The orphans need FOSTER: Towards a sustainable model for reimbursement of orphan drugs
Who we are

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Background: rising number of orphan drugs

of 16 December 1999
on orphan medicinal products

Number of new orphan drugs
Total number of active orphan drugs
Current situation

- EMA approved: 78
- Reimbursed: 53

- Age restrictions
- Further testing required
- Exclusion of certain subtypes
- Administrative hassle
- ...

KU LEUVEN
Current situation

- **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

- 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”

**References**

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

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

- BI: 4%-5% van [4000mln;4500mln]
- [160mln;225mln]
thank you!

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