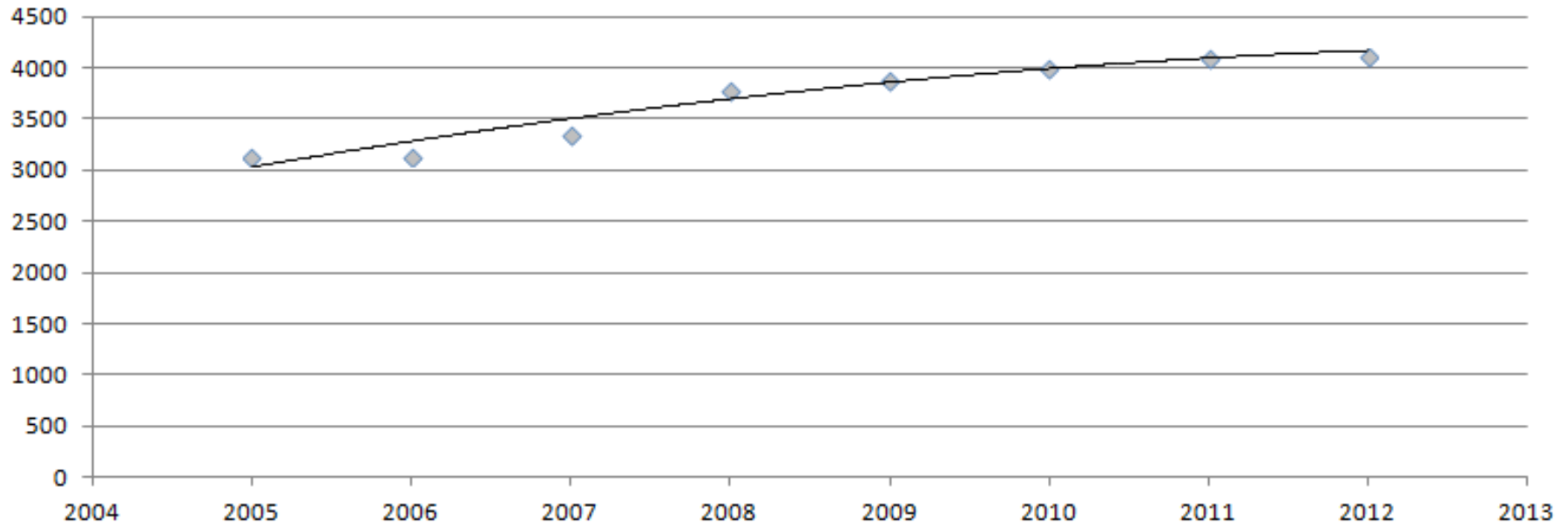


# Budget impact likely to plateau between 4 and 5%



# Estimated expenditure

**Net yearly RIZIV pharmaceutical expenditure (in mln)**



- ▶ BI: 4%-5% van [4000mln;4500mln]
- ▶ [160mln;225mln]

thank you!



[eline.picavet@pharm.kuleuven.be](mailto:eline.picavet@pharm.kuleuven.be)  
[david.cassiman@uzleuven.be](mailto:david.cassiman@uzleuven.be)