DOLON

Exploring alternative pricing approaches for orphan medicines

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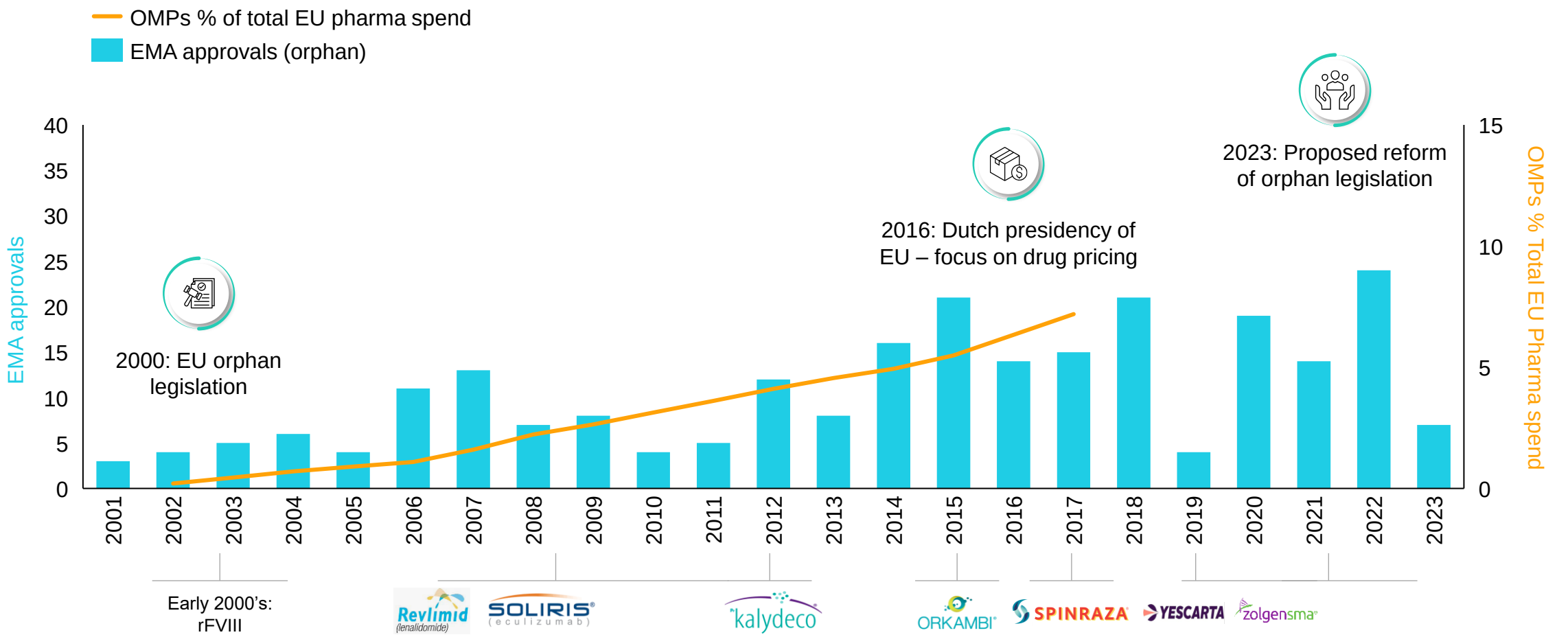
All opinions our own

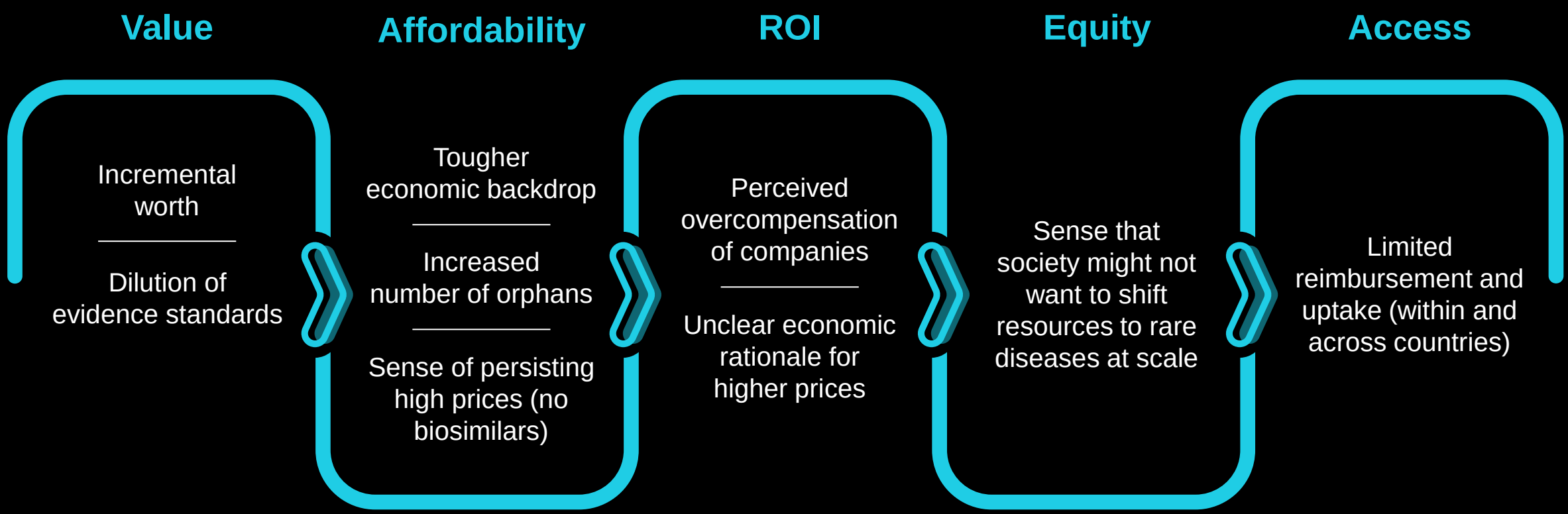
March 2024 – World EPA Congress, Amsterdam

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[1]

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

Define an optimal pricing approach for orphan medicines that…

- Addresses different stakeholder concerns
- Is feasible to implement
- Builds transparency and trust

We drew from existing and proposed pricing approaches

01. Inspiration

OHE competition (2022)

Literature

Existing payer frameworks

02. Deconstruction

Framework 1

Value		Affordability
QALY	x	ICER threshold

Value		Equity		Affordability
QALY	x	Prevalence adjustment	+/-	Price volume incentive

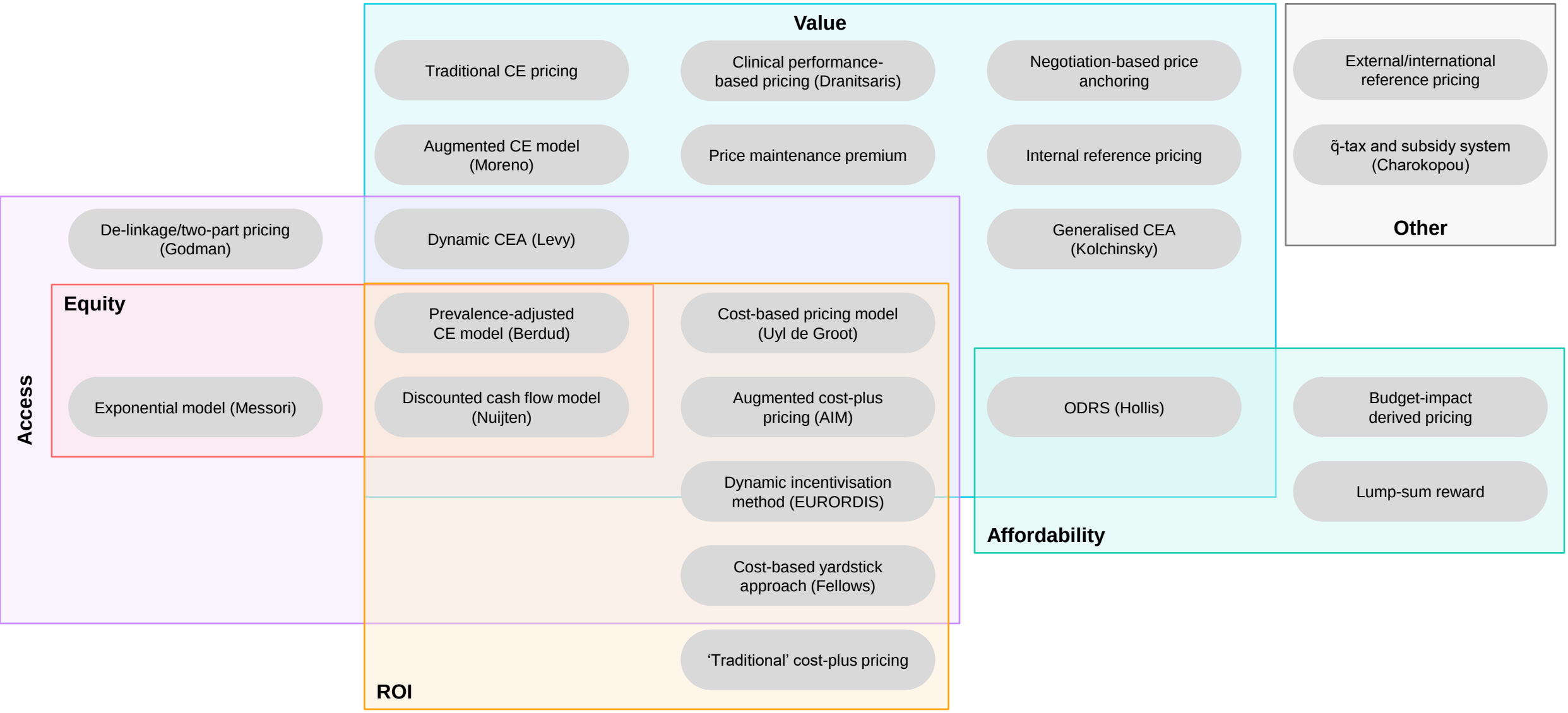
Framework 2

Value		Equity		Access
MCDA benchmarking	x	Prevalence adjustment	≥	Minimum revenue guarantee

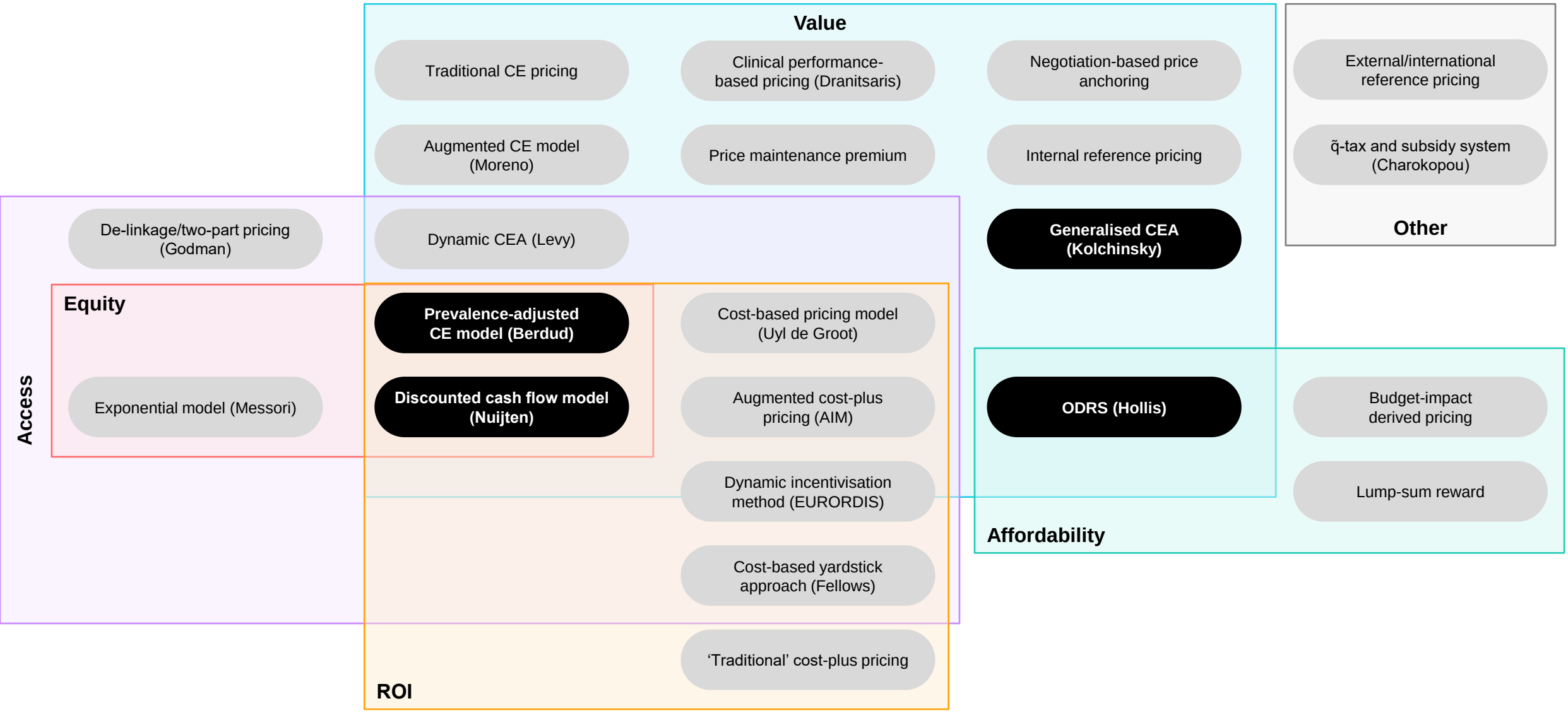
Framework 3

ROI		Value		Affordability
Sustainable innovation benchmark	x	Unmet need weighting	+/-	Price volume incentive

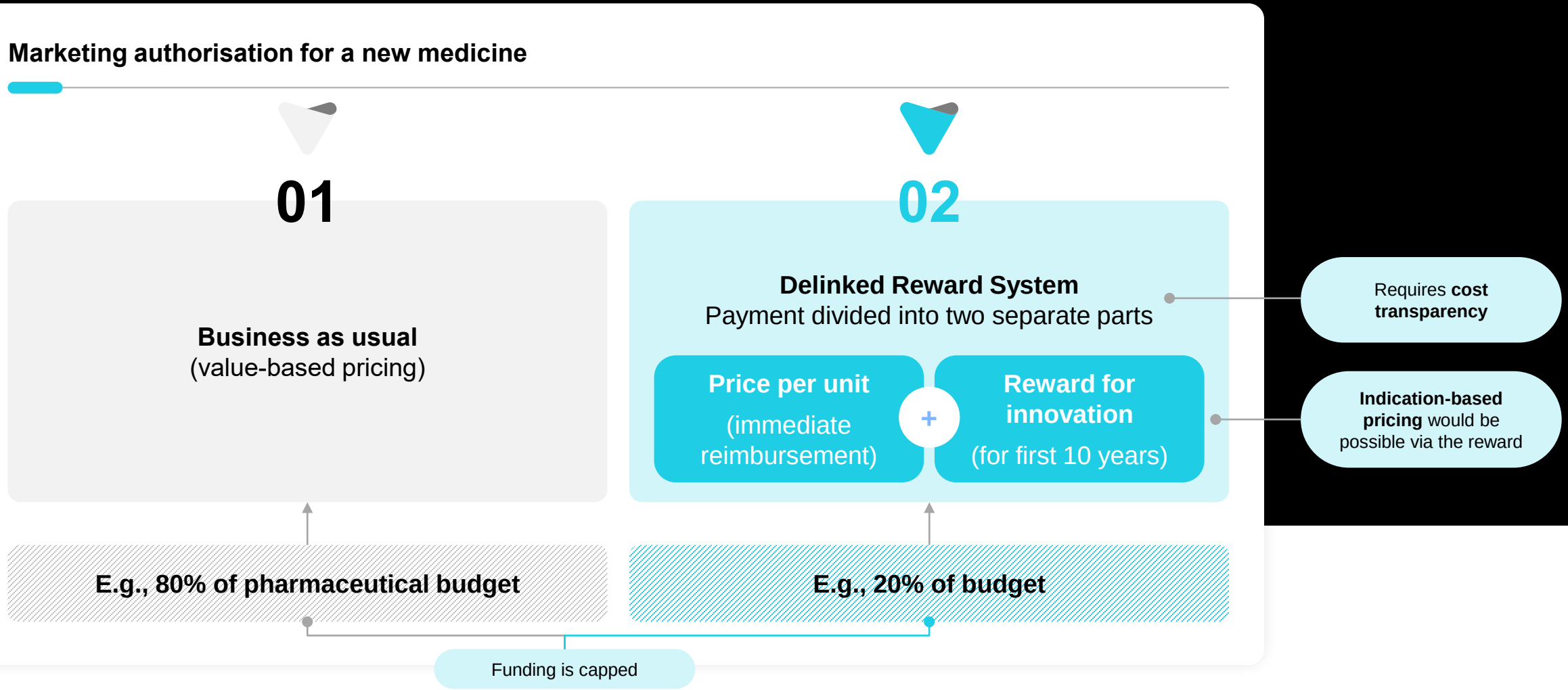
There are lots of interesting pricing ideas beyond the status quo



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

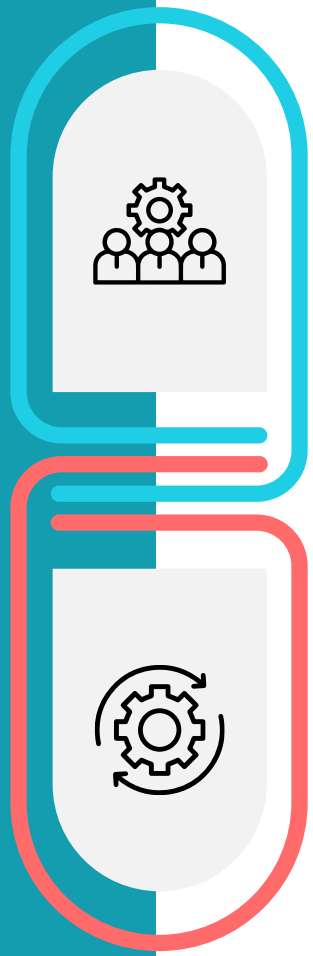


The Optional Delinked Reward system • *Hollis*



Source: Hollis 2022 (Available here)

Discounted cash flow model • *Nuijten et al.*



Upper 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

Lower price limit

Break-even price

Sets price so that NPV = 0, considering:

Revenue
Total sales from drug

&

Costs
R&D, capital production

&

Time
Time to approval

&

Risk
Probability of failure

Based on industry averages

Source: Nuijten 2018 (Available here); Nuijten 2020 (Available here)

Discounted cash flow model • *Nuijten et al.*

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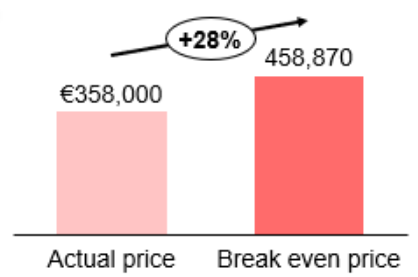
Lower price limit

Example

Break-even price for Soliris in the Netherlands

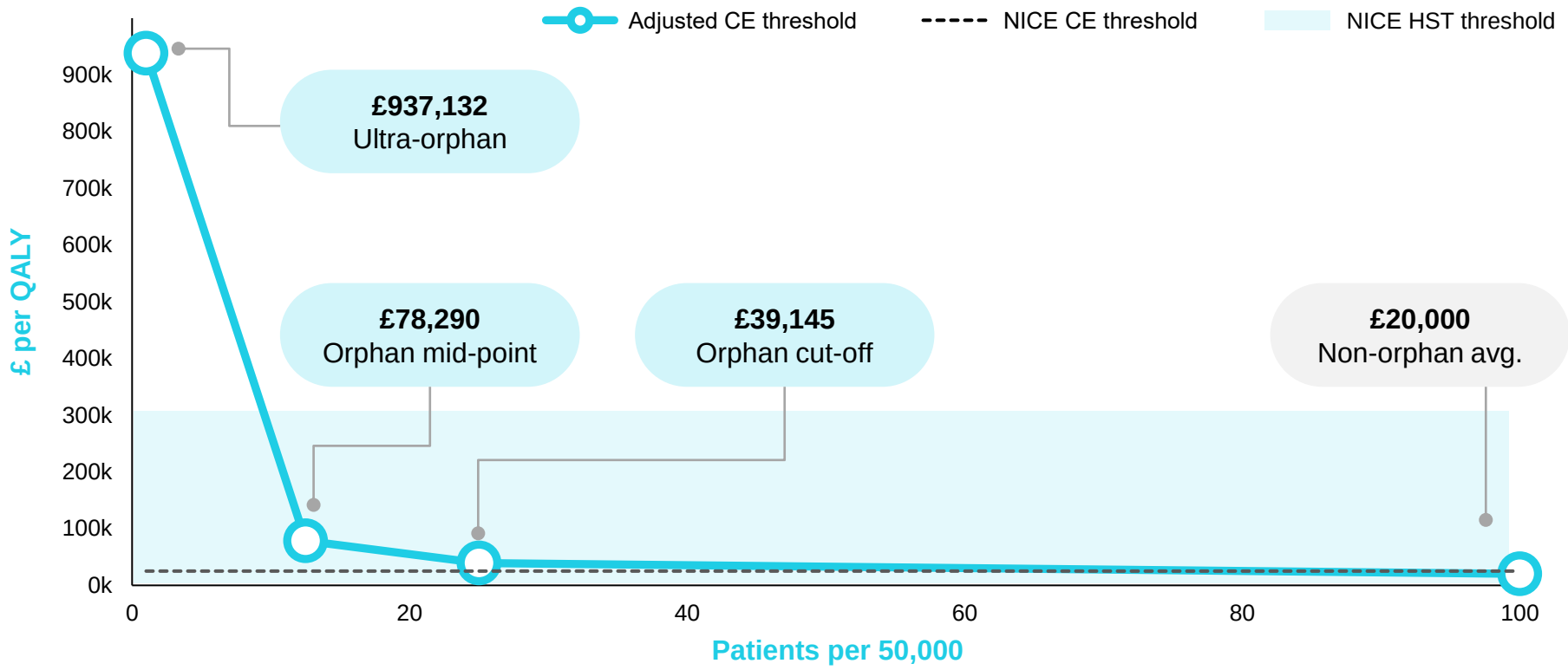


Based on industry averages

Source: Nuijten 2018 (Available here)

Modified ICER approach • *Berdud et al.*

ICER thresholds that equalise R&D incentive between rare and non-rare diseases[1]



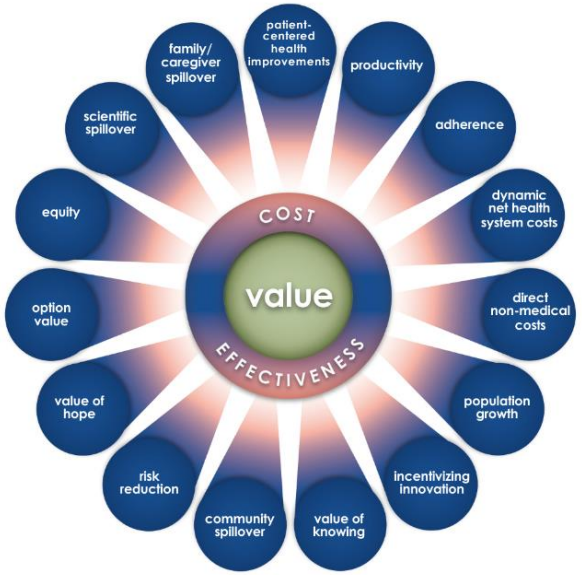
Source: Berdud et al. 2020 (Available here)

Generalised CEA • *Kolchinsky and colleagues*

Generalised CEA incorporates additional elements compared to traditional CEA

Value Flower

Includes key components of social value (e.g., equity, scientific spillovers)



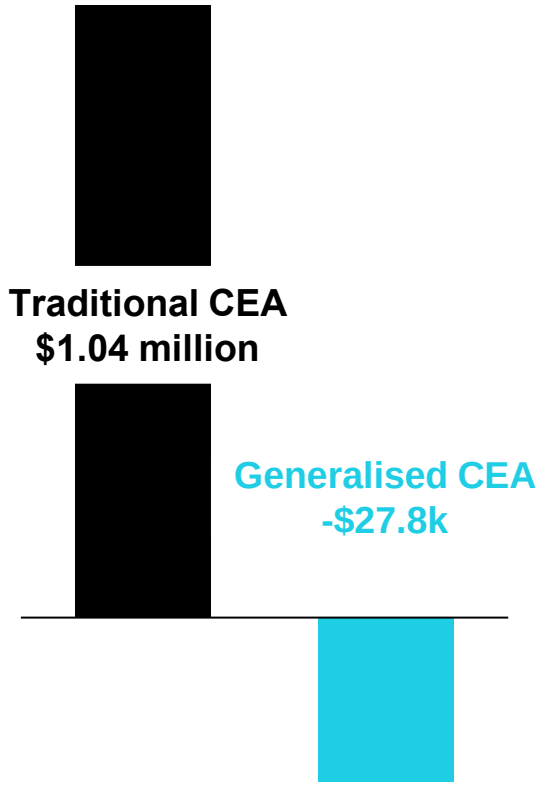
Disease severity

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

Dynamic pricing

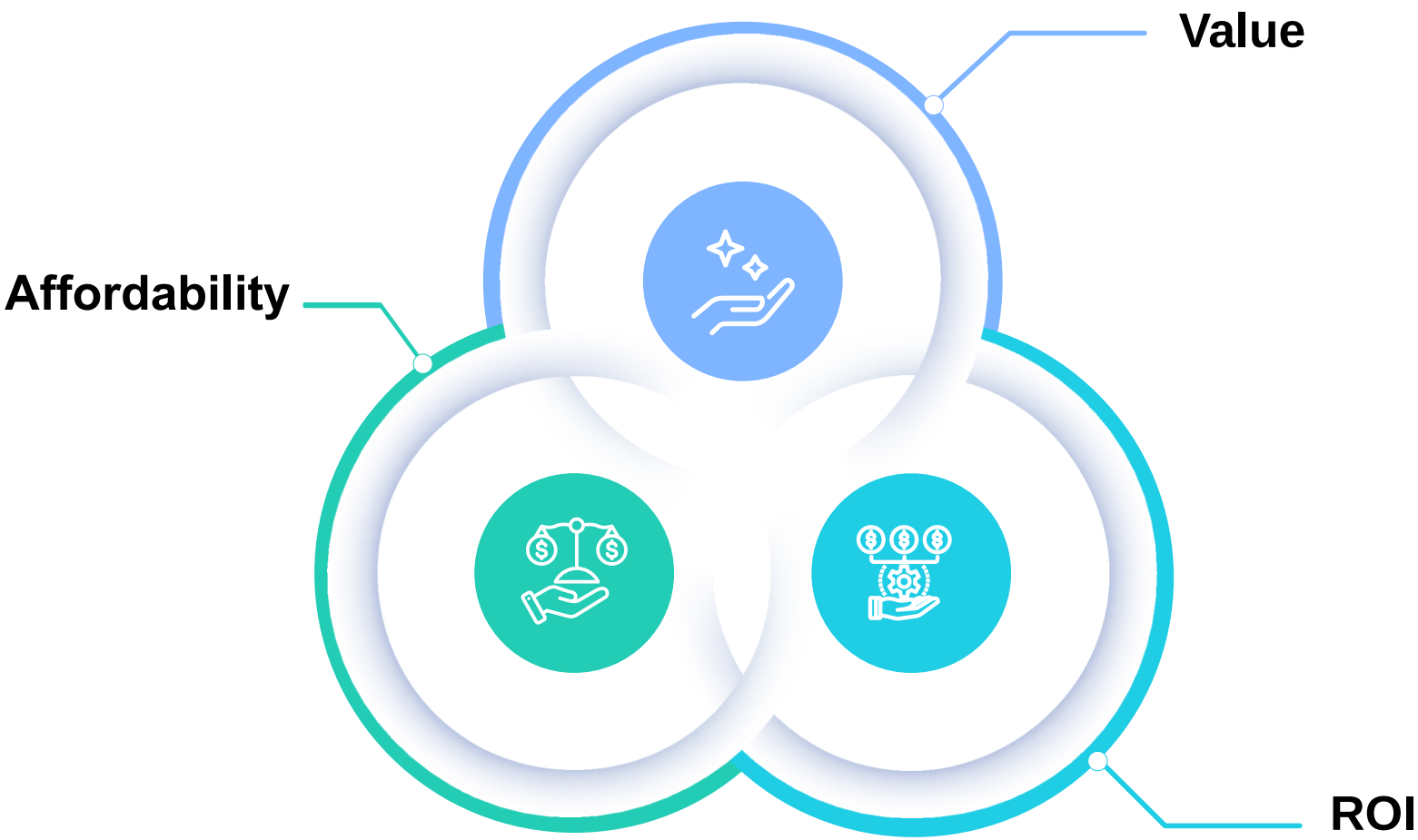
Accounting for savings that occur due to declining prices over the life-cycle of a drug (e.g., after LoE)

ICER for Trifakta in Cystic Fibrosis



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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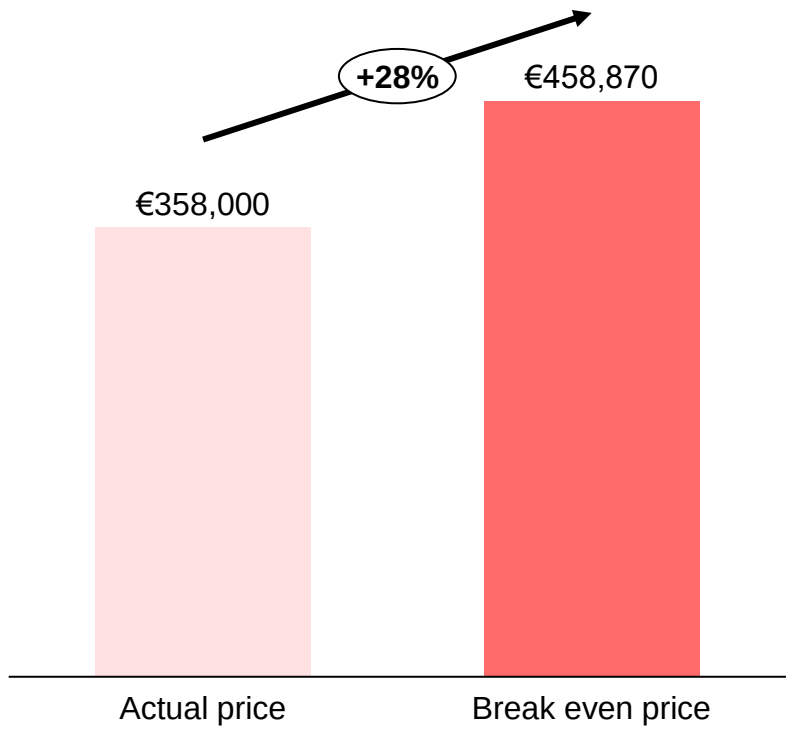
Appendix

Applying the discounted cash flow model to Soliris in the Netherlands

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



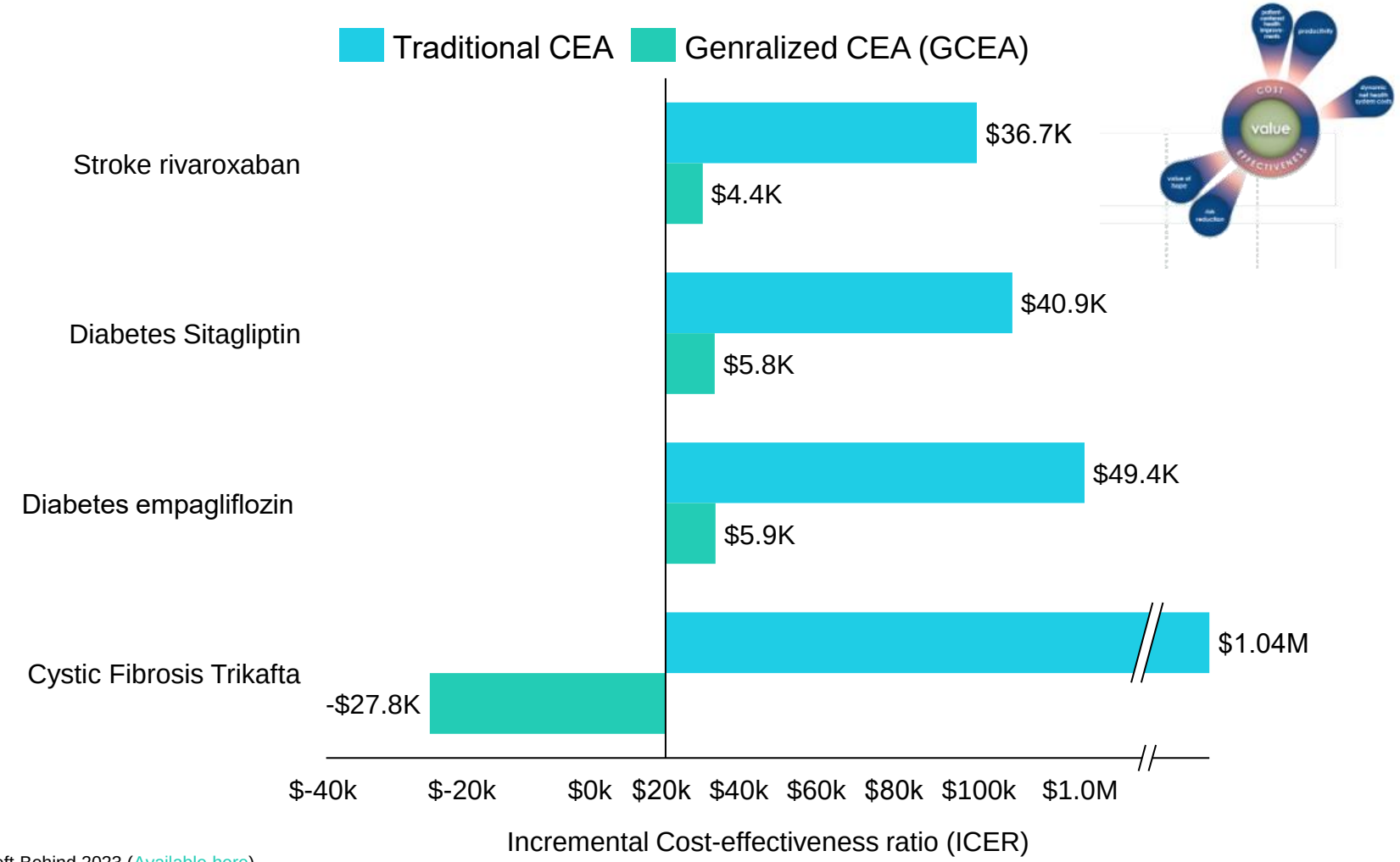
Base case

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

Source: Nuijten 2018 (Available here)

Applying the Generalised CEA to selected medicines



Source: No Patient Left Behind 2023 (Available here)