

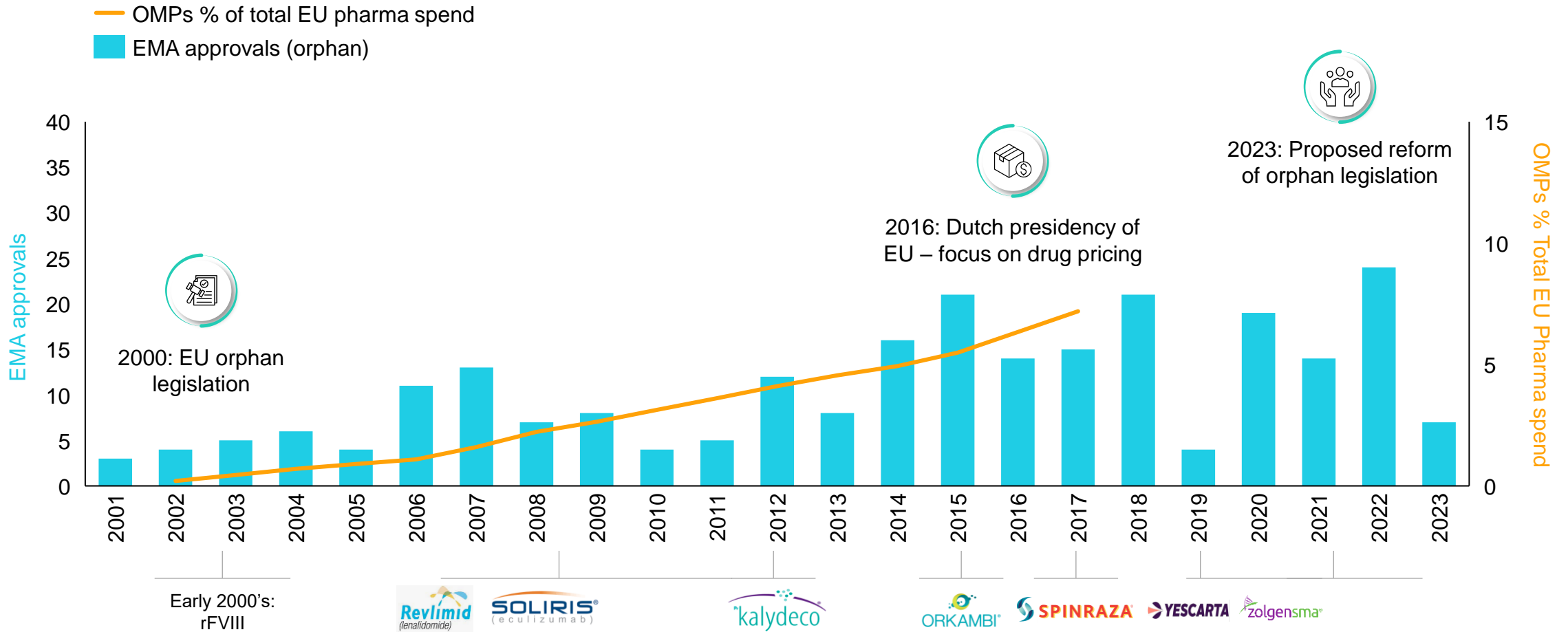
DOLON

# Exploring alternative pricing approaches for orphan medicines

Richard Sear & Emilie Neez, Dolon Ltd

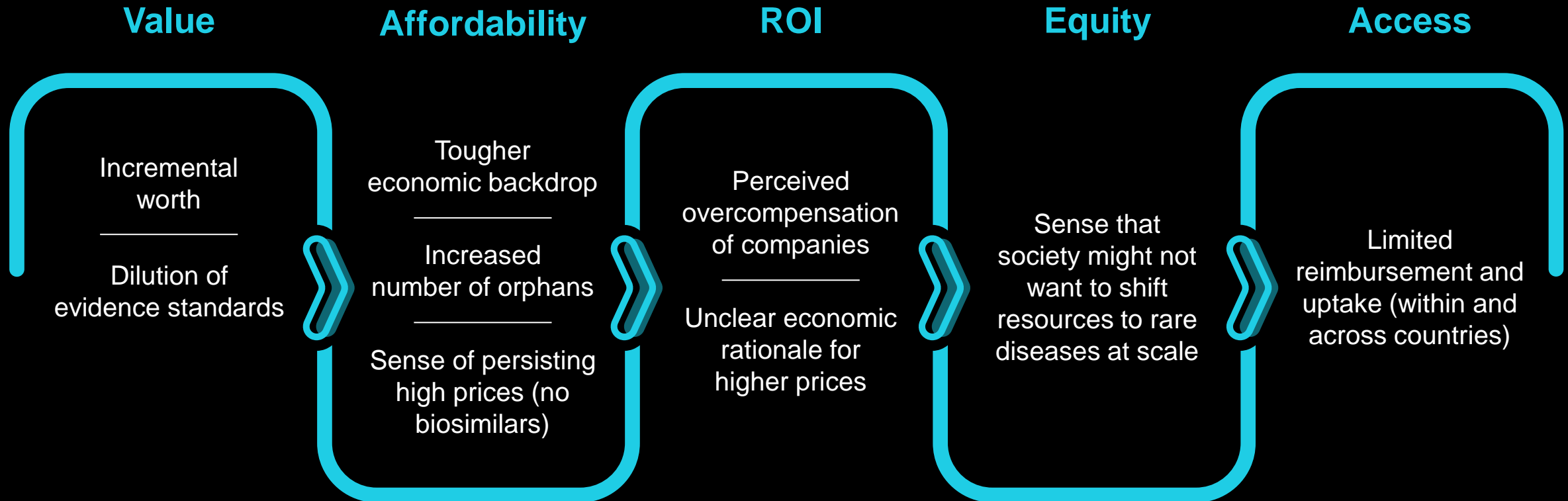
*All opinions our own*

# Orphan medicines in Europe: 20+ years on



Sources: EMA website ([Available here](#)); Mestre-Ferrandiz et al. 2019 ([Available here](#))

# What are some of the concerns put forward by payers & policymakers around orphan medicines?



Price is relevant to all these concerns

# Industry has concerns about pricing trends across Europe and support for innovation



**With the pricing situation 'untenable' in Europe, Bluebird will wind down its operations in the 'broken' market**

Bayer AG [+ Add to myFT](#)

Bayer shifts pharma focus away from 'innovation unfriendly' Europe

**Sanofi Chief Issues Warning To Innovation-Unfriendly EU**

02 Feb 2024 | NEWS

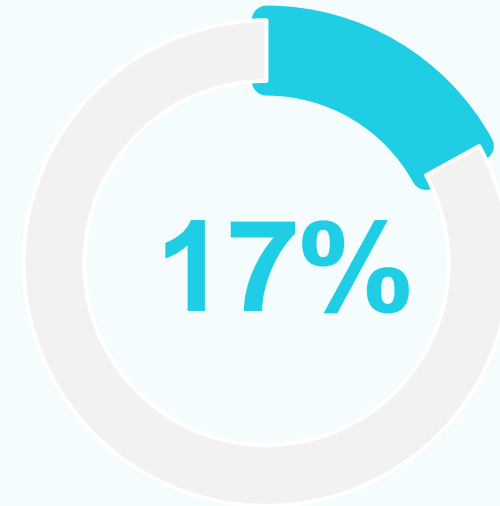
STORY - Tuesday, 07 March 2023 - 09:39 GMT

**BMS says not launching Opdualag for melanoma in Germany because of unfavourable AMNOG changes**

Sustainable innovation

**Estimated % OMPs (2000-2020) economically viable in Europe<sup>1</sup>**

Estimated rNPV - €6.7 million

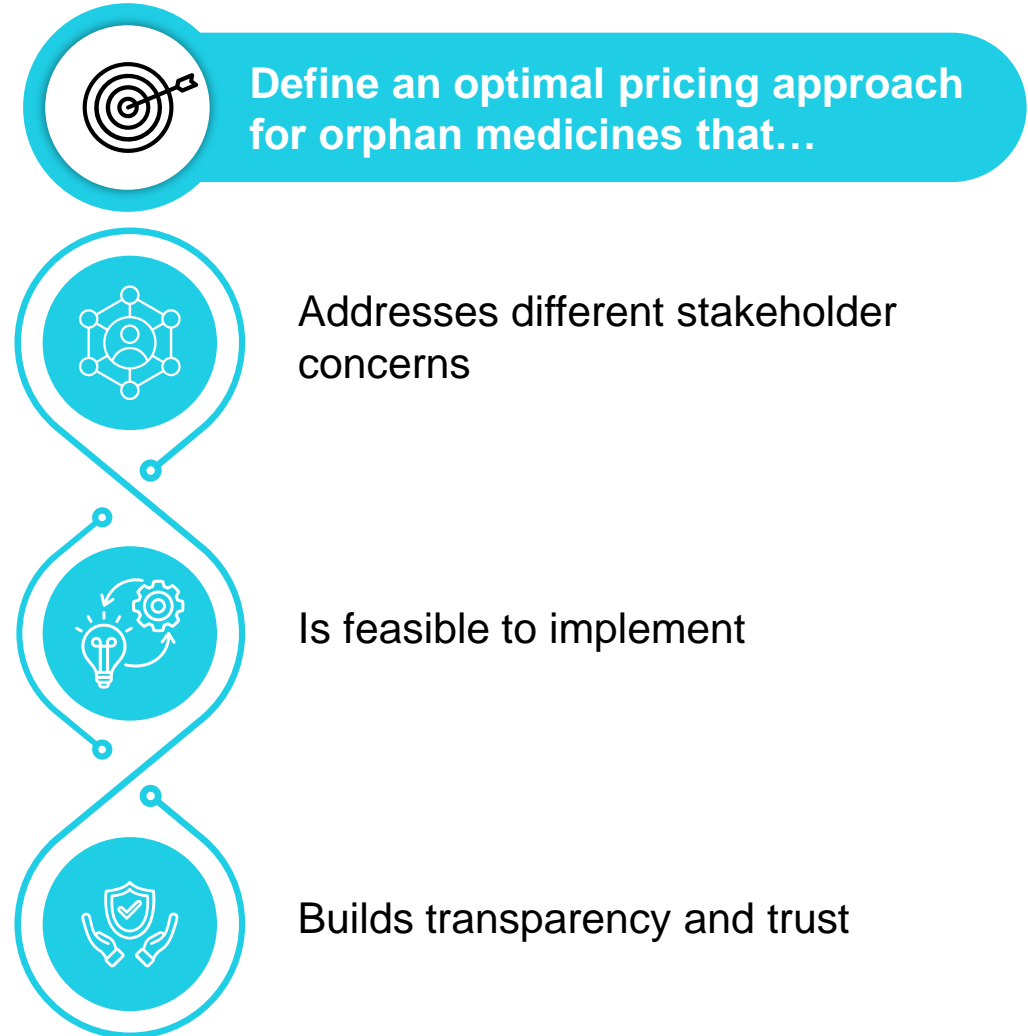


Sources: Financial Times; FiercePharma; APMHealth Europe; Dolon 2023

# We worked with 10 orphan pricing experts to explore innovative pricing approaches



**Workshop with 10 pricing experts  
from companies with orphan medicines**



# We drew from existing and proposed pricing approaches

## 01. Inspiration

## 02. Deconstruction



OHE  
competition (2022)



Literature

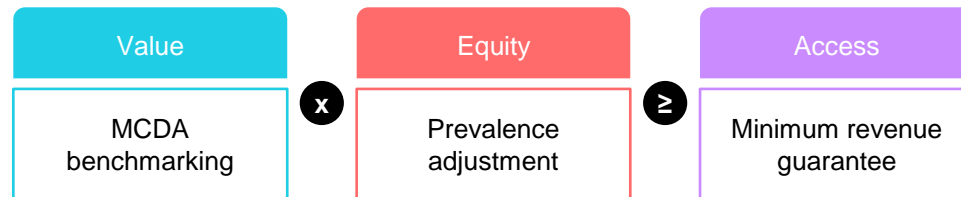


Existing payer  
frameworks

### Framework 1



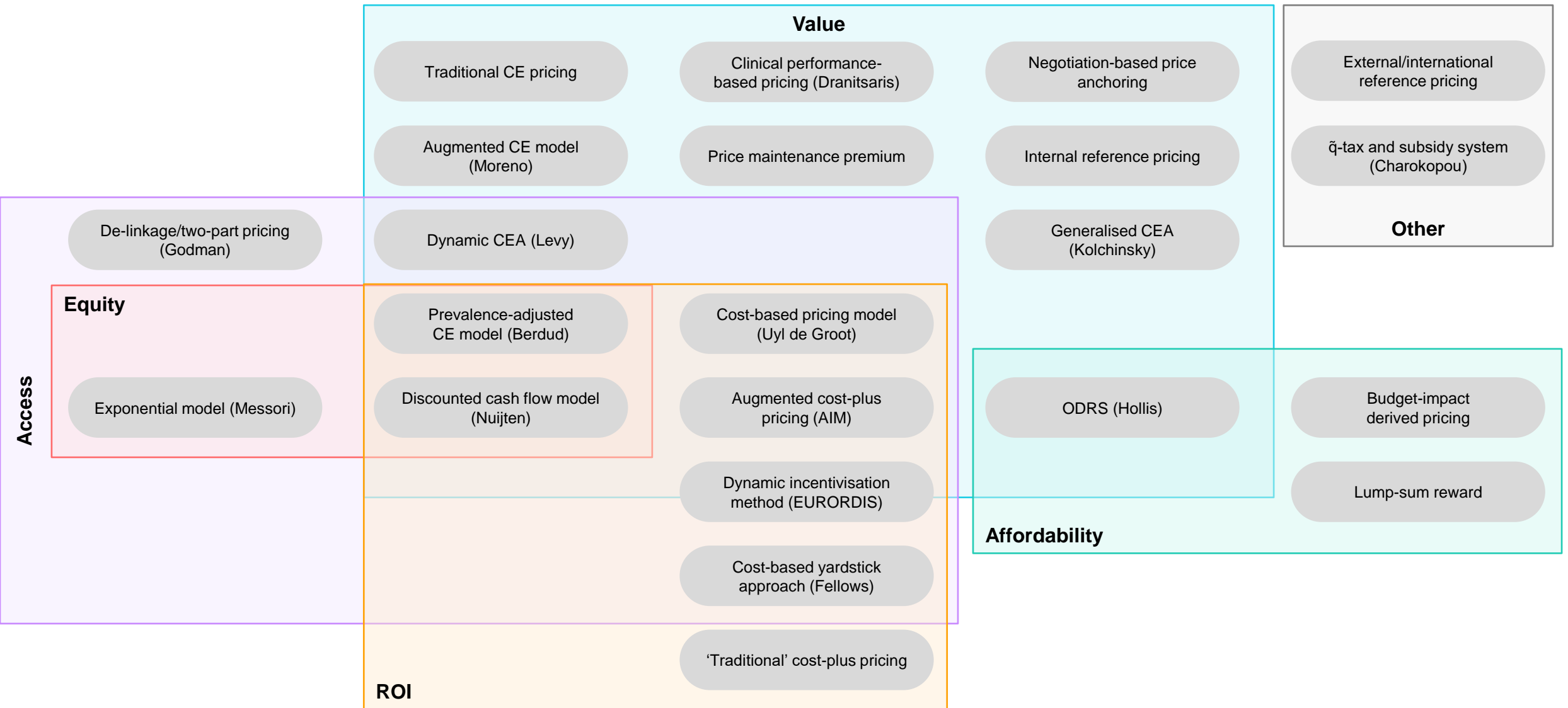
### Framework 2



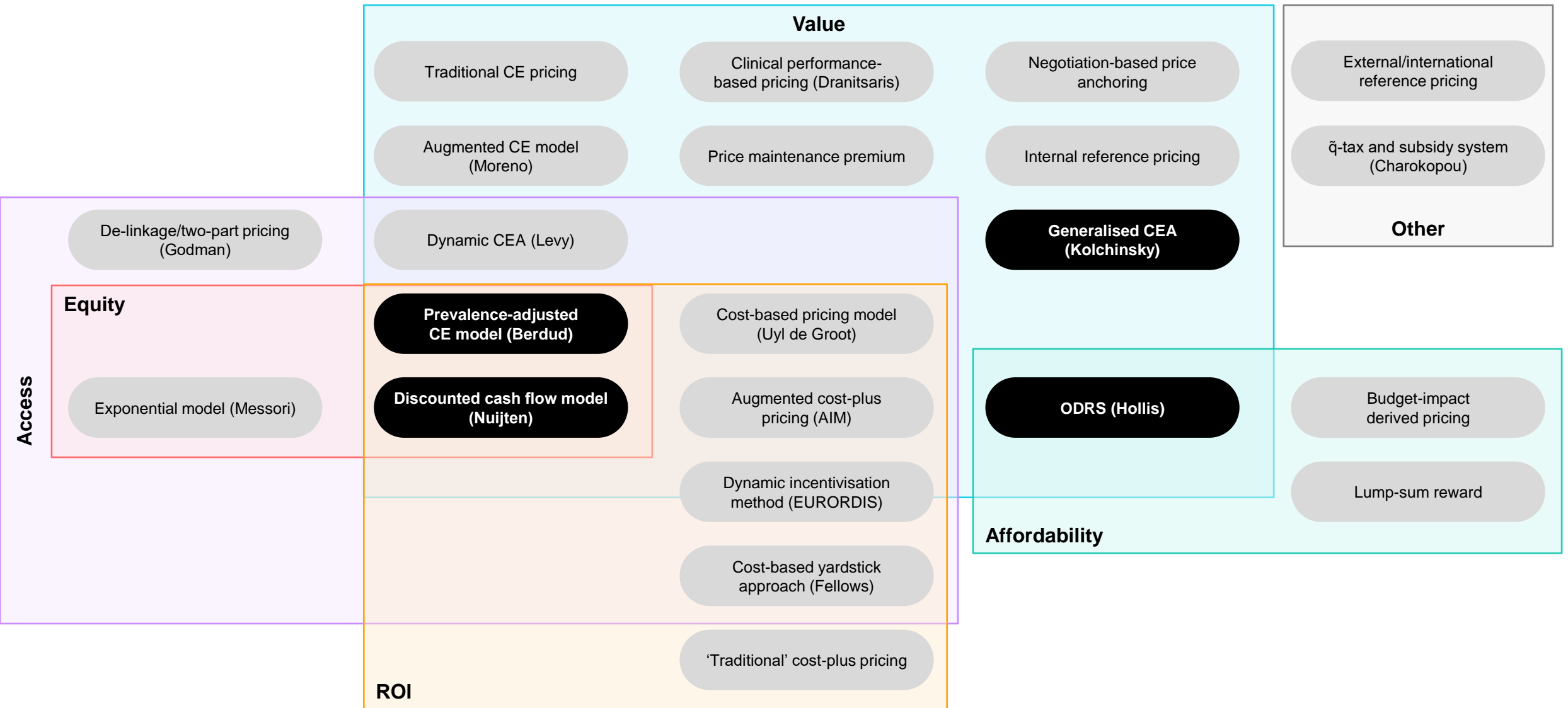
### Framework 3



# There are lots of interesting pricing ideas beyond the status quo



# Today we would like to zoom in on four novel pricing approaches





## Marketing authorisation for a new medicine

01

**Business as usual**  
(value-based pricing)

E.g., 80% of pharmaceutical budget

02

**Delinked Reward System**  
Payment divided into two separate parts

**Price per unit**  
(immediate reimbursement)

+

**Reward for innovation**  
(for first 10 years)

E.g., 20% of budget

Requires **cost transparency**

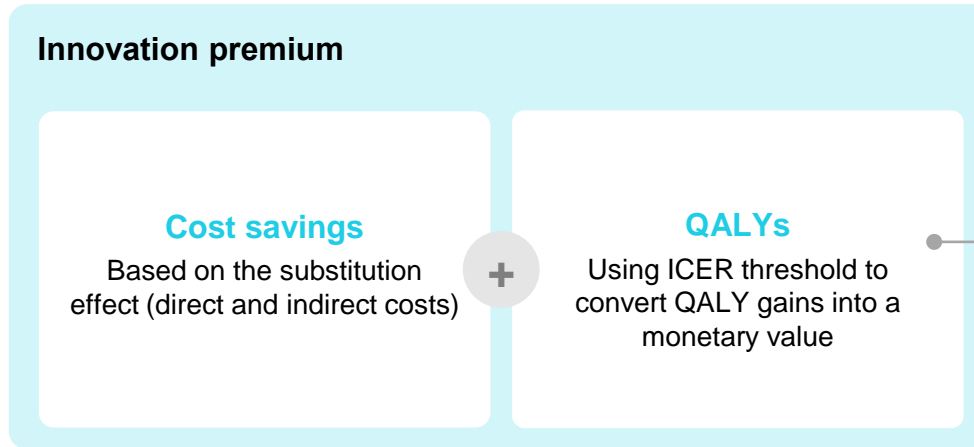
**Indication-based pricing** would be possible via the reward

Funding is capped

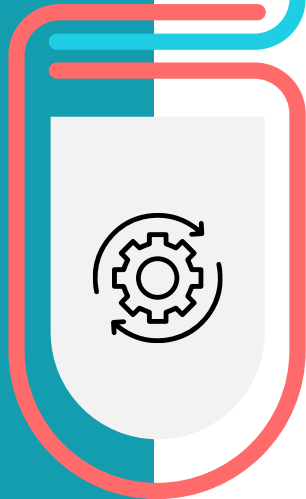
Source: Hollis 2022 ([Available here](#))



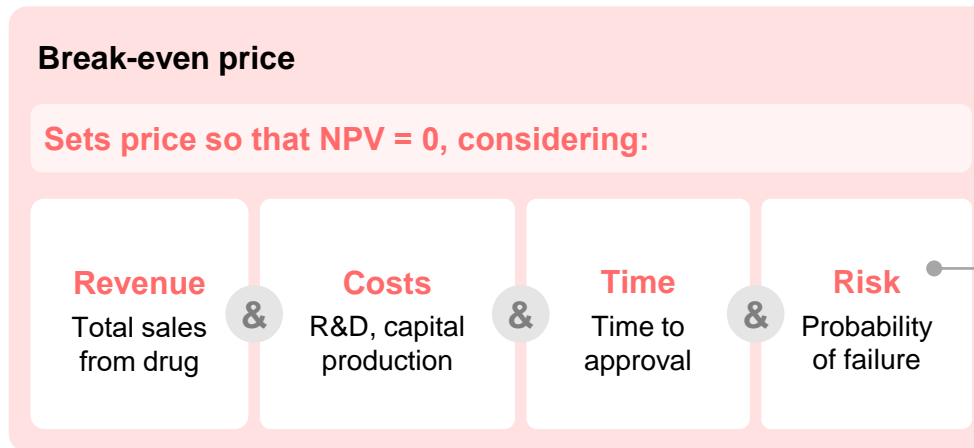
## Upper price limit



Model applies only to first-in-class medicines



## Lower price limit



Based on industry averages

Source: Nuijten 2018 ([Available here](#)); Nuijten 2020 ([Available here](#))



**Upper price limit**



**Lower price limit**

## Innovation premium

### Cost savings

Based on the substitution effect (direct and indirect costs)

+

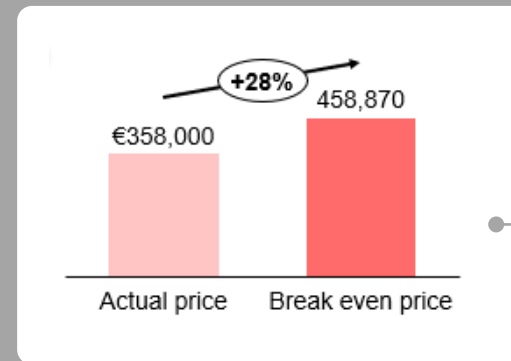
### QALYs

Using ICER threshold to convert QALY gains into a monetary value

Model applies only to first-in-class medicines

### Example

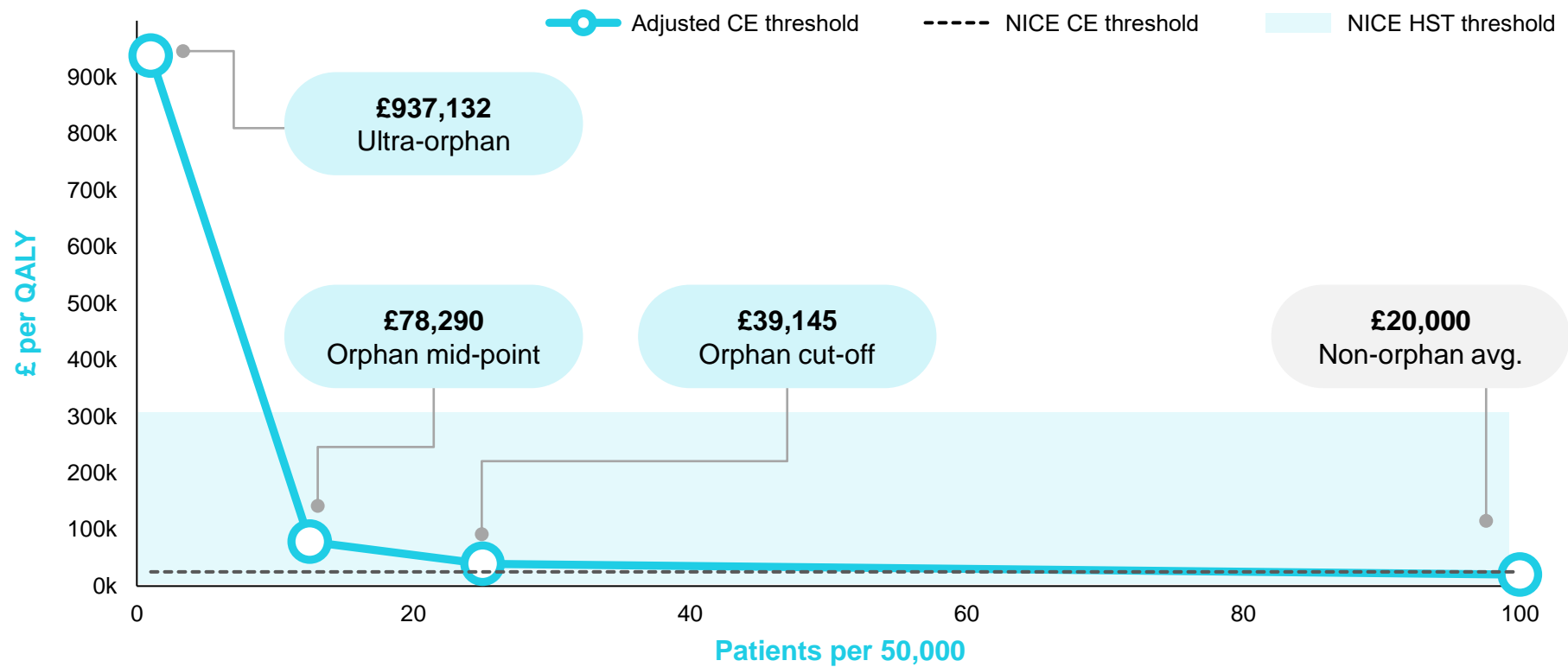
**Break-even price for Soliris in the Netherlands**



Based on industry averages

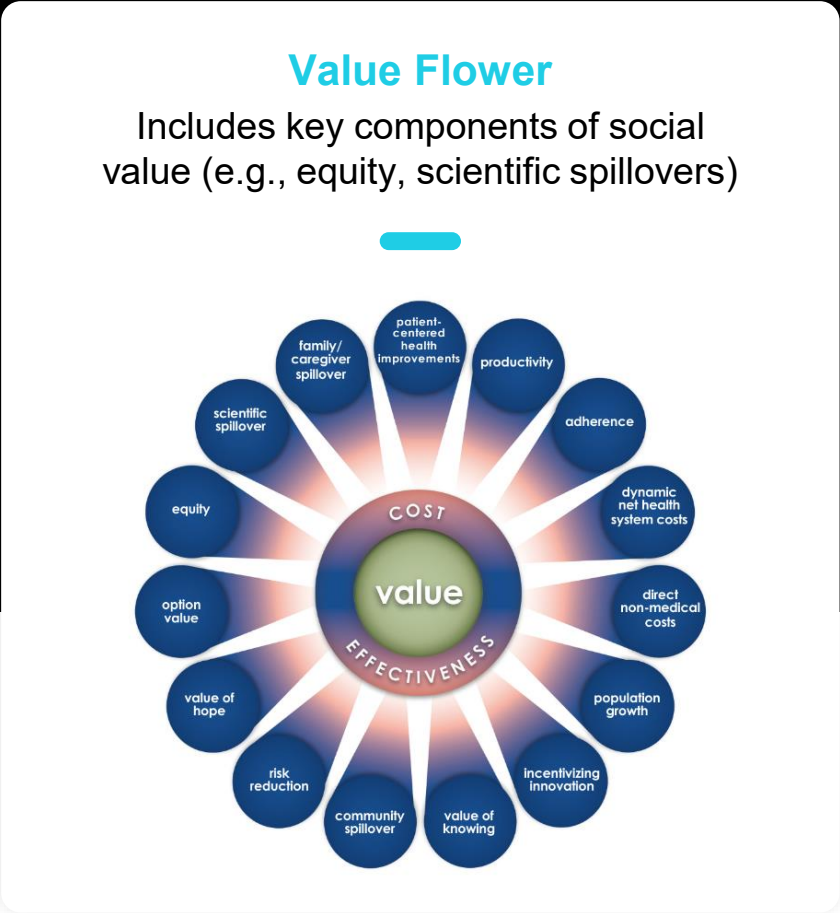
Source: Nuijten 2018 ([Available here](#))

## ICER thresholds that equalise R&D incentive between rare and non-rare diseases<sup>1</sup>



Source: Berdud et al. 2020 ([Available here](#))

## Generalised CEA incorporates additional elements compared to traditional CEA

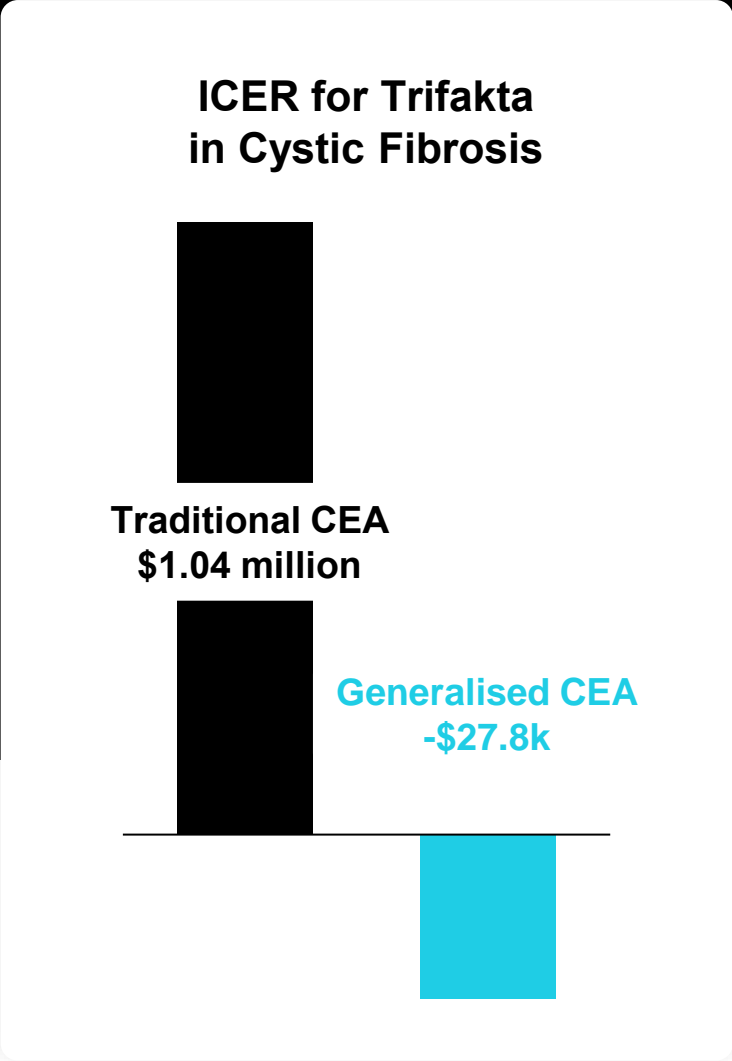


### Disease severity

Accounting for heightened value when treating patients with permanent disabilities or severe, acute diseases

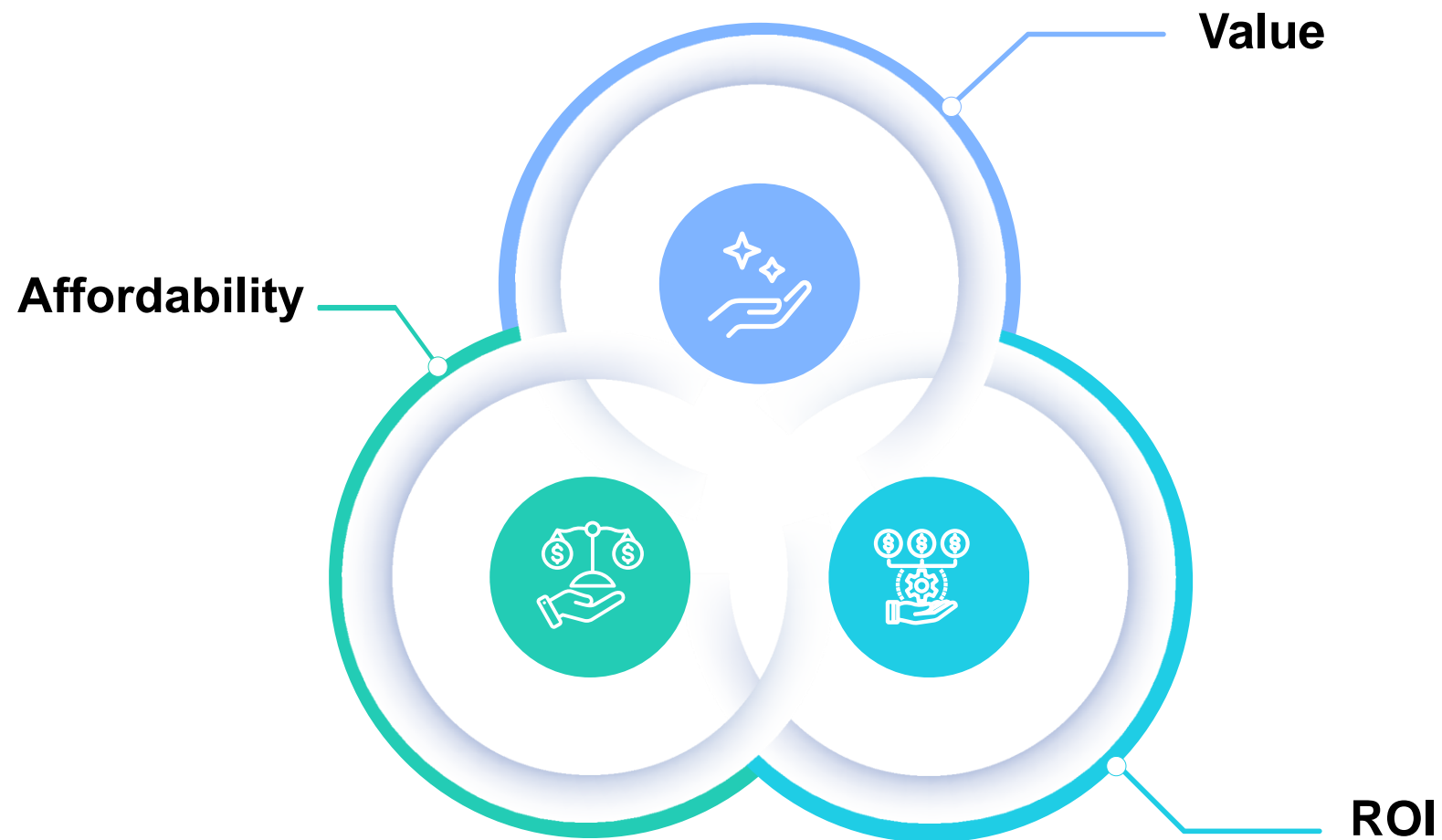
### Dynamic pricing

Accounting for savings that occur due to declining prices over the life-cycle of a drug (e.g., after LoE)



Source: Entity Risk for No Patient Left Behind 2023 ([Available here](#))

There are three domains that everyone agreed are critical to balance in pricing frameworks...



# Where do we go from here?

It is well acknowledged that prices for orphan medicines need to be higher than non-orphans to incentivize innovation in rare diseases – but there is no agreed framework for how to factor in rarity or innovation economics into pricing

Industry has historically been very shy talking about anything beyond value-based pricing.  
By only focusing on value, we risk missing out on a crucial part of the debate around orphan drug pricing

Other stakeholders are moving ahead and proposing what is sustainable / affordable  
Important to engage in fact-based multi-stakeholder discussions

We encourage exploring some of these alternative pricing frameworks when defining price

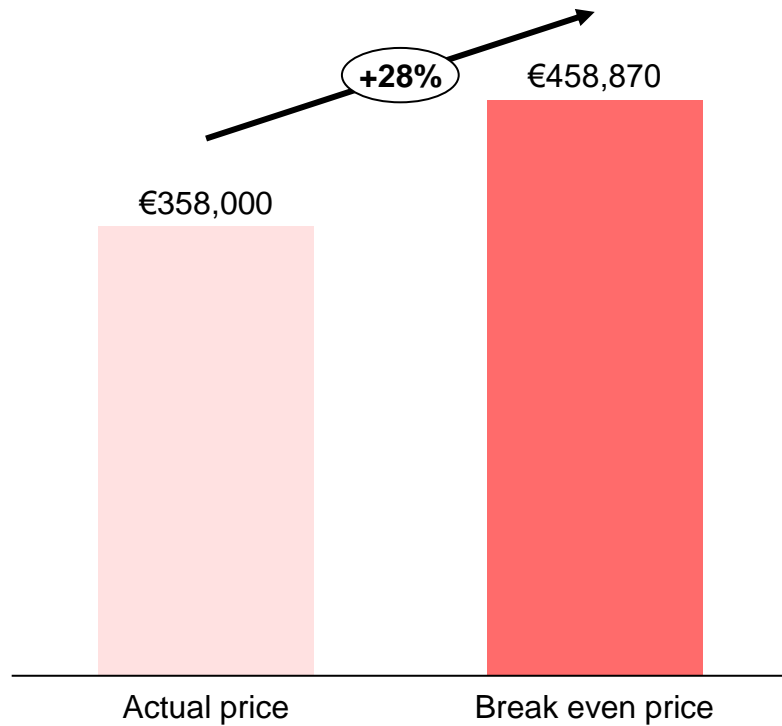
**Thank you!**



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# Appendix

## Actual vs. break-even price for Soliris in the NL



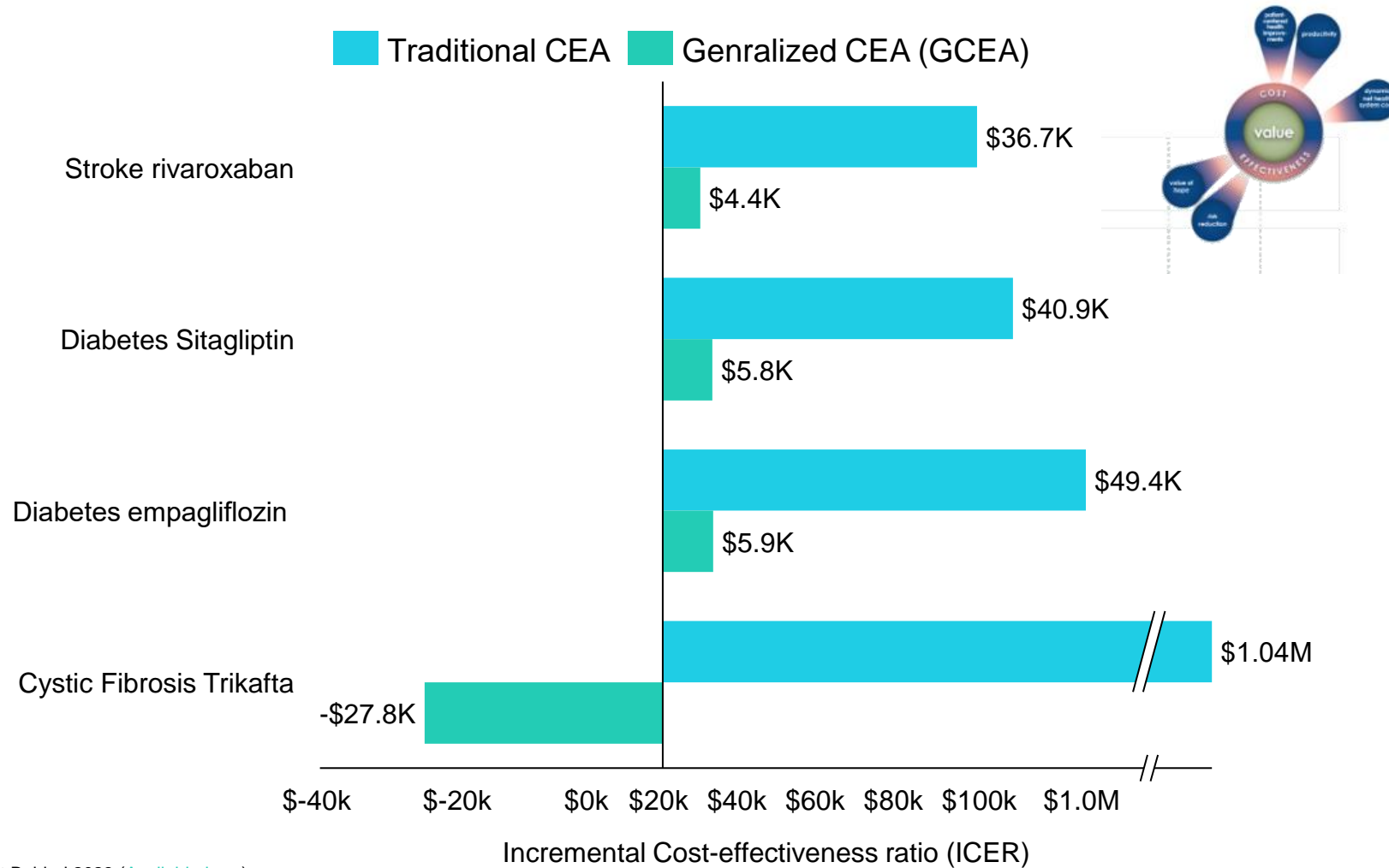
Source: Nuijten 2018 ([Available here](#))

## Base case

|                                      |                    |     |
|--------------------------------------|--------------------|-----|
| Minimum acceptable rate of return    | 12%                |     |
| Cost of revenue                      | 40%                |     |
| Cost of development                  | US\$701 million    |     |
| Years of development and approval    | 8 years            |     |
| Time to reimbursement                | 1 year             |     |
| Net patent period after registration | 12 years           |     |
| Uptake                               | 80% from year 1    |     |
| Probability of failure               | Phase I-II         | 30% |
|                                      | Phase II-III       | 61% |
|                                      | Phase III-approval | 31% |

Most inputs are non-orphan, industry averages

# Applying the Generalised CEA to selected medicines



Source: No Patient Left Behind 2023 ([Available here](#))