# From GMP

# to

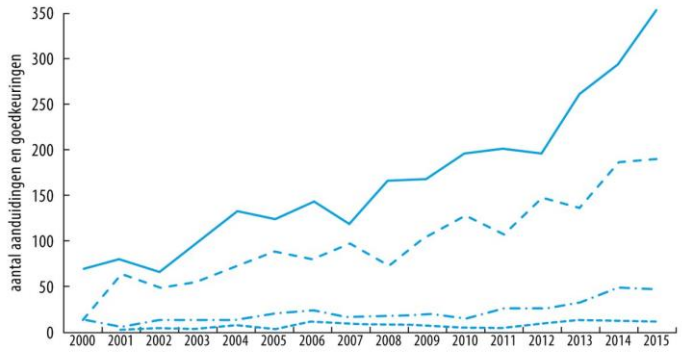
# SMP?

CbusineZ

Partner in zorginnovatie

# The current system, although provides successes, will not provide accessibility and sustainability

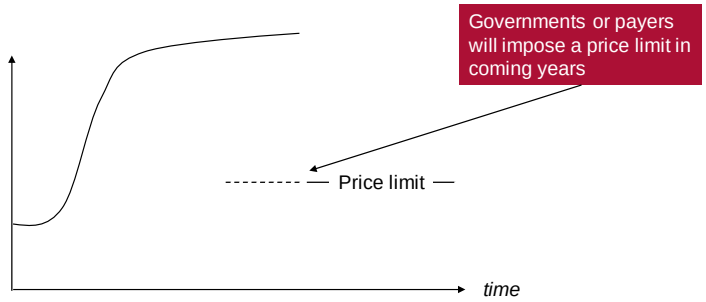
***OD development is, under current system, too slow***




**FIGUUR** Aantal aanduidingen en goedkeuringen van weesgeneesmiddelen in de Verenigde Staten en de Europese Unie, in de periode 2000-2015.

With 90 out of 6000 OD covered and pipeline very focussed on cancer, development is too slow

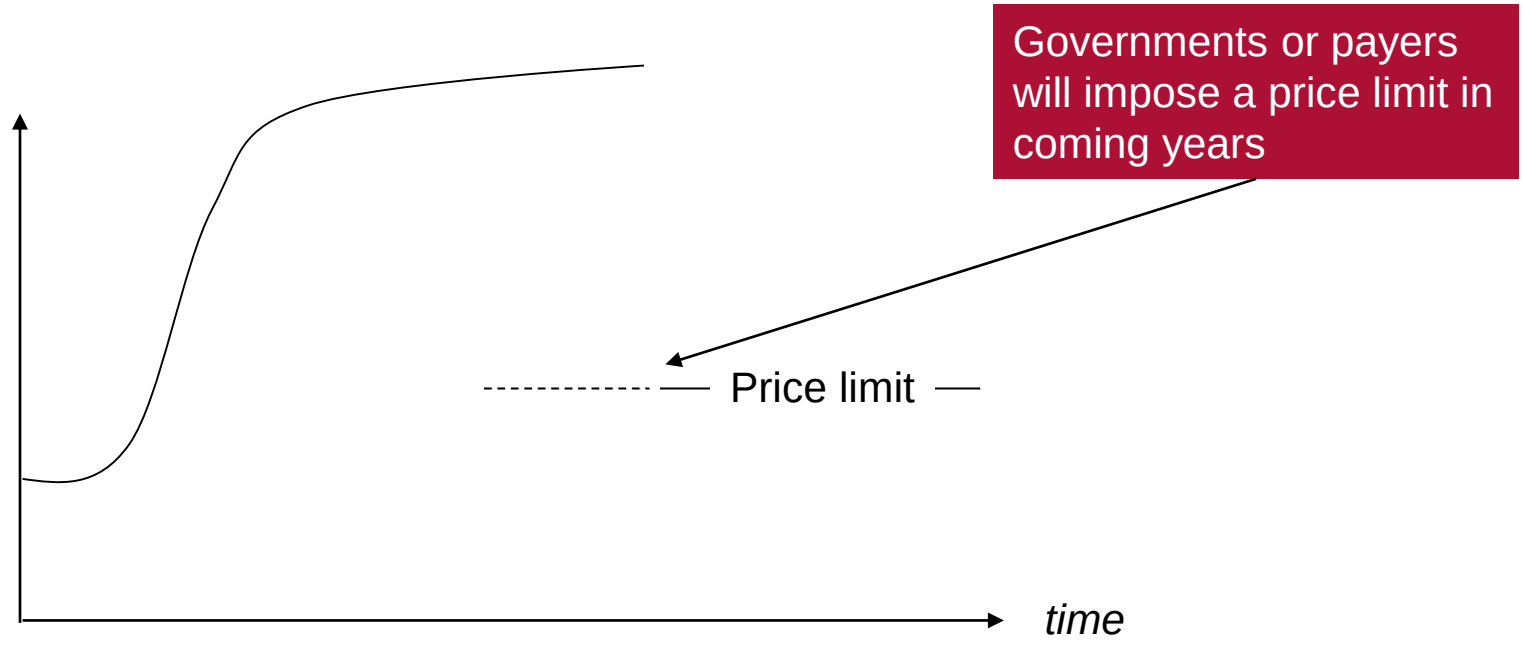
***With current pricing levels OD become unsustainable***




Current pricing level for OD x New drugs in pipeline = Unsustainable

With current pricing levels (still rising) and pipeline the inconvenient truth is that OD will be unsustainable

# The inconvenient truth: the cost challenge will lead to a price or budget limit




Current pricing level for OD x New drugs in pipeline = Unsustainable

# A partnership for the future:
## Sustainable Manufacturing Practice (SMP)

***New technological options***

- Personalised medicine
- Companion diagnostic (example organoids)
- Big data
- Small Scale Production

***Legal – societal innovations***

- Fast track
- Pressure to change the model

~~GMP~~

Sustainable Manufacturing Practice

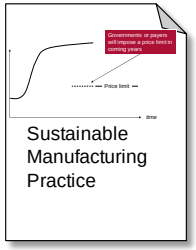
Within pricing limits

Personalised

Based on companion diagnostic

effective

# “SMP” requires a paradigm shift




***Research*** ***Development*** ***Admission*** ***Treatment***

Regulatory affairs

university

Pharma

registry

Public funded: only sell/licence under SMP….

Framework to develop and test for small groups.

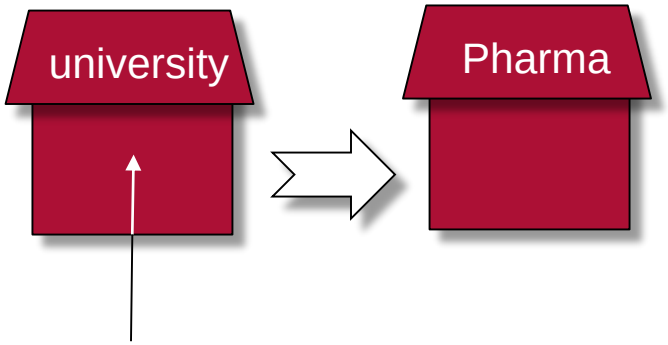
Conditional Approval Pending efficacy In practice

Companion diagnostic

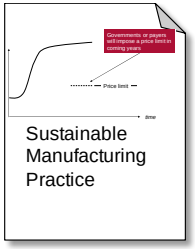
Small Scale Production

Public registry

# If the research is funded with ‘public money’ there should be an obligation to enforce SMP




Public money to fund research
- Grants
- Taxes
- Funds from patient organisations

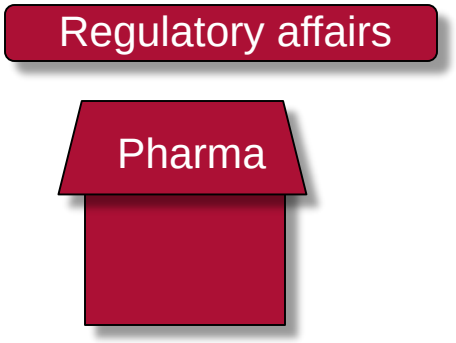



A legal obligation to research under ‘SMP’ when using public funds

The innovation is sold / licensed under SMP:
- The next party has the obligation to ensure that end pricing level doesn’t exceed Sustainable Pricing Limit

# Switch from the large volume framework to small group framework

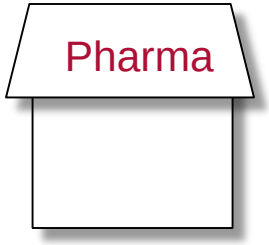
***The regulatory framework is not suited for small groups***




Our frameworks were designed in the blockbuster era

Large scale trials, strict focus on production

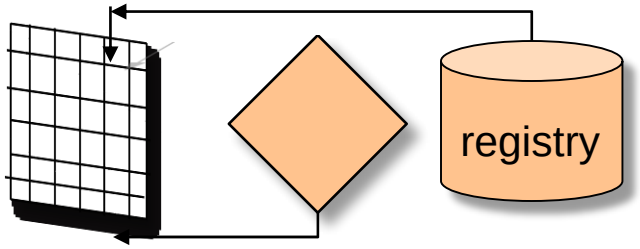
***Create a framework for small groups: faster and cheaper***




Create a new framework that still focusses on safety but acknowledges the difficulty of small groups

# Under ‘SMP’: conditional admission pending efficacy evidence from registry

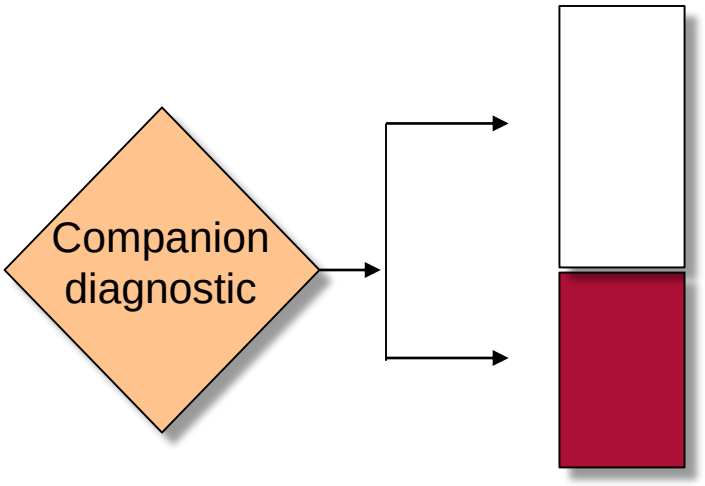
Under ‘SMP” price is not the issue… admission should focus on efficacy




Conditional admission Pending efficacy in practice
Admission based on Companion Diagnostic
‘If it works it’s reimbursed

| | |
|---|---|
| Accessibility is greatly improved if drugs are conditionally approved for a short term and based on CD<br><br>During this approval period the efficacy has to be established, in practice, using a public registry | ***Example***<br><br>Drugs for CF not based on gen mutation but on CD: Organoids |

# Companion Diagnostics facilitate Personalised Medicine and reduces waste




Actual case

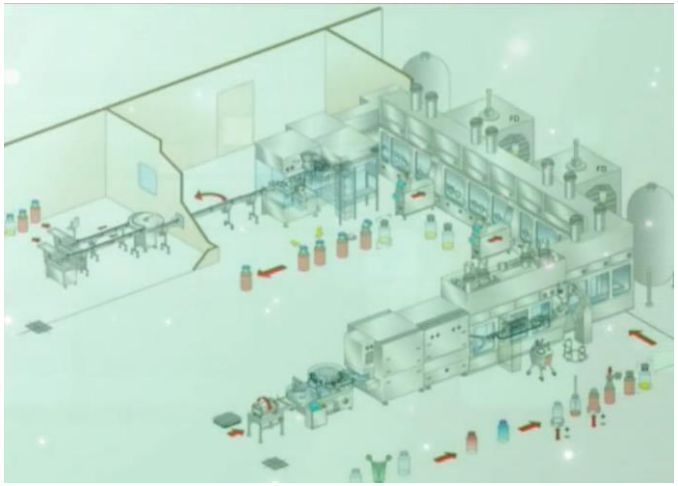
60% no effect Within patients with designated gen mutation

40% of patients with designated gen mutation show effect

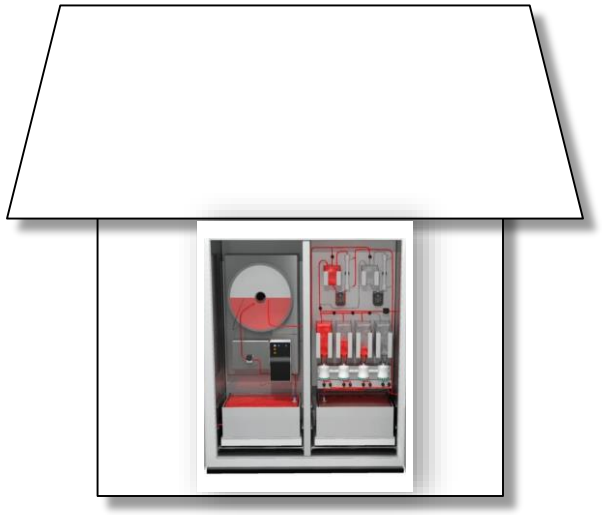
Under ”SMP” Companion diagnostic is compulsory for OD

# New technologies for small scale production could facilitate personalised medicine at low cost

***From large scale production***



***To small scale production***



# We will hit the wall…. The ‘SMP’ can help us to ensure accessibility and sustainability