# Budget Impact Modeling of Cell and Gene Therapies

## Evaluating Payment Models and Real-World Effectiveness in Gene Therapy Budgeting

Varun Ektare
Co-founder – Indence Health

ISPOR EU Annual Conference, Barcelona
November 19, 2024

# Outline

01

Challenges of traditional financing of gene therapy and alternate payment models considered

02

Budget impact analysis under different payment models

03

Short-term and medium-term performance of payment mechanisms

## Unique Challenges with Gene Therapy Budgeting

***High Upfront Costs:*** *Many gene therapies involve a large one-time upfront treatment costs*

***Growing indication Sizes:*** *Gene therapies brought to the market lately have larger indications compared to previous therapies ( e.g. sickle cell disease)*

***Advent of Genomic revolution:*** *More that 1200 cell and gene therapies currently in trials.*

***Uncertain Long-Term Effectiveness:*** *While clinical trials show promising results, real-world effectiveness may vary. Payers face uncertainty regarding whether these treatments will maintain their benefits over time.*

*Poses significant risk and financial burden for healthcare systems and insurers.*

***Need for Innovative Payment Models:*** *Traditional payment models are often insufficient for one-time, high-cost therapies. There's a growing need for payment structures that spread costs or adjust payments based on real-world outcomes to make these therapies financially sustainable.*

# It’s time for a poll

**What do you see as the biggest challenge in funding high-cost gene therapies?**

a) High upfront cost
b) Growing indication sizes
c) Advent of genomic revolution
d) Uncertain long-term effectiveness

# Key payment models for high-cost gene therapies

## Simple Discount

*Overview:* A one-time discount applied to the therapy's upfront cost[1]

*Purpose:* Lowers initial financial burden by reducing the therapy's list price.

*Considerations:* Offers immediate cost savings but doesn't adjust if therapy effectiveness varies over time.

## Amortization/ Annuity-Based Payment

*Overview:* The therapy cost is spread over several years in installments[1,2]

*Purpose:* Reduces immediate budget impact and distributes costs, helping payers manage high upfront expenses.

*Considerations:* Financially sustainable if the therapy provides sustained benefits, but can be challenging if outcomes decline over time.

## Outcomes-Based Contract

*Overview:* Payment is linked to patient outcomes, with total payment contingent on real-world effectiveness[1,3]

*Purpose:* Aligns costs with value, paying only if the therapy achieves agreed-upon health outcomes.

*Considerations:* Reduces financial risk for payers, especially if real-world results don't match clinical trials, but requires tracking of patient outcomes.


1. Horrow, Caroline, and Aaron S. Kesselheim. "Confronting high costs and clinical uncertainty: innovative payment models for gene therapies: study examines costs, clinical uncertainties, and payment models for gene therapies." Health Affairs 42.11 (2023): 1532-1540.
2. DeMartino, Patrick, et al. "A budget impact analysis of gene therapy for sickle cell disease: the Medicaid perspective." JAMA pediatrics 175.6 (2021): 617-623.
3. Jørgensen, Jesper, Eve Hanna, and Panos Kefalas. "Outcomes-based reimbursement for gene therapies in practice: the experience of recently launched CAR-T cell therapies in major European countries." Journal of market access & health policy 8.1 (2020): 1715536.

# It’s time for a poll

**Which model would you consider the most viable for your context**

a) Simple discount
b) Annuity based payment
c) Outcomes based contract

# We built an illustrative BIM to test the budget impact of a hypothetical gene therapy with the alternative payment models

# Model inputs and assumptions

**Epidemiology**

- Only the prevalent population in current year considered for simplicity
- Gene therapy assumed to cure patients fully of the disease, taking them out of the at-risk population in the next year

**Market share**

- Standard of care assumed to have 100% of market share pre-launch of gene therapy
- Gene therapy assumed to take up 10% market share of the eligible prevalent population

**Costs**

- SoC assumed to cost EUR 50,000 per year
- Gene therapy assumed to incur a one-time cost of EUR 1.8 million
- Only pharmacy costs are included for simplicity

Estimation of target population eligible for gene therapy

| Parameter | Input |
|---|---|
| Country population | 68,000,000 |
| Prevalence of disease | 0.0125% |
| **No. of patients eligible for gene therapy in year 1** | **8,500** |

Population distribution pre and post gene therapy launch

| Current scenario | 2025 | 2026 | 2027 | 2028 | 2029 |
|---|---|---|---|---|---|
| Gene therapy | 0 | 0 | 0 | 0 | 0 |
| Standard of care | 8,500 | 8,500 | 8,500 | 8,500 | 8,500 |
| **Reference scenario** | **2025** | **2026** | **2027** | **2028** | **2029** |
| Gene therapy | 850 | 765 | 689 | 620 | 558 |
| Standard of care | 7,650 | 6,885 | 6,197 | 5,577 | 5,019 |

# Payment model related inputs

## Simple discount

Apply **a 50% discount** on the one time payment of drug acquisition cost (such large discounts may come with volume guarantees)

## Annuity payment model

% drug acquisition cost paid annually in **five installments** as follows[1]

- Year 1 – 5%
- Year 2 – 10%
- Year 3 – 15%
- Year 4 – 25%
- Year 5 – 45%

## Outcomes-based payments

% price reduction based on outcomes (assuming outcomes worse than clinical trials)[1]

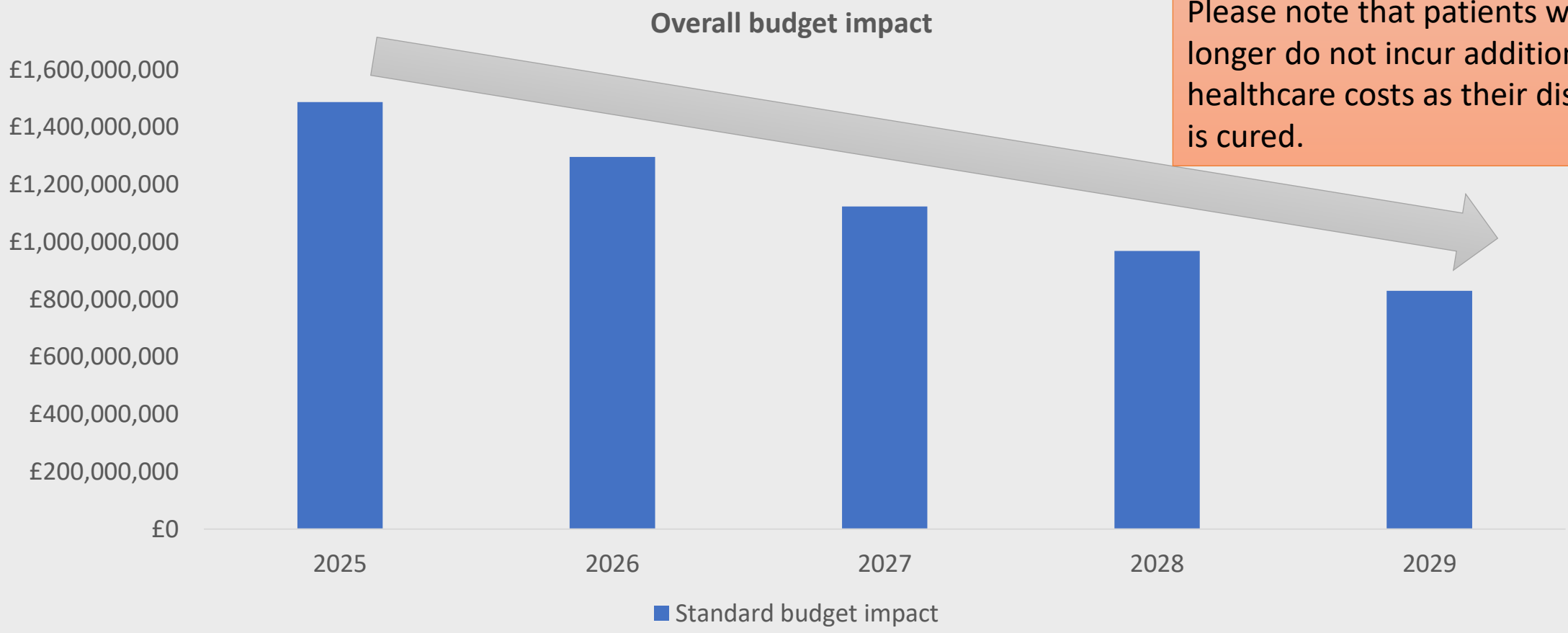
- Year 1 – 0%
- Year 2 – 10%
- Year 3 – 15%
- Year 4 – 20%
- Year 5 – 25%

Temporal pattern where average discount goes up each year as more and more patients lose response

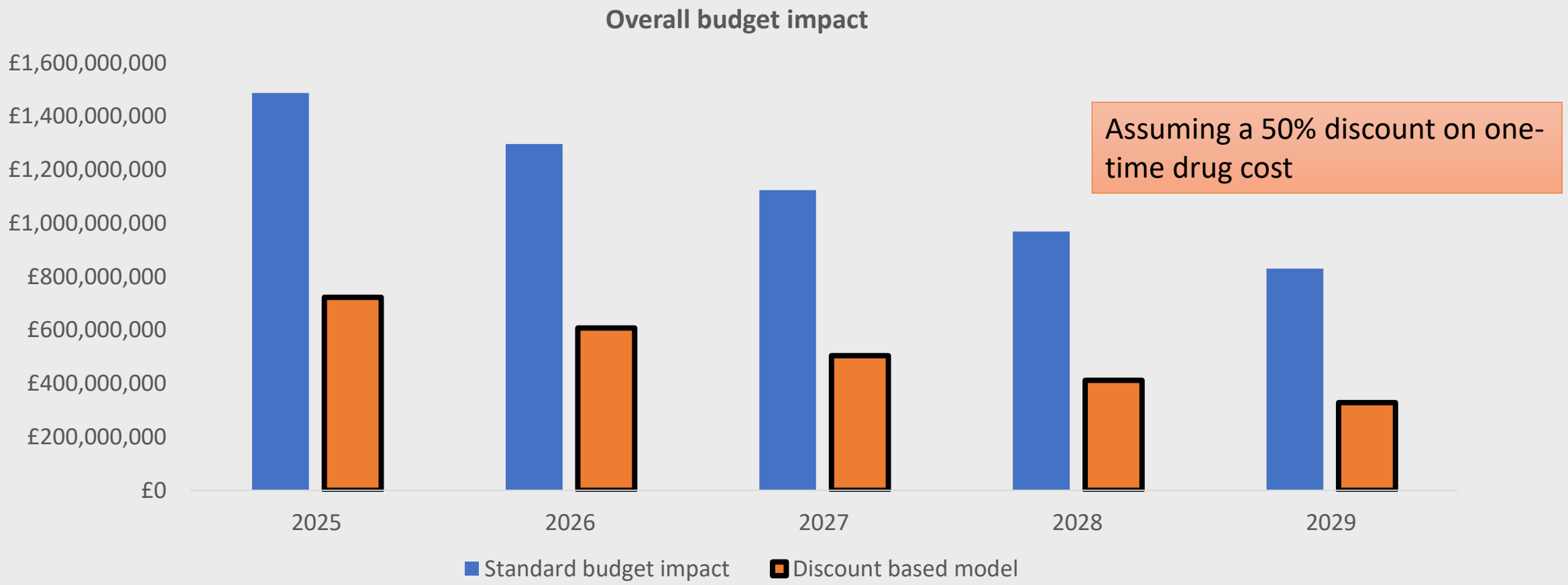
1. Callenbach, et al. "Illustrating the financial consequences of outcome-based payment models from a payers perspective-the case of autologous gene therapy atidarsagene autotemcel (Libmeldy®)." Value in Health (2024).

# Budget impact results – standard payment model

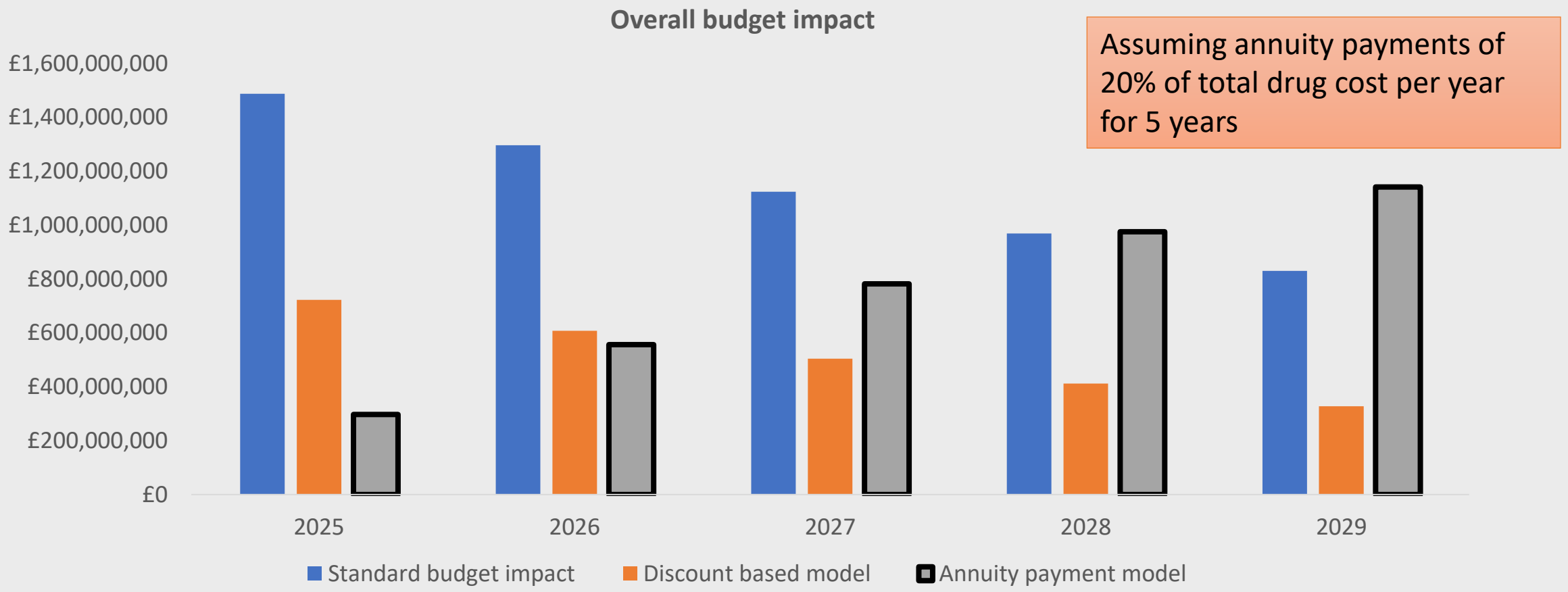
Declining budget impact as patients get cured each year from the prevalent pool.
Please note that patients who live longer do not incur additional healthcare costs as their disease is cured.



# Budget impact results – discount-based model



# Budget impact results – annuity payment model

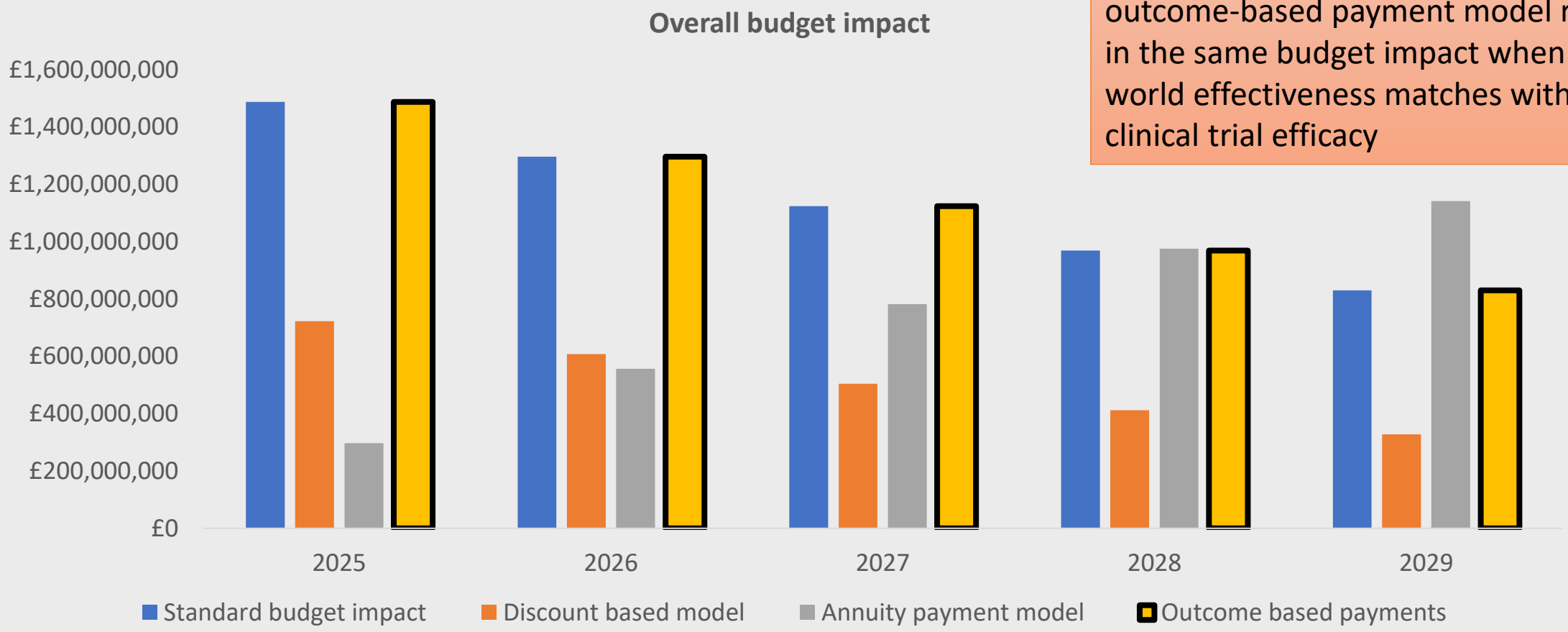



Assuming annuity payments of 20% of total drug cost per year for 5 years

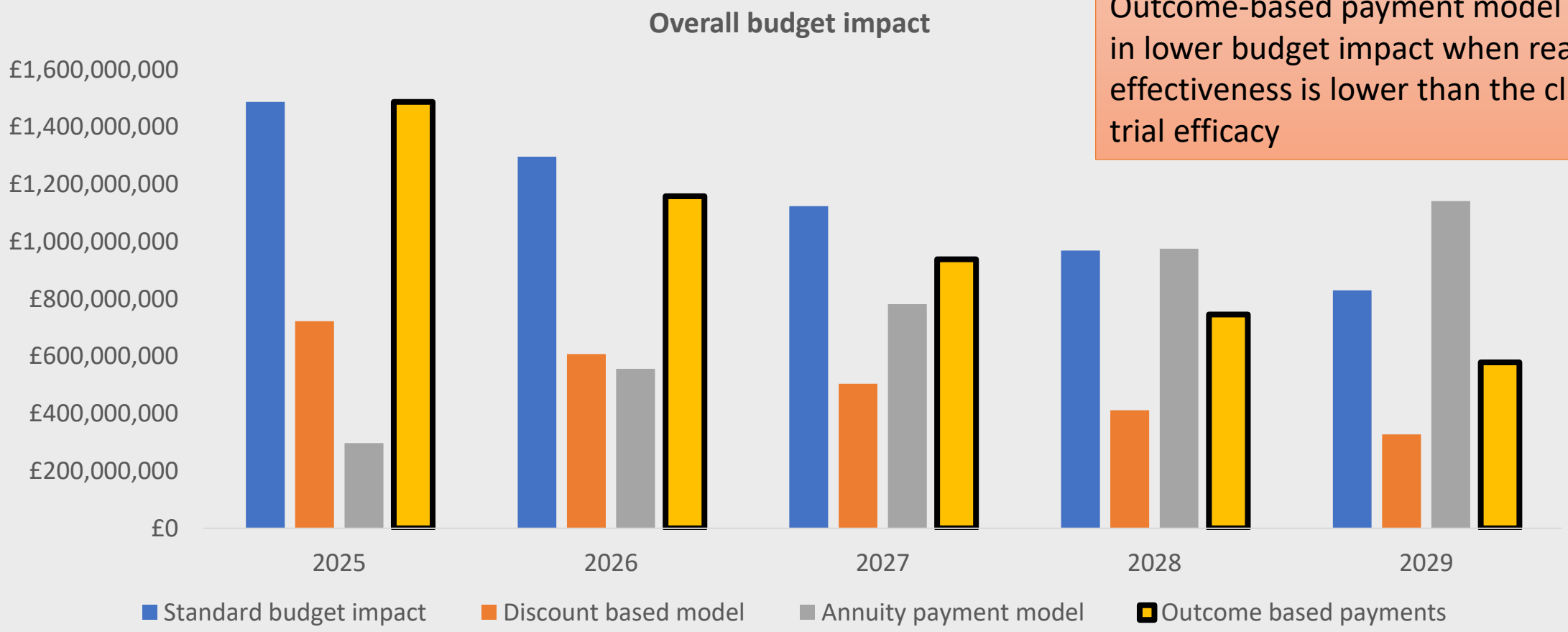
Notice that the amount is increasing under the annuity model vs the standard or the discount models. Why?

# Budget impact results – outcomes-based model (assuming no decline in durability over time)

Notice that the standard model and the outcome-based payment model result in the same budget impact when real-world effectiveness matches with clinical trial efficacy



# Budget impact results – outcomes-based model (assuming decline in durability over time)




Outcome-based payment model results in lower budget impact when real-world effectiveness is lower than the clinical trial efficacy

# What are the circumstances when OBP model will add value to payers?

- When trial efficacy has higher uncertainty due to following reasons
  - Trial sample size is too small and treatment effect has high variability
  - When effect size is large but the trial population is dissimilar to real-world population resulting in uncertainty of effect size in real-world
- In such cases, the durability of clinical trial efficacy in the real-world may get negatively impacted
- OBP models will add value from a payer's perspective in such a scenario

# It’s time for a poll

**Which model would you consider the most viable for your context**

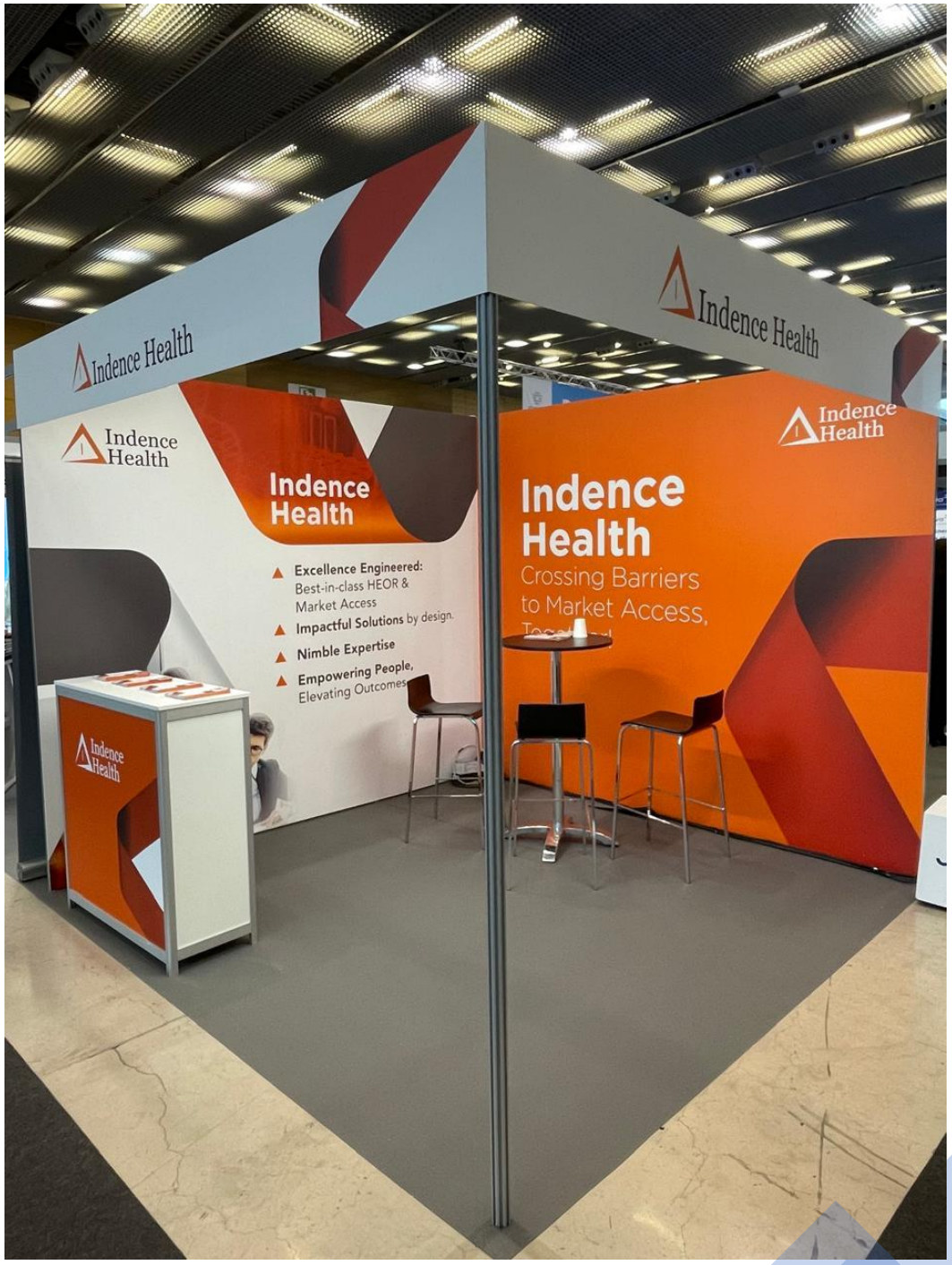
a) Simple discount
b) Annuity based payment
c) Outcomes based contract

# Comparison of Payment Models Across Scenarios

Scenario 1 (High Effectiveness): All models perform well; except the upfront discount model results in loss of value for the manufacturer

Scenario 2 (Lower Effectiveness): Outcomes-based contract provides the most risk mitigation; annuity model offers some flexibility

Key Insight: Each model has strengths depending on whether real-world effectiveness aligns with or diverges from trial results


Indence Health
Indence Health
Indence Health
Indence Health
Indence Health
Excellence Engineered:
Best-in-class HEOR &
Market Access
Impactful Solutions by design.
Nimble Expertise
Empowering People,
Elevating Outcomes
Indence Health
Crossing Barriers
to Market Access,