

Did demand-pull deliver?

Medicare Part D and pharmaceutical approval rates

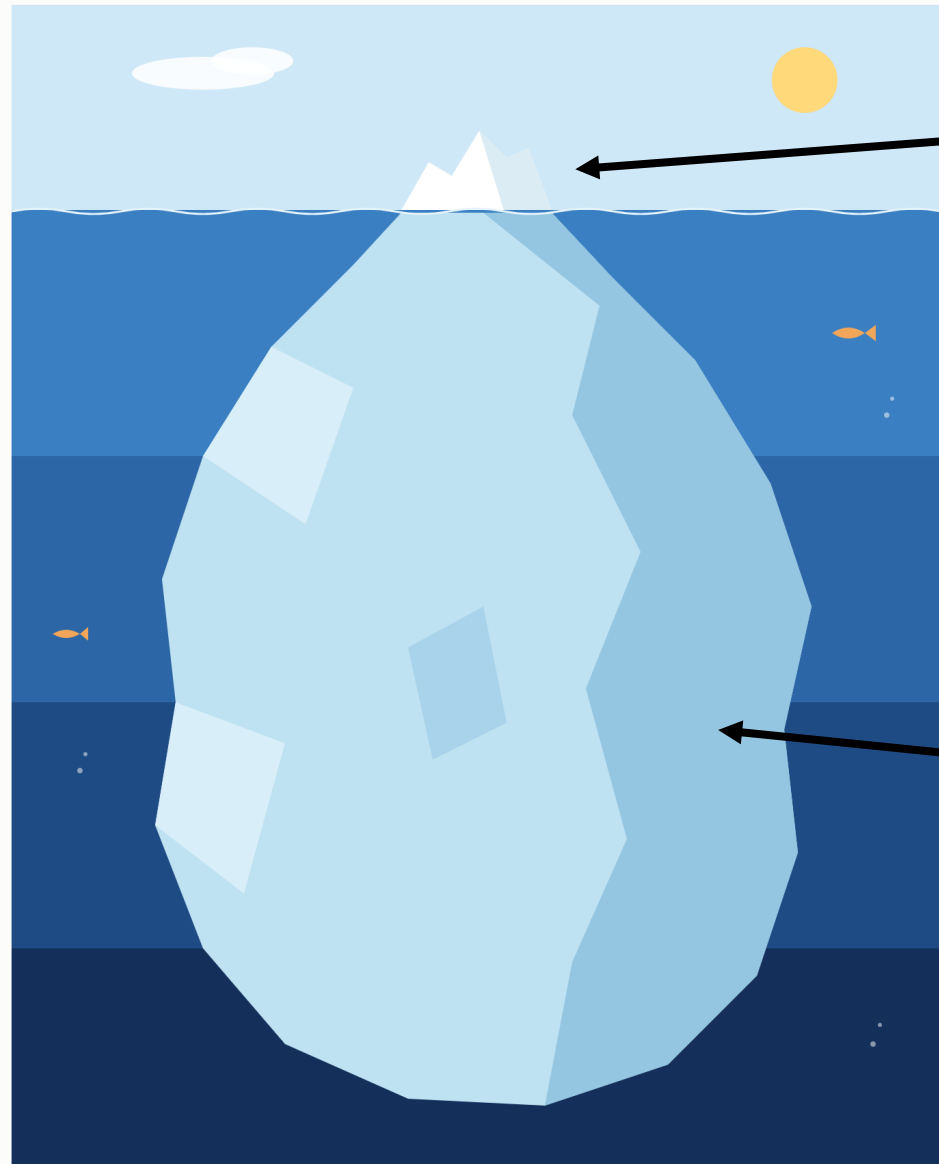
Meg Blume-Kohout

Presentation and discussion by Luca Maini

October 3, 2026

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Luca's published research on pharmaceutical innovation

Amount of Luca's time spent reading (and discussing/ refereeing/talking to journalists) about pharmaceutical innovation

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- ▶ **13 fewer new drugs** over 30 years
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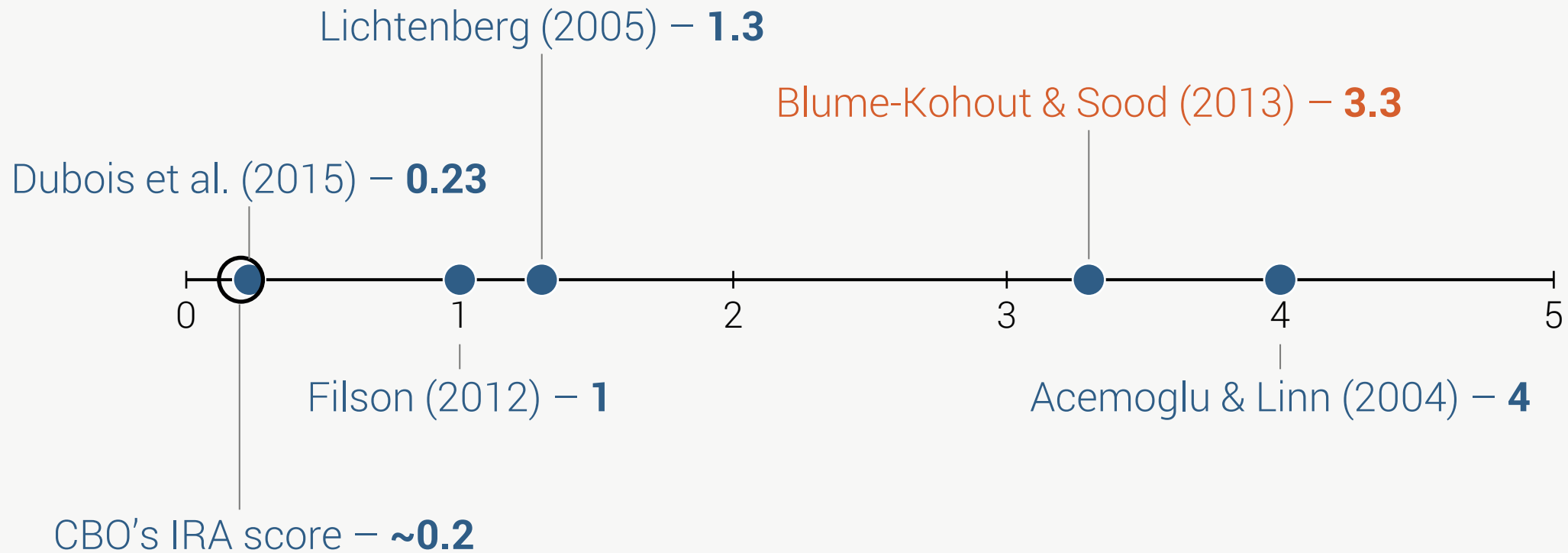
$\epsilon \sim 0.2$

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- ▶ **135 fewer new drugs** over 20 years
- ▶ R&D spending falls by 18.5%
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$\epsilon \sim 1.5$

But unfortunately, we don't know much about this number



And we know even less about *which* drugs are held back

- ▶ In theory, marginal drugs should be less profitable/valuable
 - ▶ Otherwise they would have been developed already
- ▶ Some existing evidence supports this conjecture
 - ▶ Dranove et al. (2022): clinical trials following Part D test non-novel mechanisms
 - ▶ Agha, Kim & Li (2022): exclusion risk cuts R&D for incremental candidates
- ▶ But drug development is highly uncertain
 - ▶ Firms may not know in advance which projects are more likely to succeed
 - ▶ Pfizer terminated development of GLP-1s in the early 90s

This paper tests the BKS (2013) results & checks whether the “marginal” drugs are more/less likely to be approved

- ▶ Blume-Kohout and Sood (2013, BKS):
 - ▶ Effect of Part D on Phase I entries by therapeutic class 1998–2010
 - ▶ DiD with heterogeneous exposure (pre-Part D Medicare share)

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- ▶ Updates the methodology
 - ▶ Data updates
 - ▶ New empirical specification
- ▶ Tracks Phase I trials to FDA approvals 15 years later

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- ▶ Updates the methodology
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- ▶ Tracks Phase I trials to FDA approvals 15 years later
- ▶ **Key finding:** the **marginal trials pulled in by Part D succeed less often**

Empirical design overview: effect of Part D on Phase I CTs

$$\mathbb{E}[\text{Trials}_{ct}] = \exp(\alpha_c + \lambda_c t + \sum_{k=1}^4 \beta_k \text{MedShare}_c \times \text{Post}_{kt} + \gamma \log \text{PredMkt}_{ct} + \theta' \text{NIH}_{ct})$$

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Controls for:

► **Class fixed effects**

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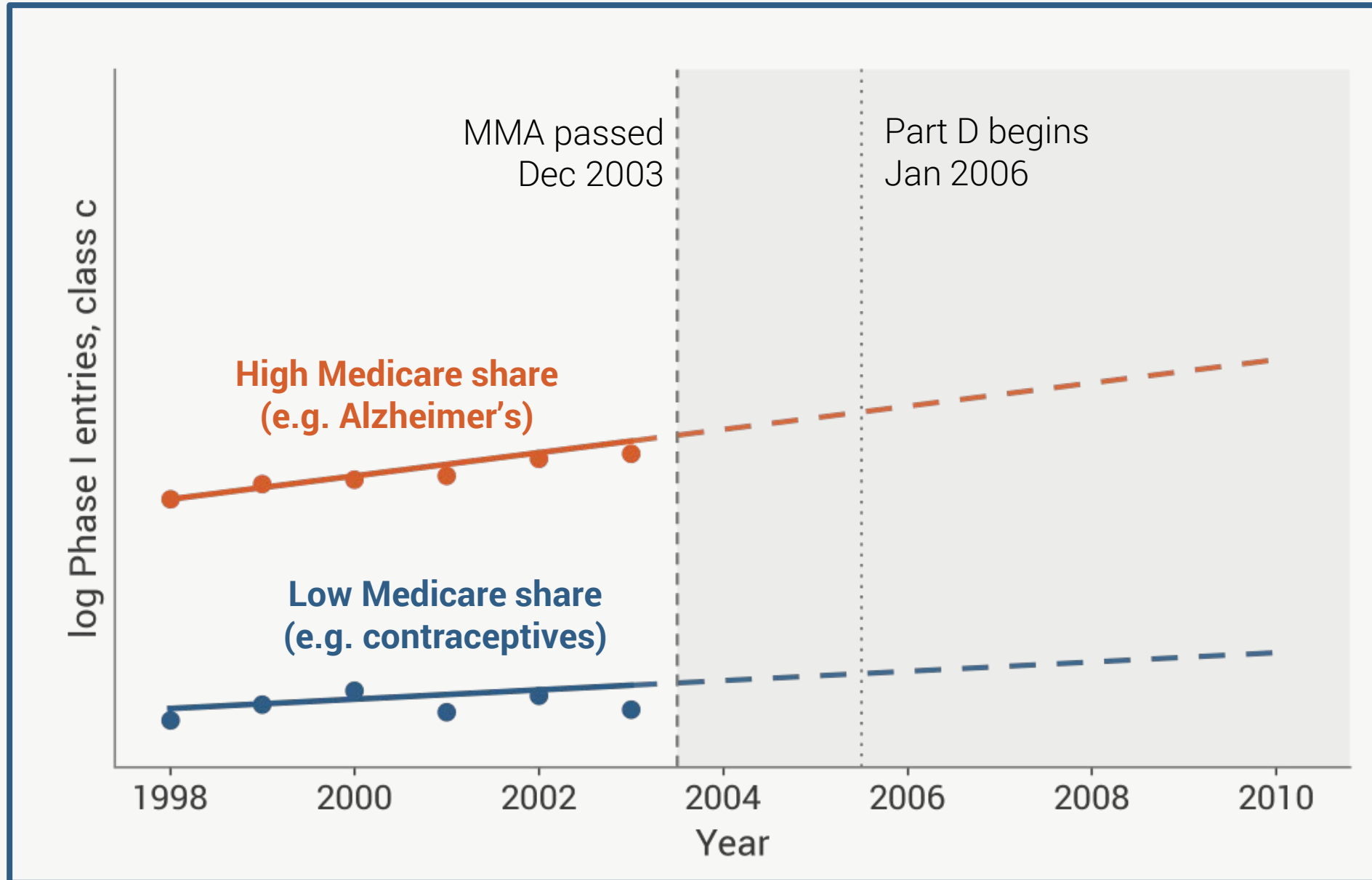
Controls for:

- ▶ Class fixed effects
- ▶ Class-specific trends
- ▶ Predicted market size and NIH funding
- ▶ Intuition: **effect is identified by comparing post-Part D increase in Phase I trial across classes with different Medicare exposure**

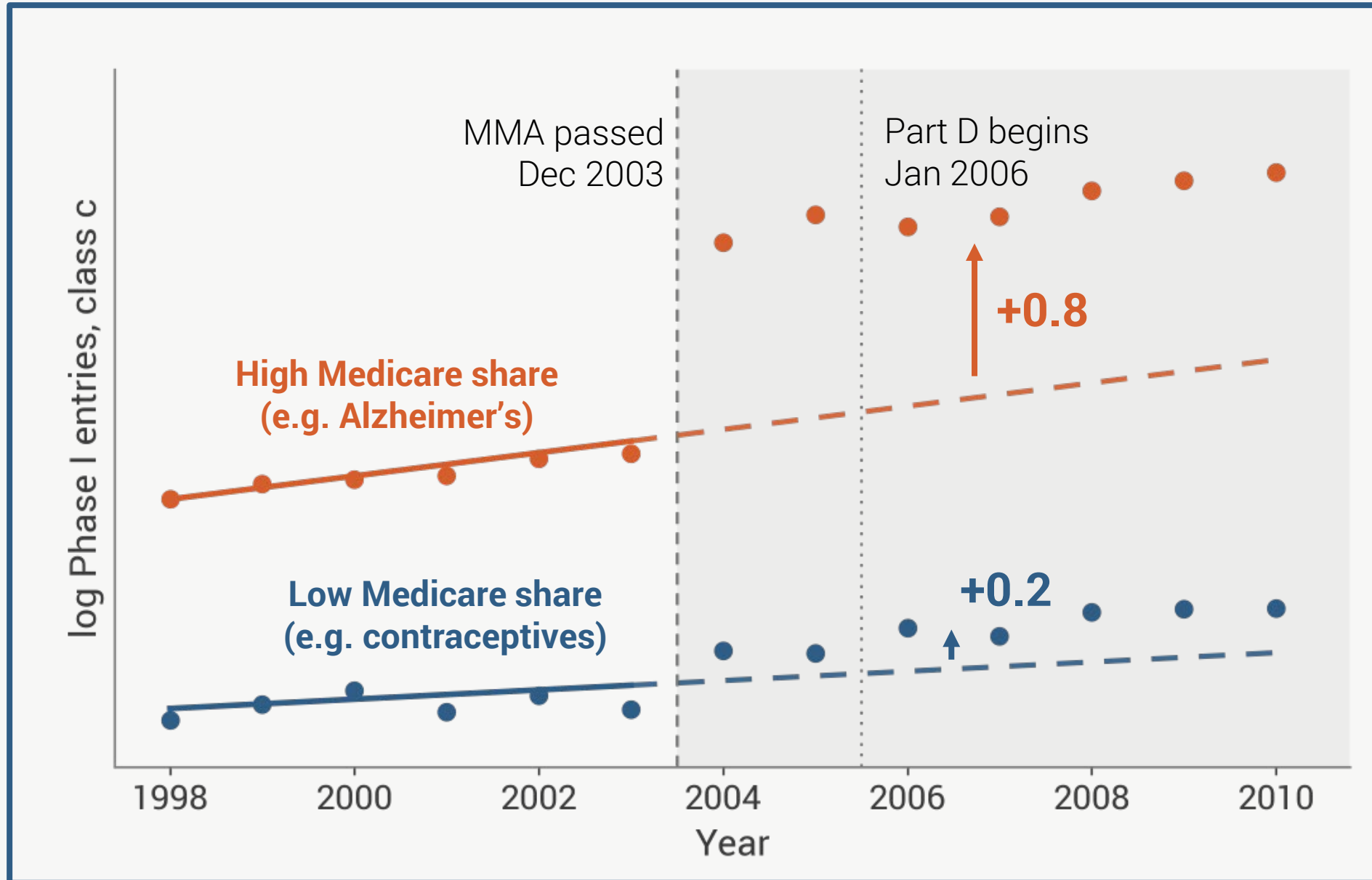
The original BKS empirical design

Phase I entries	
Medicare share × 2003	0.37 [-0.03, 0.77]
Medicare share × 2004–05	0.62 [0.30, 0.95]
Medicare share × 2006–07	0.83 [0.37, 1.29]
Medicare share × 2008–10	1.28 [0.60, 1.96]
log predicted market (8–20 yr lead)	2.70 [-13.85, 19.26]
Class FE / class trends / year FE	yes / yes / no
N (class × year cells)	650

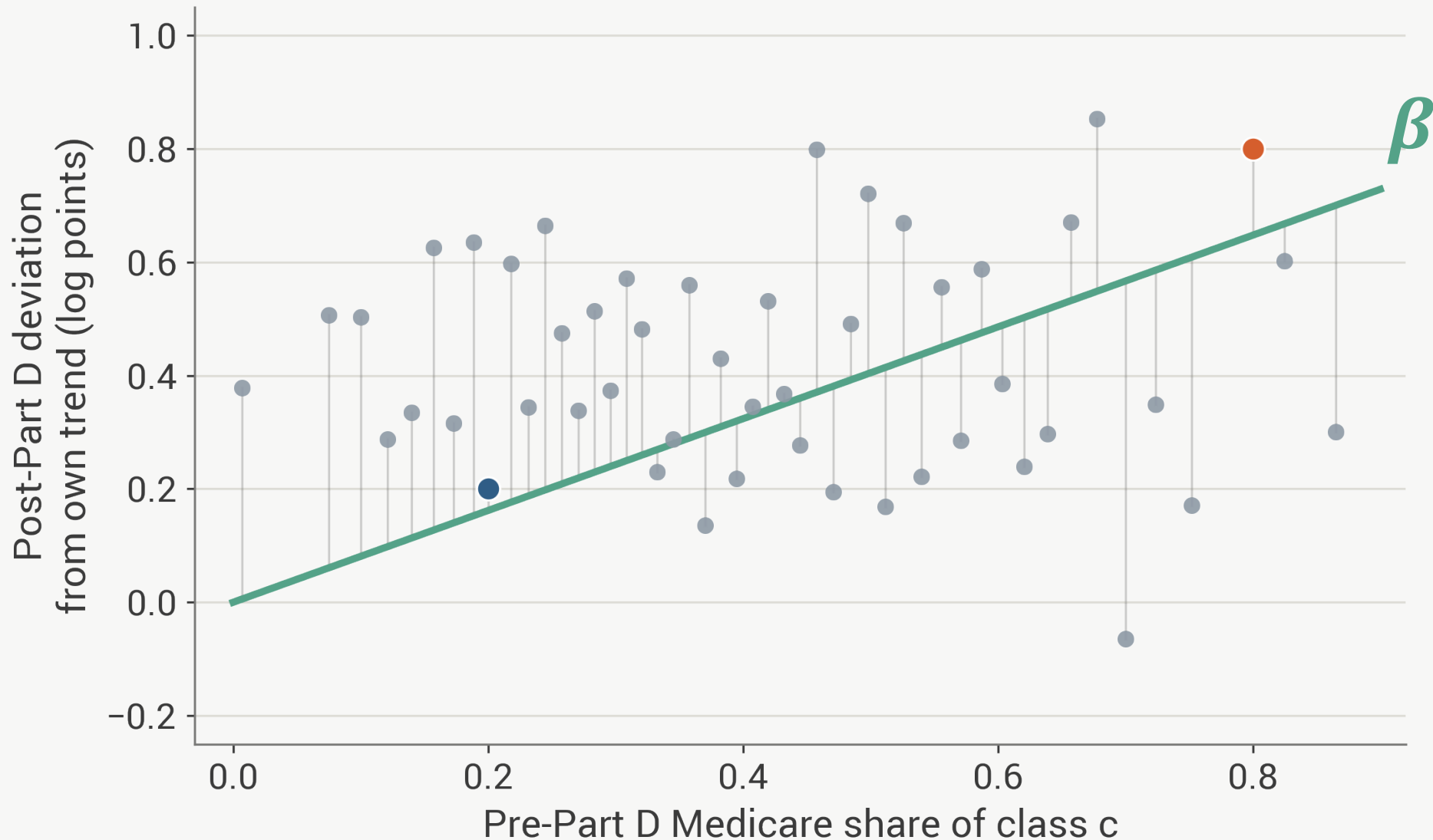
Why is omitting year FEs problematic?



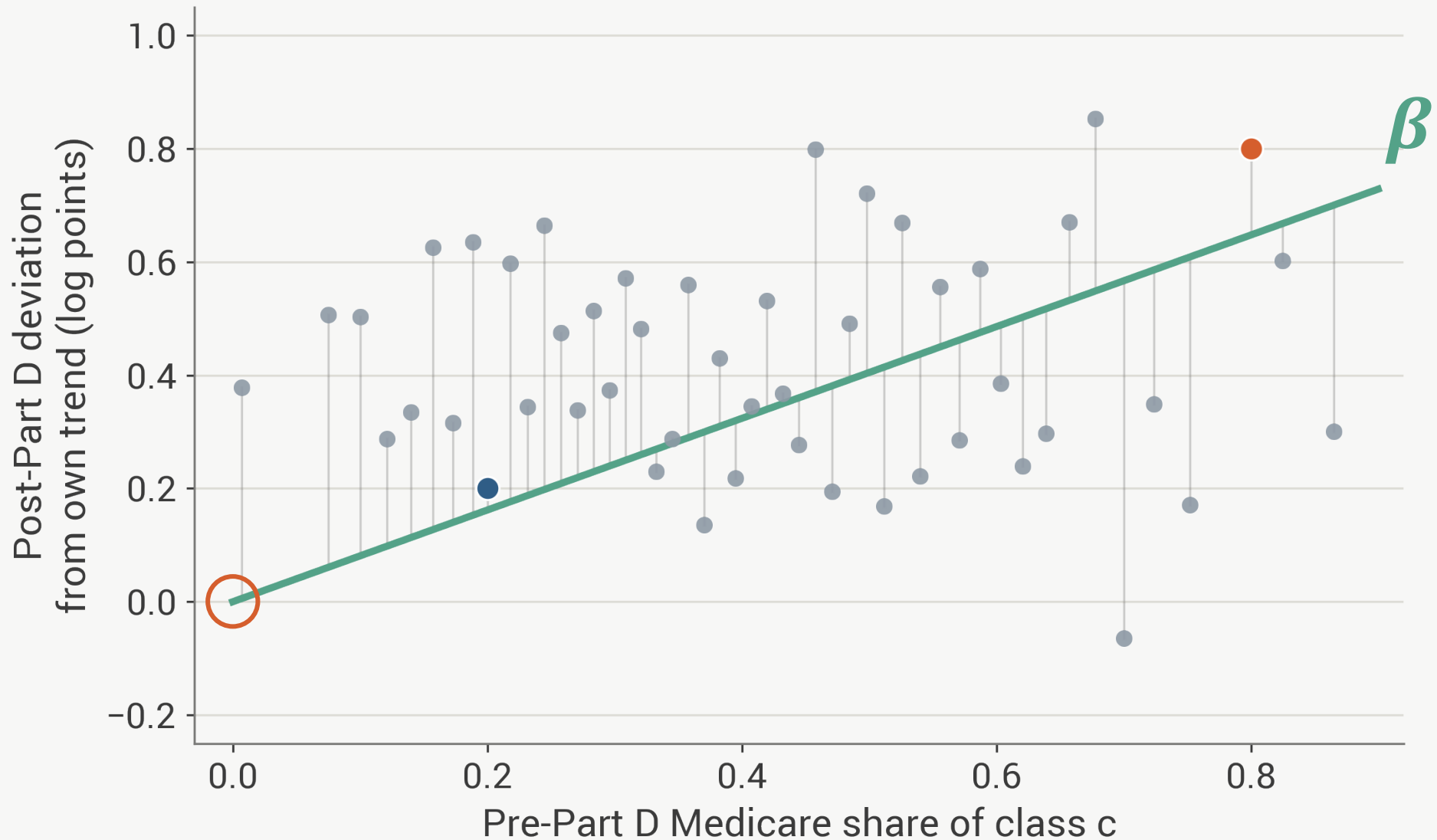
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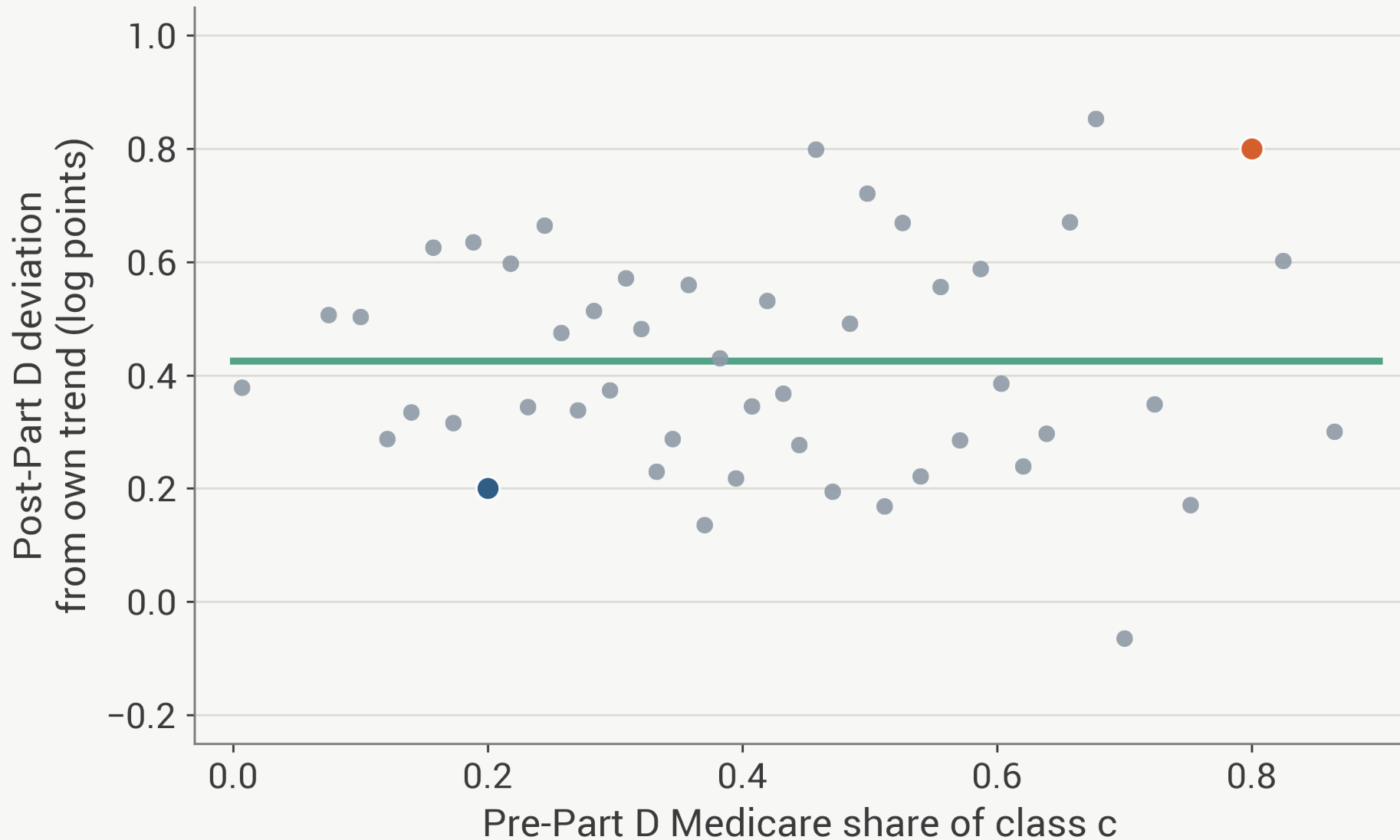
The post-Part D coefficients are the slope of the best-fit line of class coefficients against their Medicare exposure



But omitting year fixed effects implies that the effect for a zero-exposure drug is mechanically set at 0



If Part D generated a market-wide increase in trials, omitting year FEs can generate an upward bias



This matters because Part D may indeed have had a market-wide effect

- ▶ Krieger et al. (2022) finds that manufacturers whose portfolio was more exposed to Part D increased R&D activity
 - ▶ Likely channel → relaxation of financial constraints
- ▶ Implies that even zero-exposure classes may see increased R&D activity

Results appear robust to including year FEs

	Phase I entries	Phase I entries
Medicare share × 2003	0.37 [−0.03, 0.77]	0.46 [−0.41, 1.33]
Medicare share × 2004–05	0.62 [0.30, 0.95]	0.95 [0.28, 1.63]
Medicare share × 2006–07	0.83 [0.37, 1.29]	1.27 [0.36, 2.19]
Medicare share × 2008–10	1.28 [0.60, 1.96]	1.36 [0.12, 2.61]
log predicted market (8–20 yr lead)	2.70 [−13.85, 19.26]	19.55 [−9.28, 48.39]
Class FE / class trends / year FE	yes / yes / no	yes / yes / yes
N (class × year cells)	650	650

Empirical design overview: effect on success rates

$$\begin{aligned}\mathbb{E}[\text{Approvals}_{ct}] = & \text{Trials}_{ct} \times \exp(\beta_0 \text{MedShare}_c + \sum_{k=1}^3 \beta_k \text{MedShare}_c \times \text{Post}_{kt} \\ & + \gamma \log \text{PredMkt}_{ct} + \theta' \text{NIH}_{ct})\end{aligned}$$

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Controls for:

- **Baseline success rate by Medicare Market Share**

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Controls for:

- ▶ Baseline success rate by Medicare Market Share
- ▶ **Predicted market size and NIH funding**

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Controls for:

- ▶ Baseline success rate by Medicare Market Share
- ▶ Predicted market size and NIH funding
- ▶ Intuition: **effect is identified by comparing pre- and post-Part D success rates of Phase I trials in classes with different Medicare exposure**

Phase I trials started post-Part D in high-exposure classes are less likely to be approved

	Phase I entries	Phase I entries	Approvals per Phase I entry
Medicare share (pre-Part D gradient)			1.13 [−0.14, 2.39]
Medicare share × 2003	0.37 [−0.03, 0.77]	0.46 [−0.41, 1.33]	−0.02 [−1.47, 1.42]
Medicare share × 2004–05	0.62 [0.30, 0.95]	0.95 [0.28, 1.63]	−1.91 [−3.10, −0.72]
Medicare share × 2006–07	0.83 [0.37, 1.29]	1.27 [0.36, 2.19]	−2.57 [−3.57, −1.58]
Medicare share × 2008–10	1.28 [0.60, 1.96]	1.36 [0.12, 2.61]	
log predicted market (8–20 yr lead)	2.70 [−13.85, 19.26]	19.55 [−9.28, 48.39]	0.01 [−0.16, 0.17]
Class FE / class trends / year FE	yes / yes / no	yes / yes / yes	no / no / no
N (class × year cells)	650	650	594

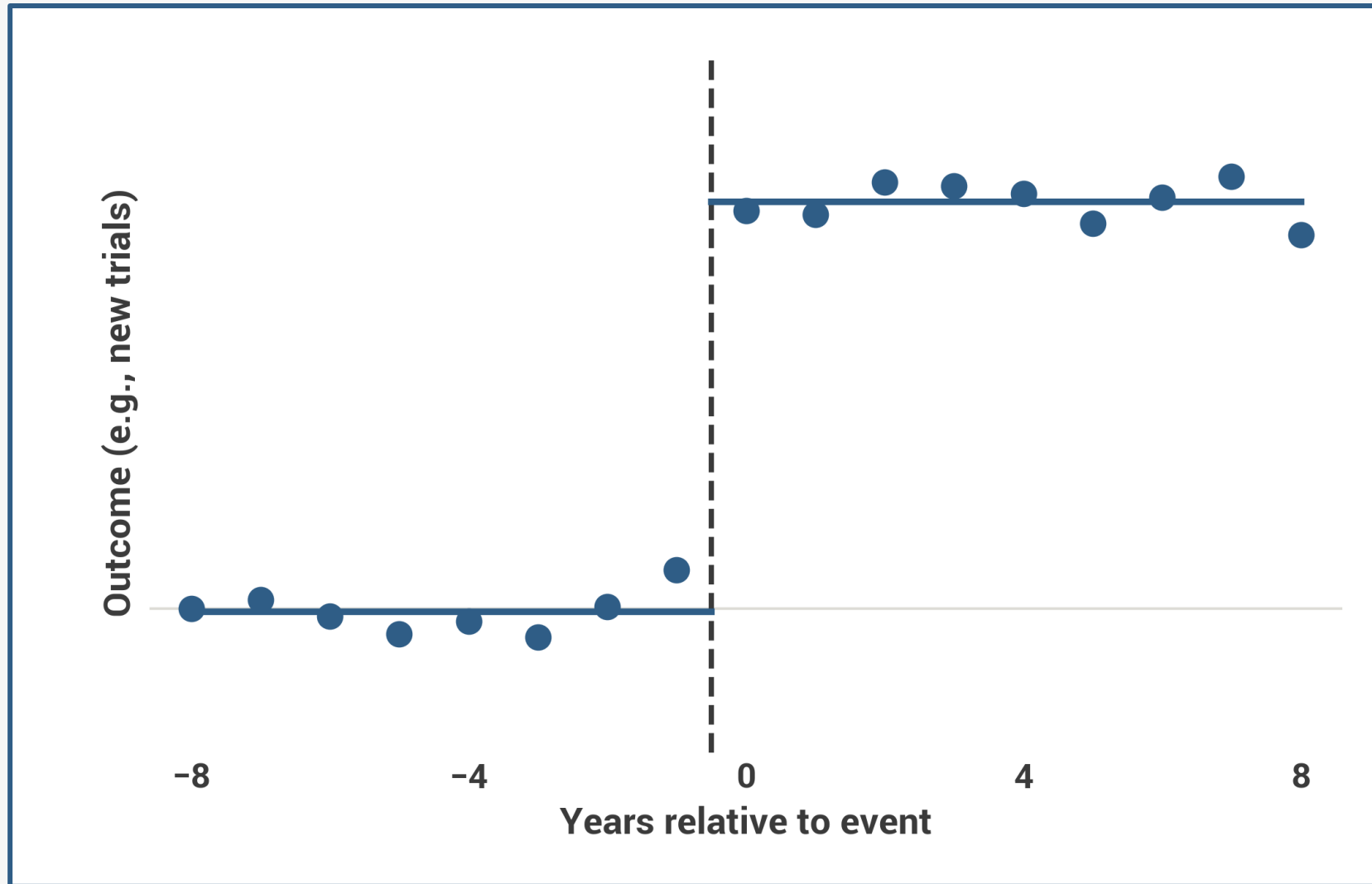
Broader thoughts

Three high-level comments

1. Using event-studies with innovation as an outcome variable
2. More general issues with measuring market size and innovation
3. What are we estimating: the effect of Part D, or the effect of market size?

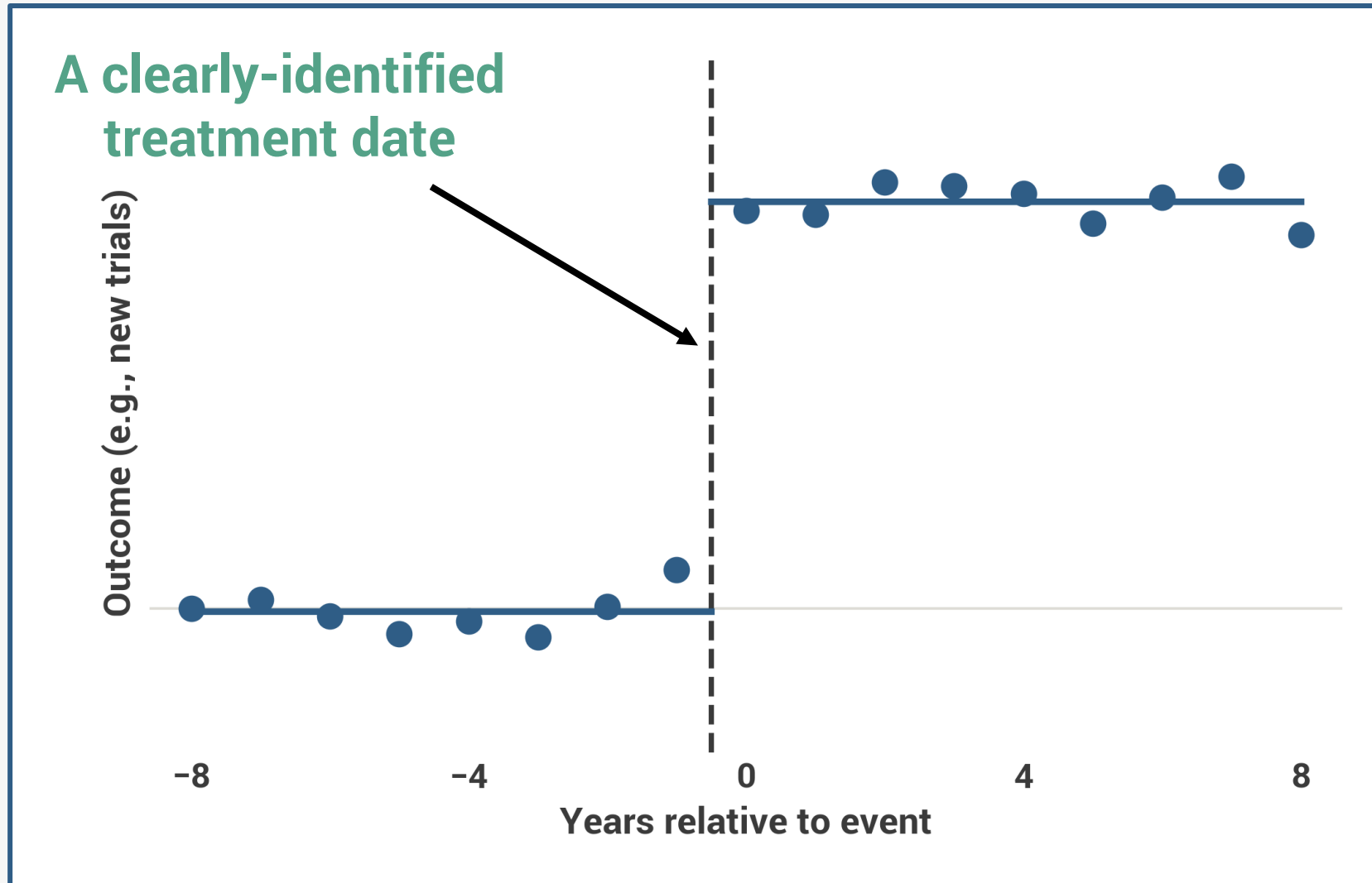
1. Innovation and event-studies

- Causal inference in event-studies depends on two elements



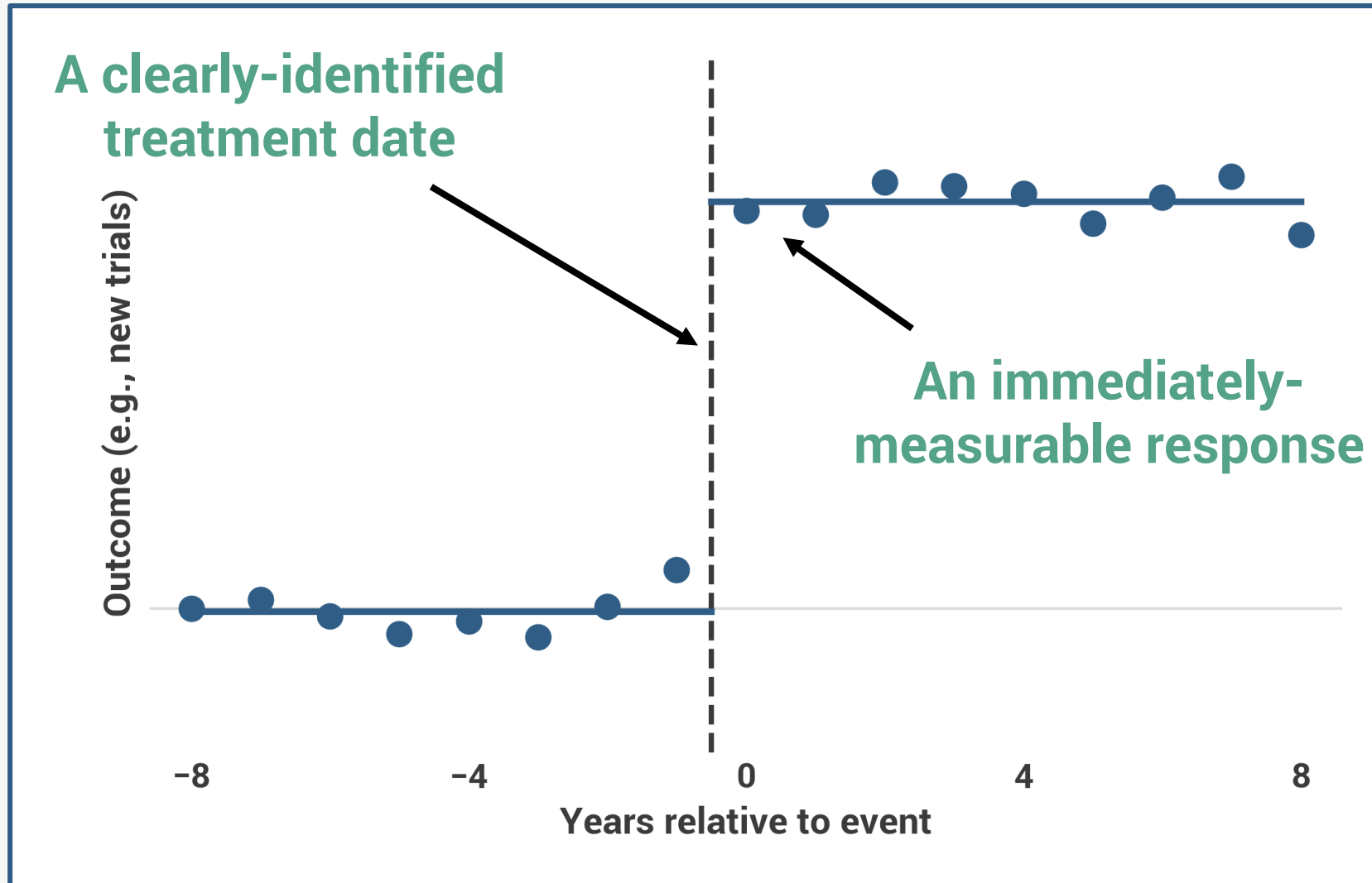
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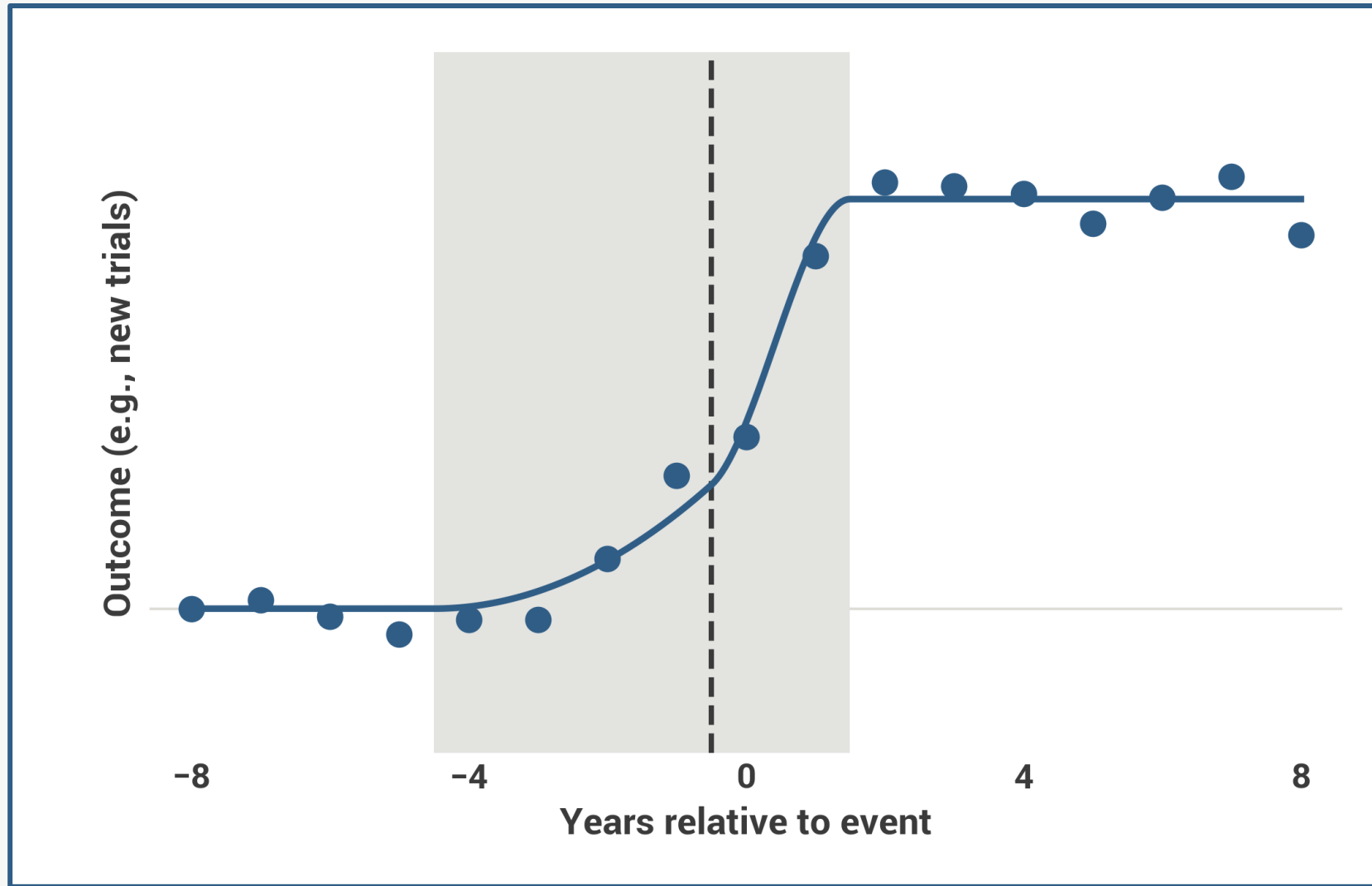
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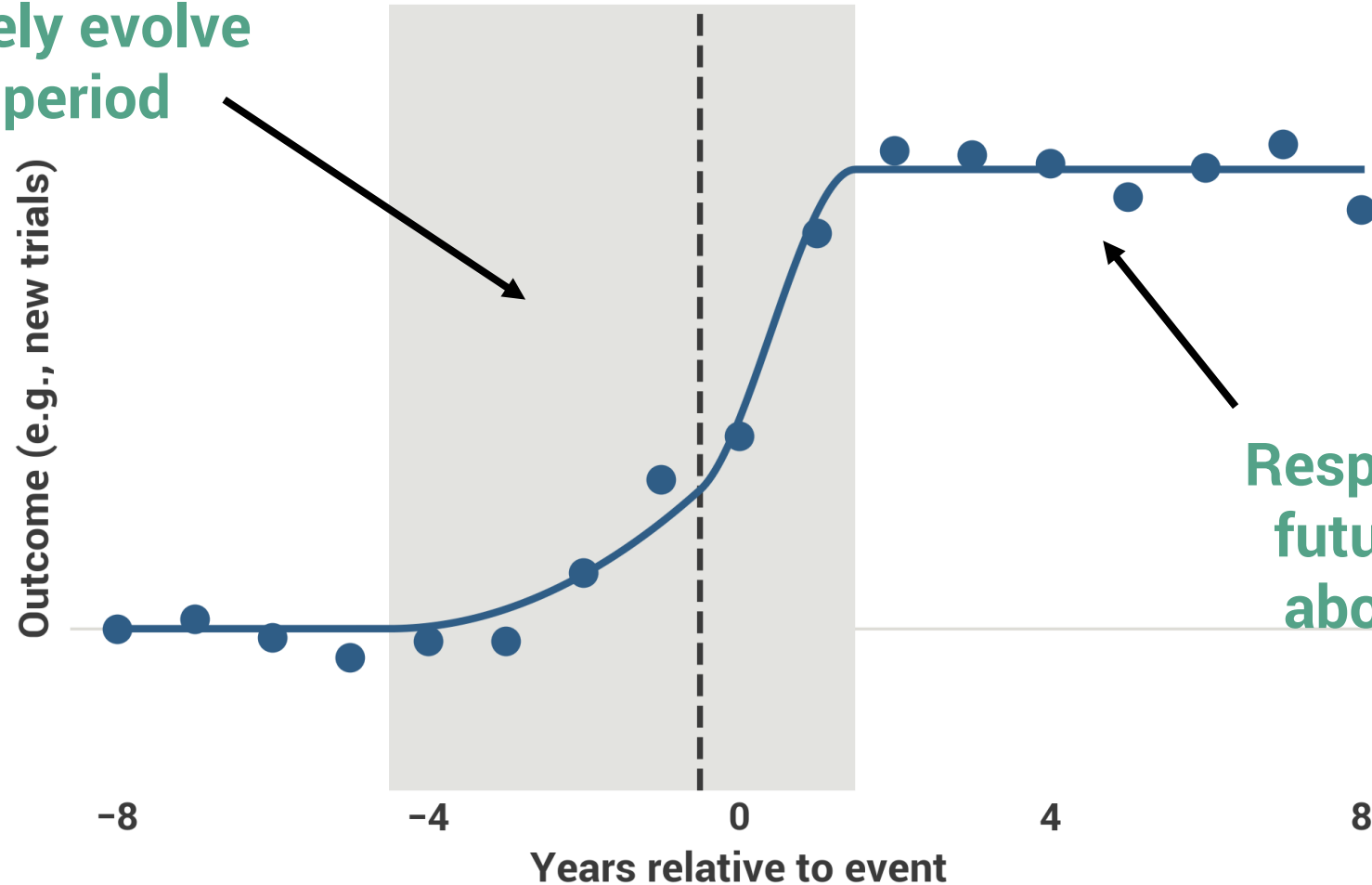
- But innovation depends on expectations that form over time



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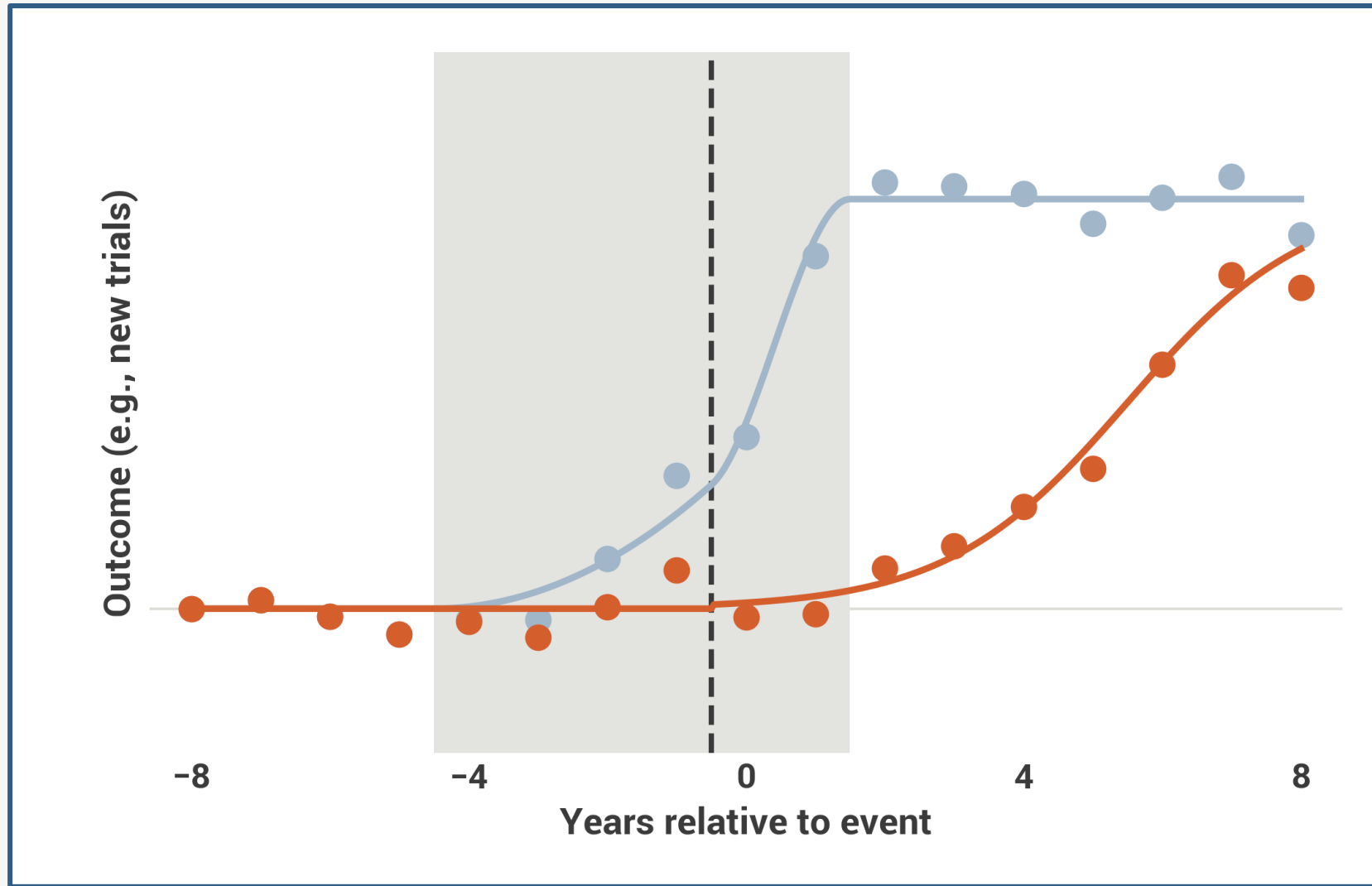
Expectations likely evolve over a longer period



Response may reflect future expectations about other events

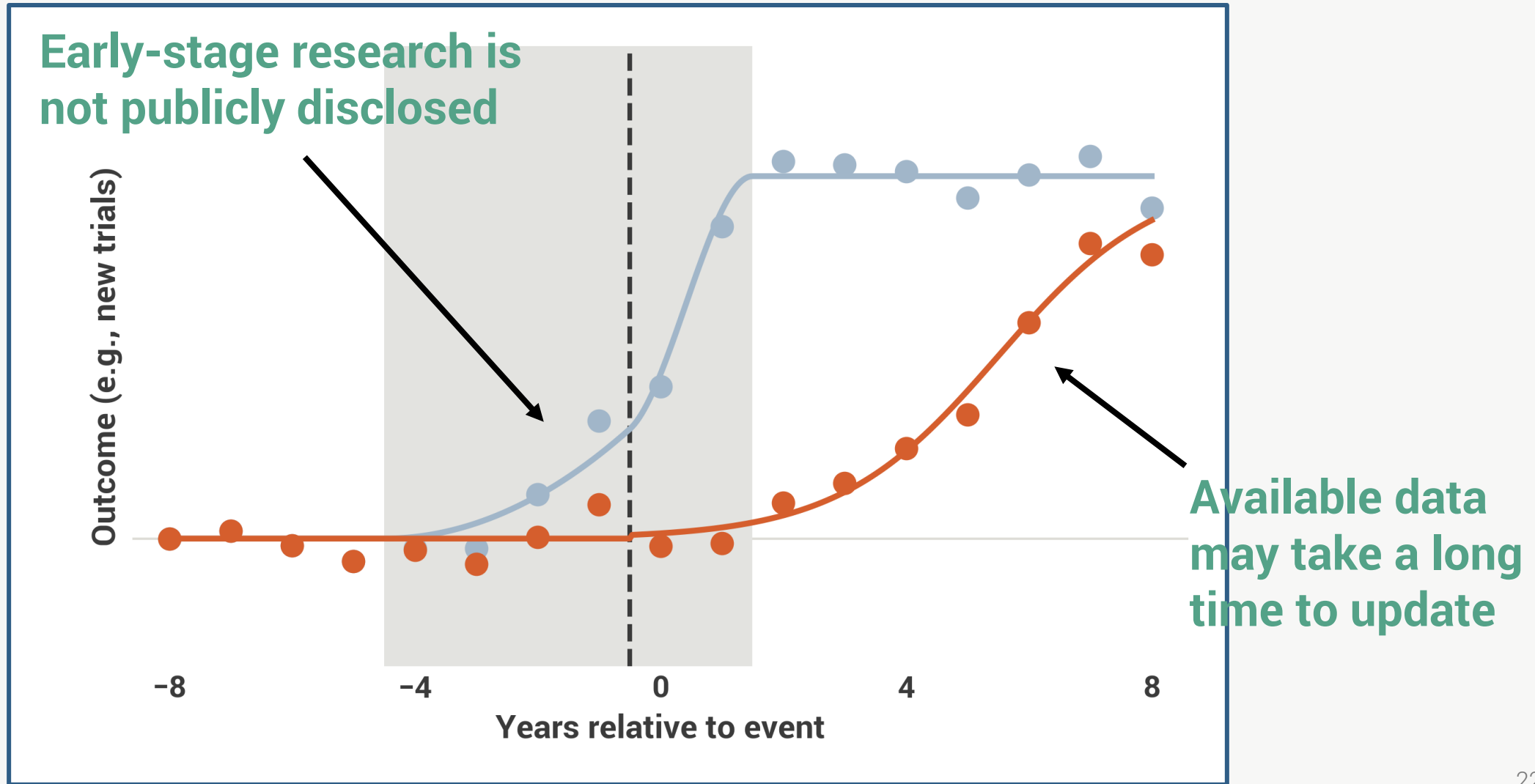
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2A. Measuring innovation: the endpoint matters

- ▶ Most commonly used metrics:
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 - ▶ US NMEs, 1980–2023: **1,402**

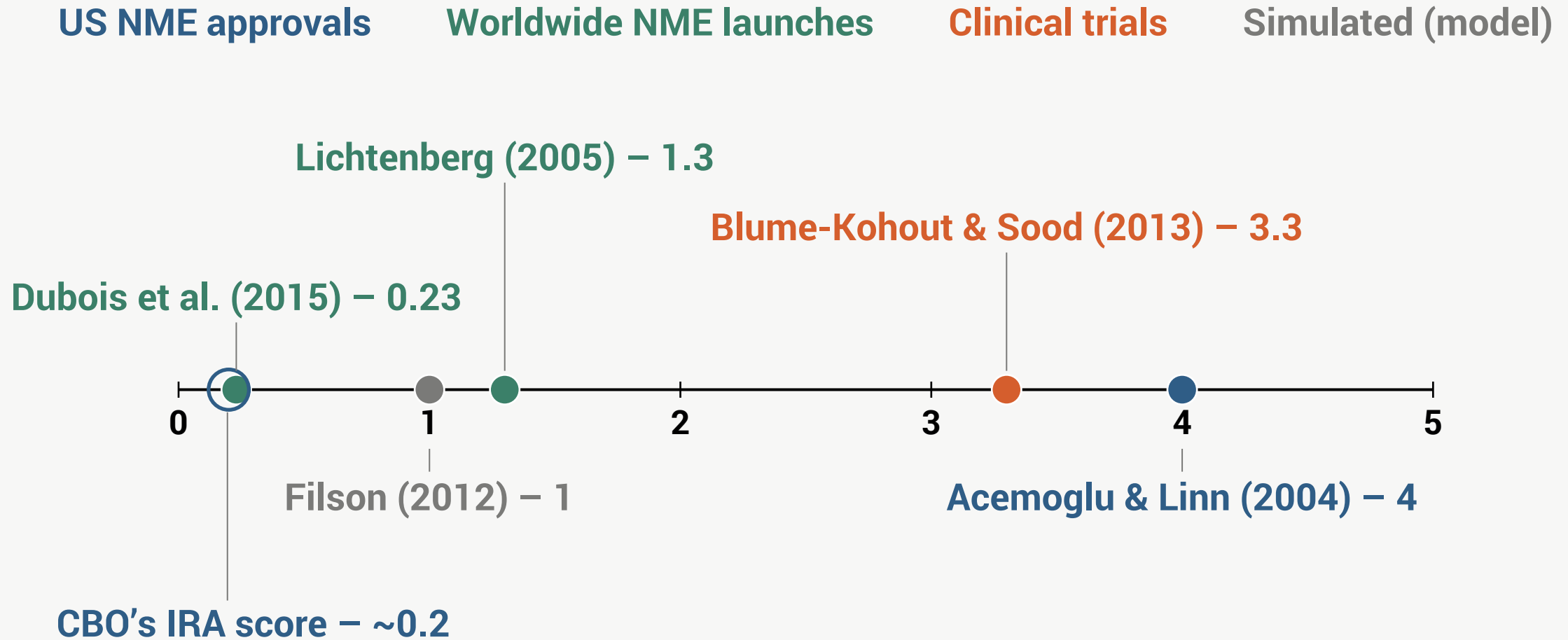
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 - ▶ All countries in IQVIA MIDAS (~70): **2,965**
 - ▶ Key distinction → development ≠ launch

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 - ▶ US NMEs, 1980–2023: 1,402
 - ▶ All countries in IQVIA MIDAS (~70): 2,965
 - ▶ Key distinction → development ≠ launch
- ▶ Elasticities of NMEs vs. CTs only comparable if success rate is constant
 - ▶ CTs go up by 20%, but marginal CT half as likely to succeed → NMEs go up by 10%
 - ▶ The paper can make this point and back it with evidence

Most of the papers estimating the elasticity of innovation with respect to market size use different approaches



2B. Measuring market size: even messier

Key tension:

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Two issues:

1. Impact on revenue $\neq$ impact on profit
 - ▶ Price cuts $\rightarrow$ lowers profit one-for-one
 - ▶ Coverage expansion $\rightarrow$ increases profit by the % markup (less than one)
2. Realized revenue $\neq$ potential revenue
 - ▶ Highest rewards where there are no treatments (i.e., no observed revenue)
 - ▶ Lowest rewards in crowded classes (think statins and PCSK9s)

3. The effect of Part D is NOT (just) the effect of mkt. size

- ▶ The original BKS paper is about market size and innovation
- ▶ But the design estimates something closer to the effect of Part D
- ▶ Estimates likely incorporate effect of higher cash flow
 - ▶ Those two effects may be correlated (so the year FEs don't pick them up)
 - ▶ Firms specialize, so firms with exposed drugs are likely investing in related R&D
- ▶ Note that each estimate is valuable in its own right
 - ▶ Market size and innovation interesting from an economic perspective
 - ▶ Effect of Part D likely more useful for policy work

Conclusion

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- ▶ The question at the center of this paper is probably the single most important question in drug policy
- ▶ We need all the evidence we can get, even if imperfect
 - ▶ This paper is a step forward in that direction
- ▶ But, the literature needs to be clear about what the design estimates
 - ▶ Part D or market size? Trials or NMEs? Is revenue the same as market size?

