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Data Exclusivity for Open Science Drug Development

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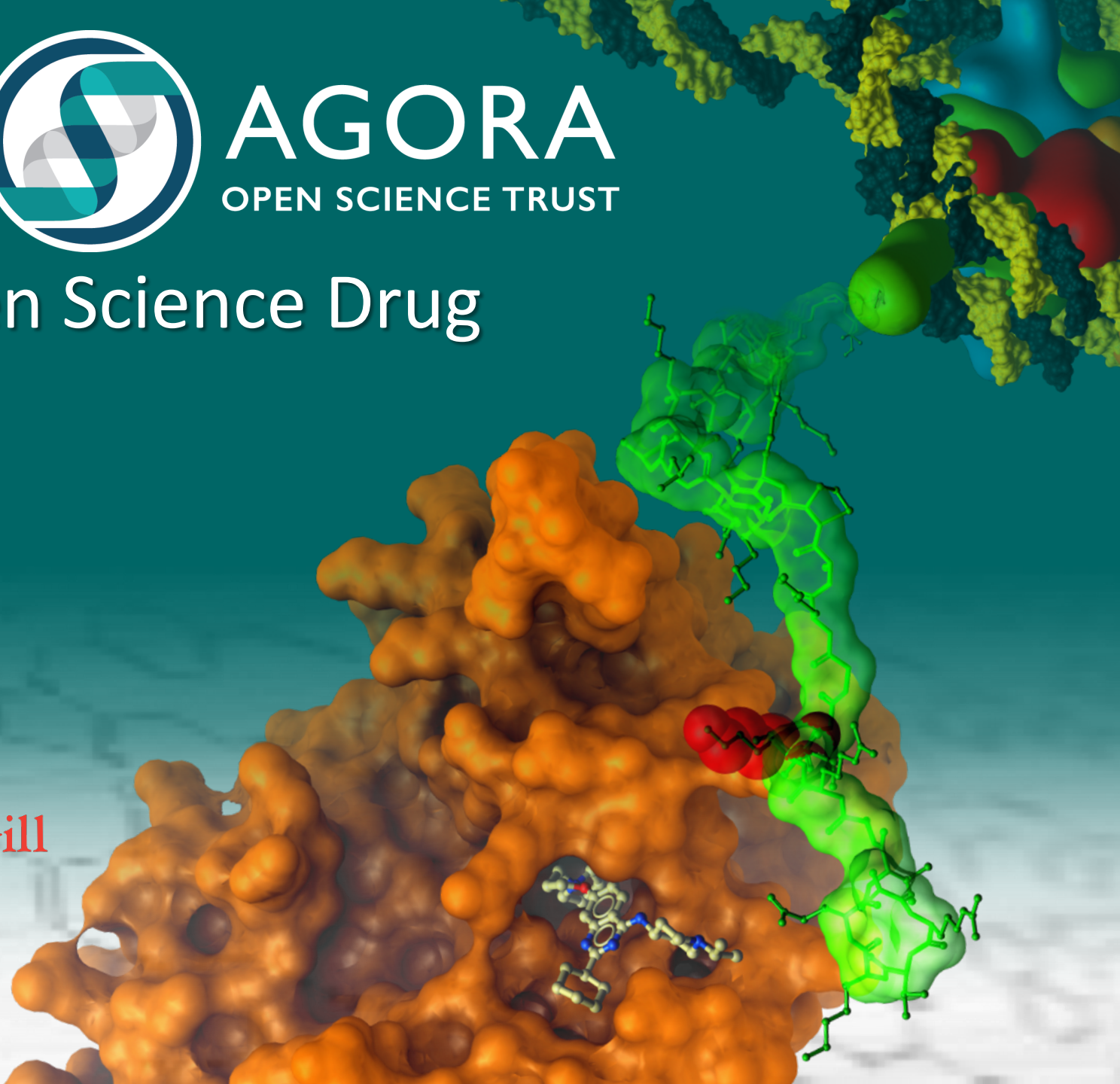
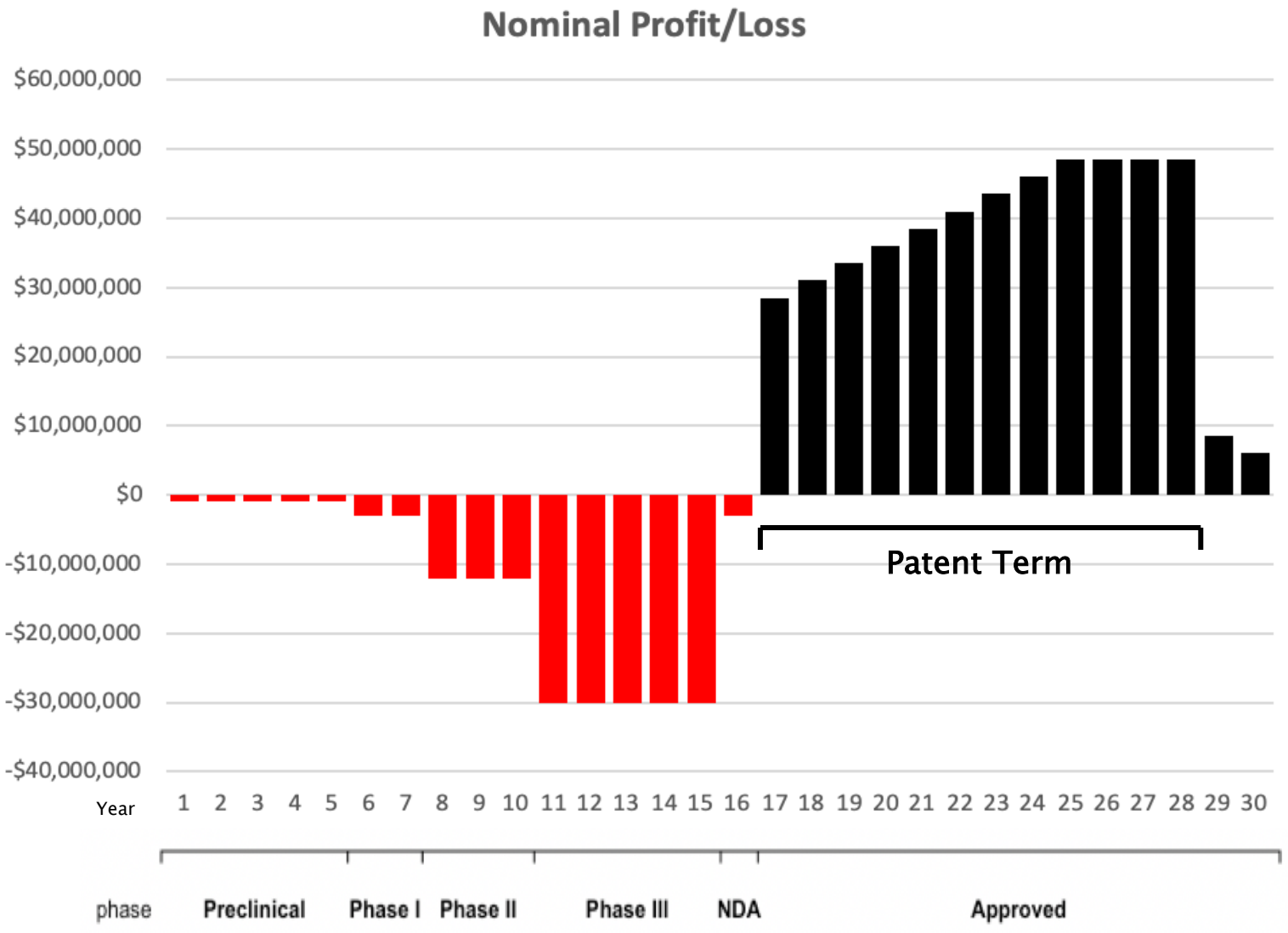
SGC Director of Policy & Legal
Agora CEO



Significant Challenges in New Drug Development

- Uneconomic investment case for many novel targets
  - Therapeutic categories where high failure rates or markets too small (rare diseases, poor populations, etc)
  - E.g. Neurodegeneration, AMR pathogens, tropical diseases, pediatric diseases, personalized medicines
- Investment case for many other novel targets relies on exorbitant pricing
  - PMPRB: price of top patented drugs in Canada up 800% in 10 years
  - Kalydeco and Orkambi pricing issues in media
  - Some new rare disease drugs approaching $1M in Canada
  - Targeted cancer therapies (mAbs, kinases inhibitors) – $$$
  - Glybera failure

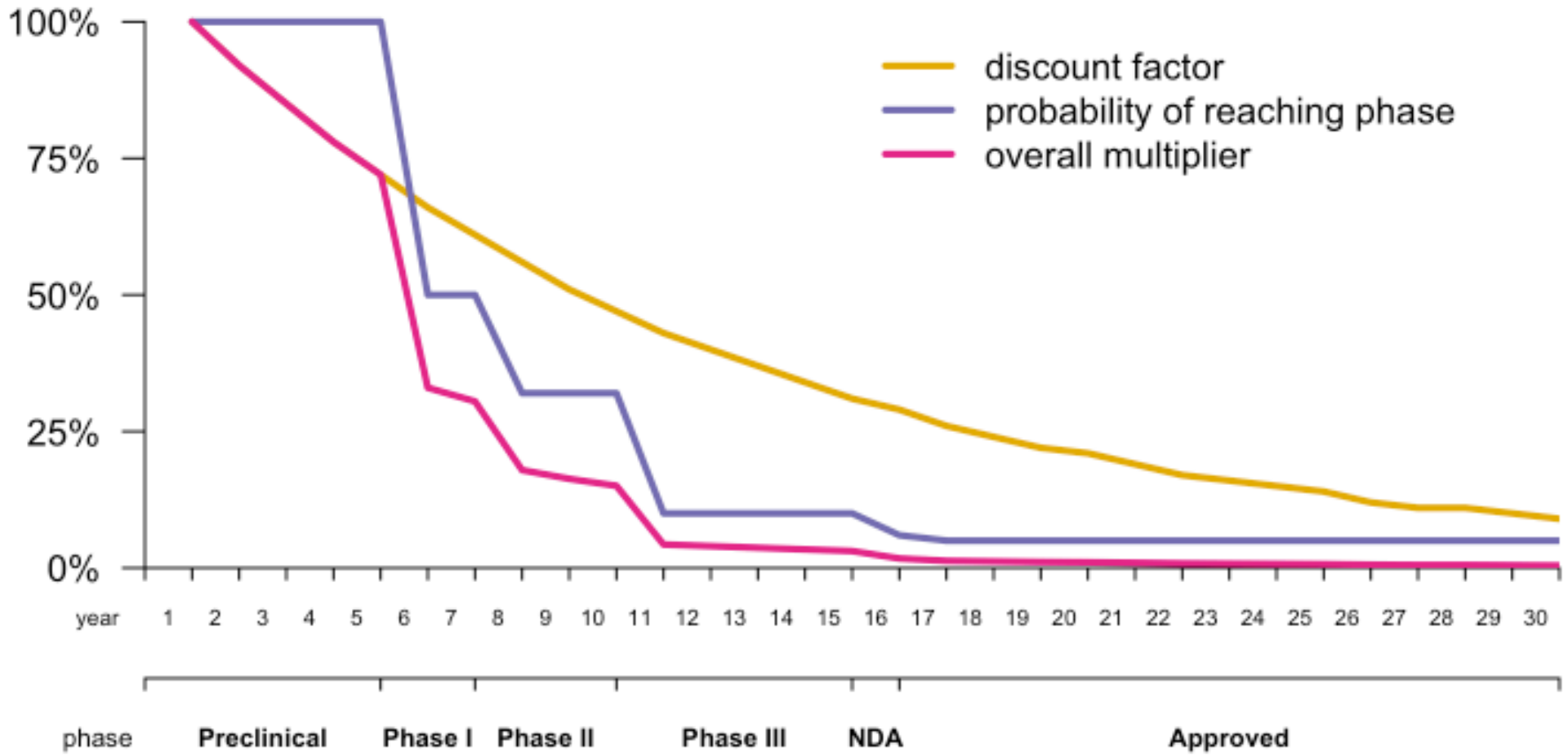
Rare Disease Investment Model – Base Case



ASSUMPTIONS	
Patient Population	2,000
Market Exclusivity	12 years (patent)
Price Per Patient Per Year	$25,000
Investment Decision Point	Early Preclinical
OUTCOMES	
Nominal Profit/Loss	$306,500,000
Business Case for Investment?	**NO**

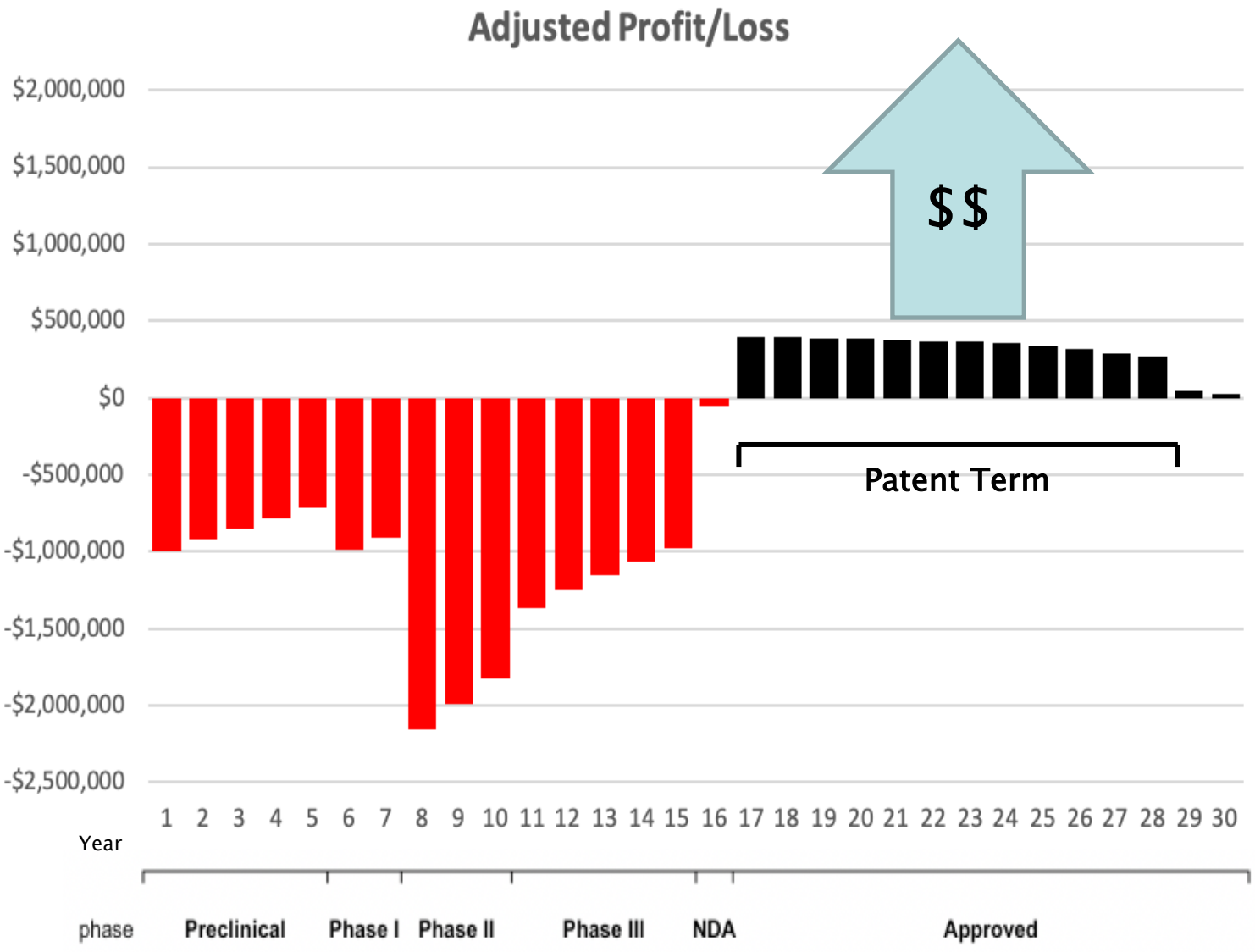
Based on financial model by
Eric Minikel, CureFFI.org

Pharma Investors Apply High Discount Rates



Source: Eric Minikel, CureFFI.org

Net Present Value Analysis – Affordable Pricing Cannot be Justified



High Nominal Profit ($306,500,000)

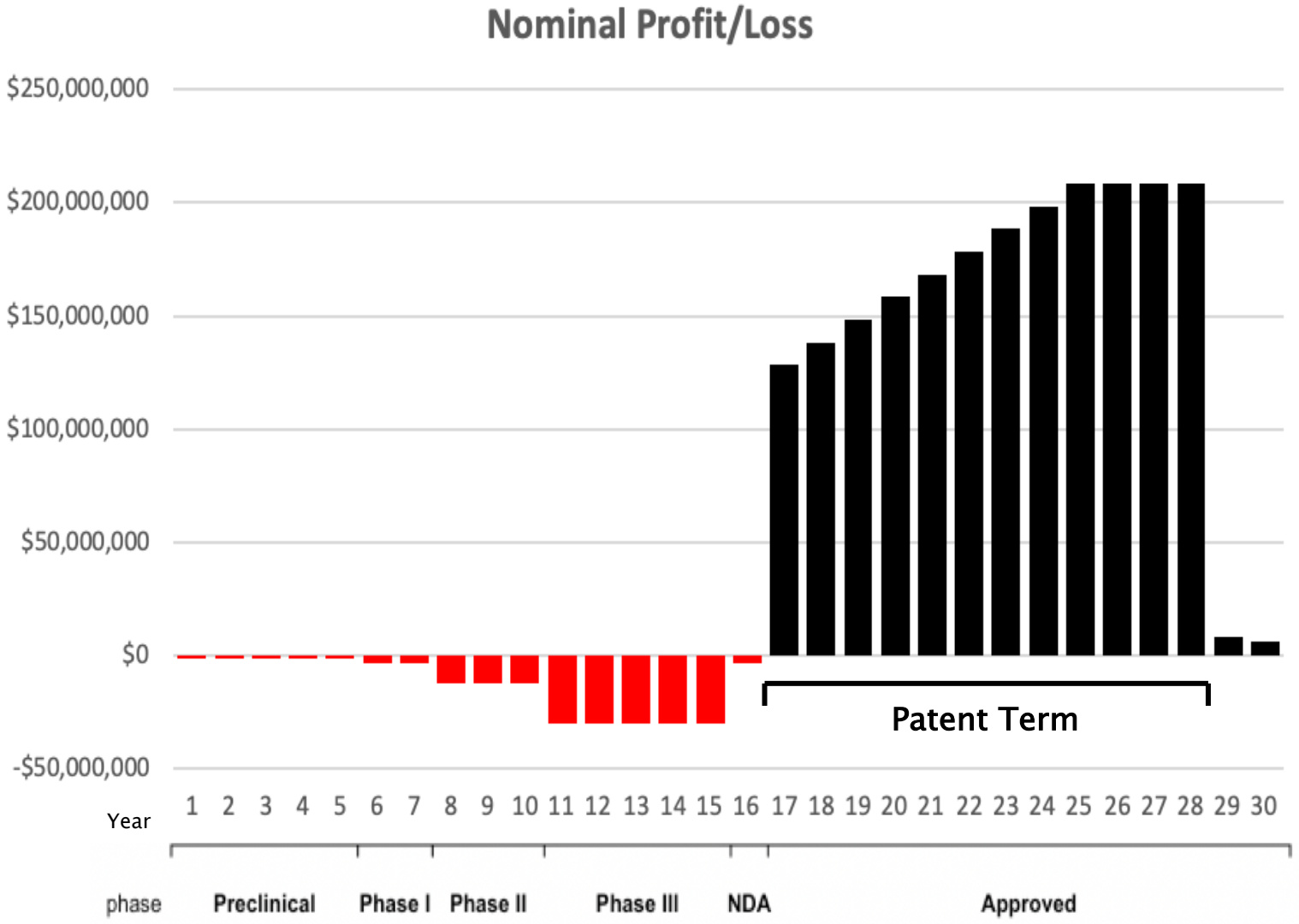
BUT

Negative NPV (–$13,675,046)

Why?

- High upfront R&D costs
- Time value of money discounts future revenues with a long lag time
- Increasing probability of failure at each stage

What would encourage early-stage private investment in this opportunity? Much higher prices!



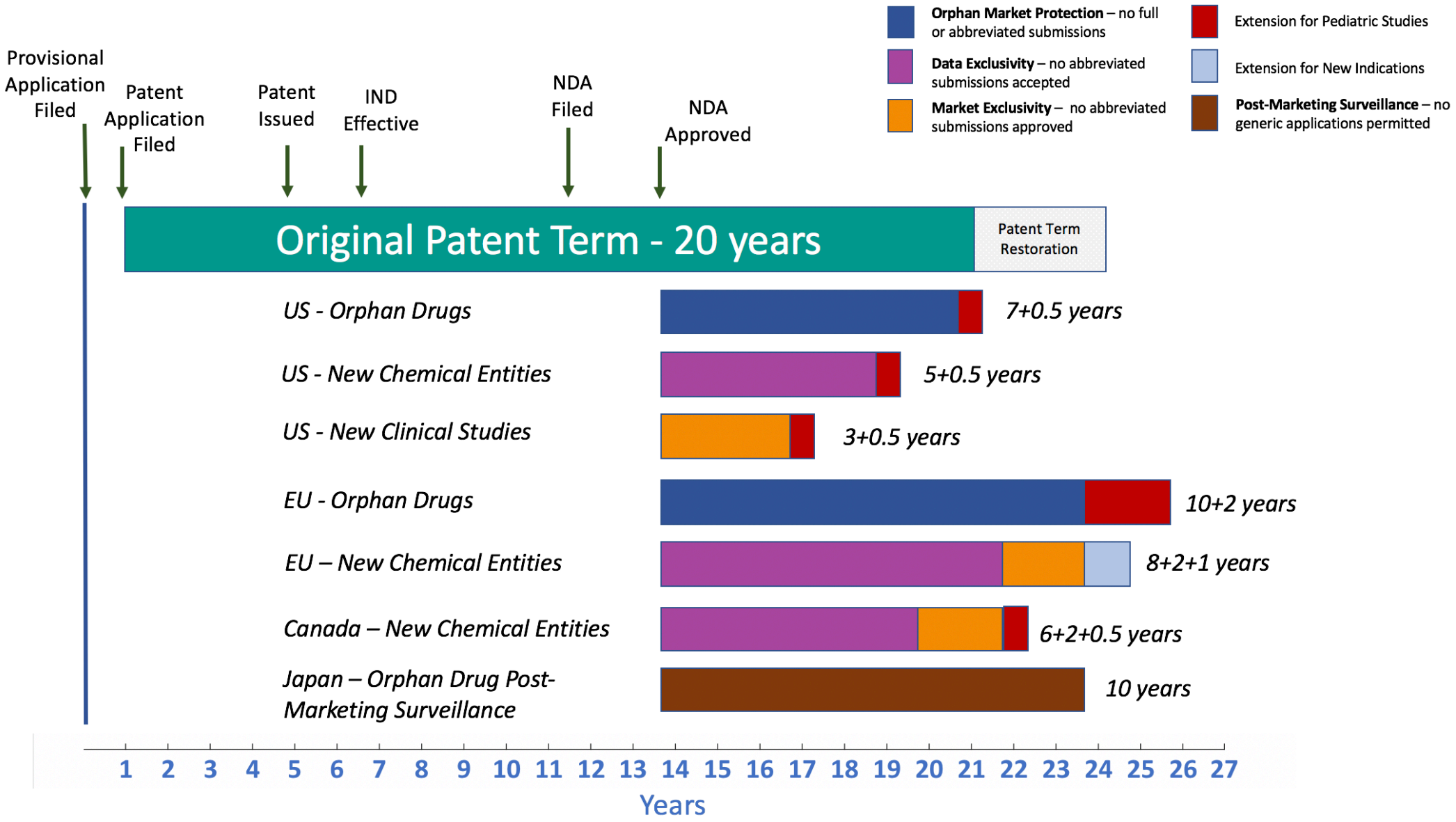
ASSUMPTIONS	
Patient Population	2,000 **OR** 500 (25% coverage)
Market Exclusivity	12 years (patent)
Price Per Patient Per Year	$105,000 **OR** $420,000 (25% coverage)
Investment Decision Point	Early Preclinical
OUTCOMES	
Nominal Profit/Loss	$1,956,500,000
Net Present Value	$630,127
Business Case for Investment?	**YES**

Open Science as a Solution to these Challenges

Hypotheses:

- Innovation networks based on 'open science' can be used to reduce the cost and risk of drug development against novel targets and make small market indications viable at affordable pricing
  - Alternative non-dilutive capital: foundations, public grants, corporate donations
  - Leverage in-kind scientific contributions from motivated academics, clinicians, and CROs
- If discovery and development costs reduced -> sustainable pricing can be lower
- Alternative IP assets (regulatory data and market exclusivities) can attract industry partners to take-up de-risked assets through registration, manufacturing, and distribution

Regulatory Exclusivity as Primary Market Asset



Regulatory Exclusivity vs. Patents

Regulatory exclusivities:

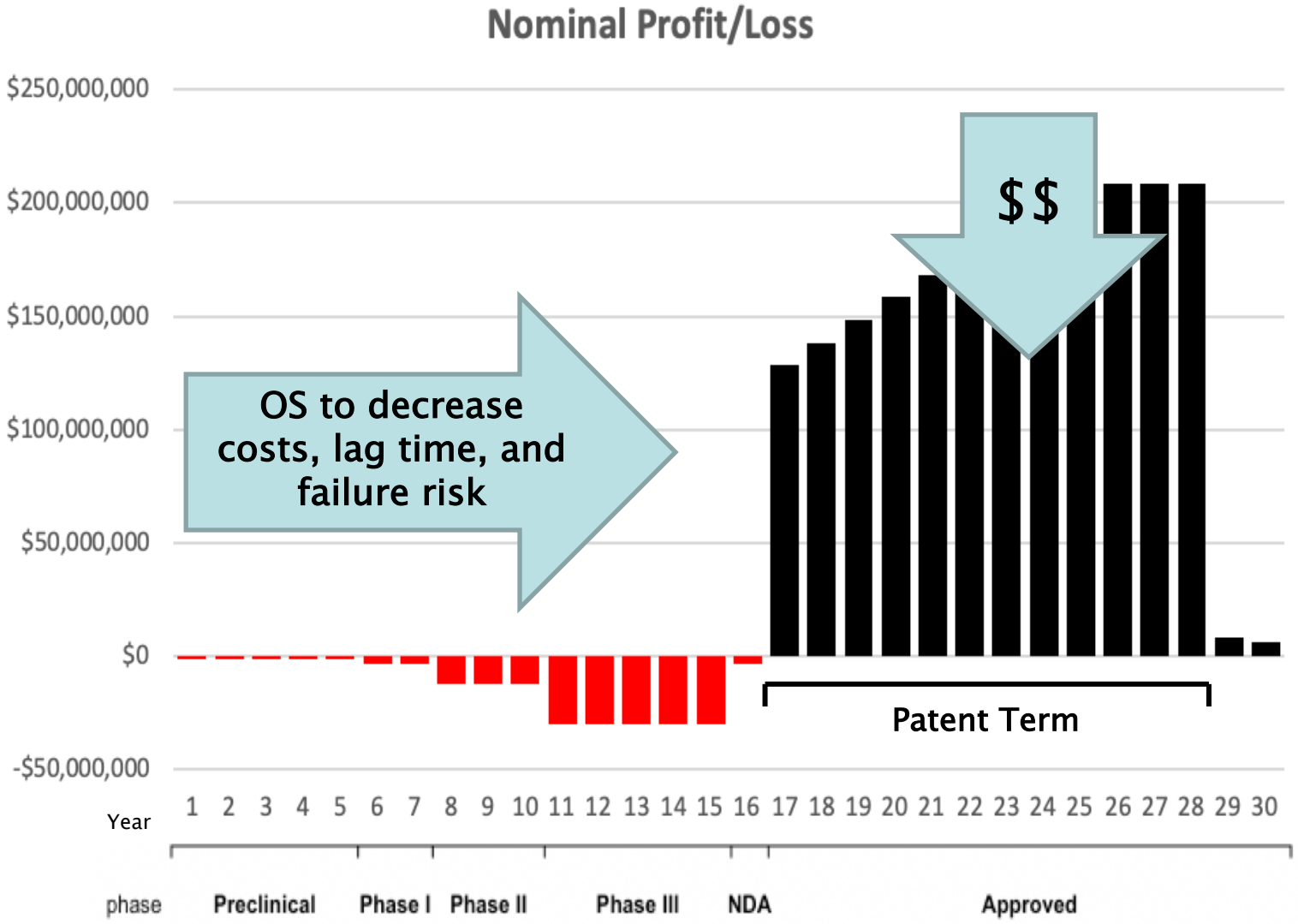
- Like patents, shield a product from generic competition
- **Consistent with open science:**
  - Are not invalidated by prior disclosure/sharing/collaboration
- Virtually **costless** to obtain and enforce
- **Not subject to challenge** by competitors
- Provide a period of market protection that is **certain *ex ante***

Orphan Drug Act  rare disease innovation with new exclusivity protection but no effect on patents

Many NCEs approved by FDA with exclusivity protection but no patents after Hatch-Waxman Act

Trade negotiators and pharma lobby spend much time pursuing expanded data protection laws in other jurisdictions

OS Can Create an Affordable Drug for this Opportunity



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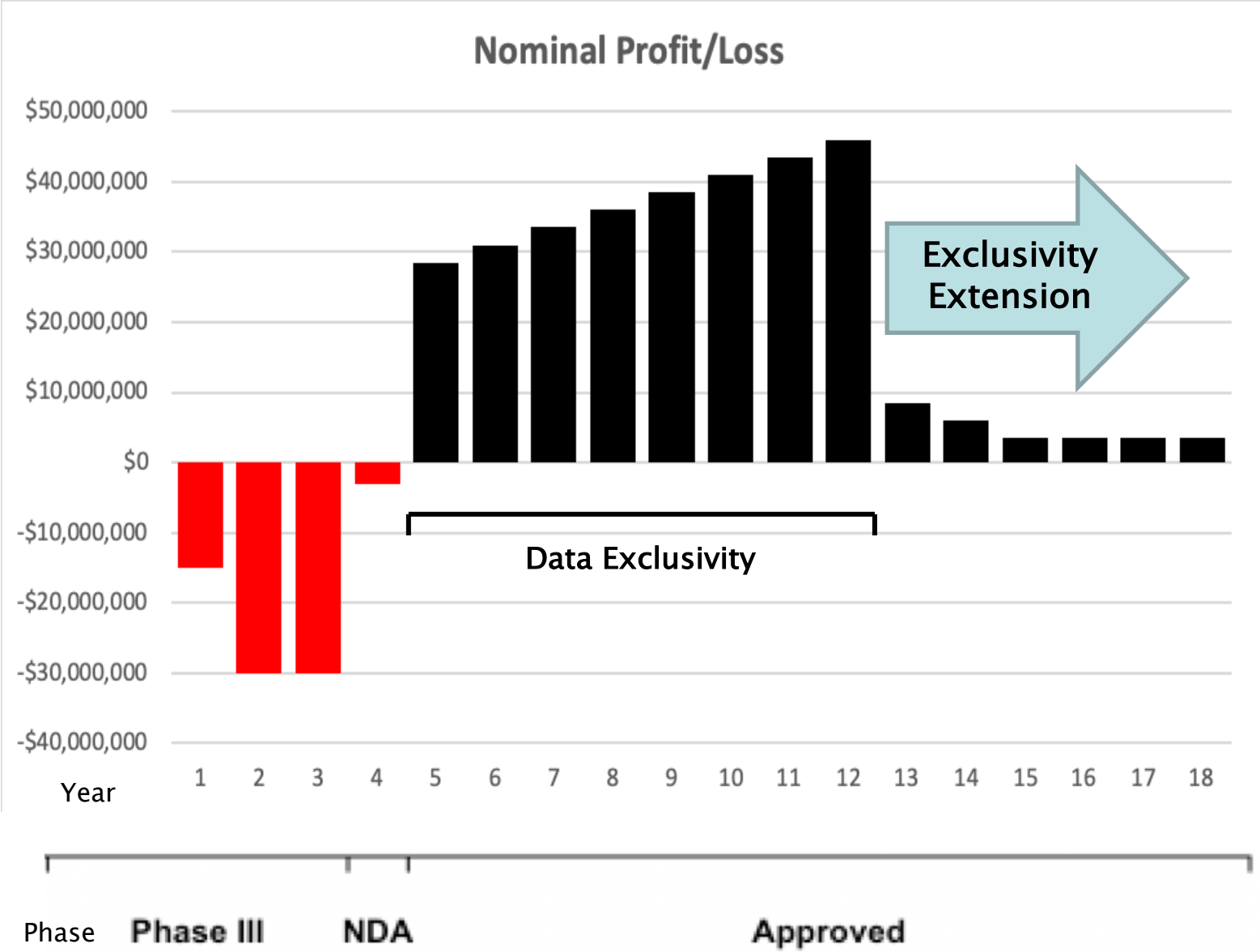
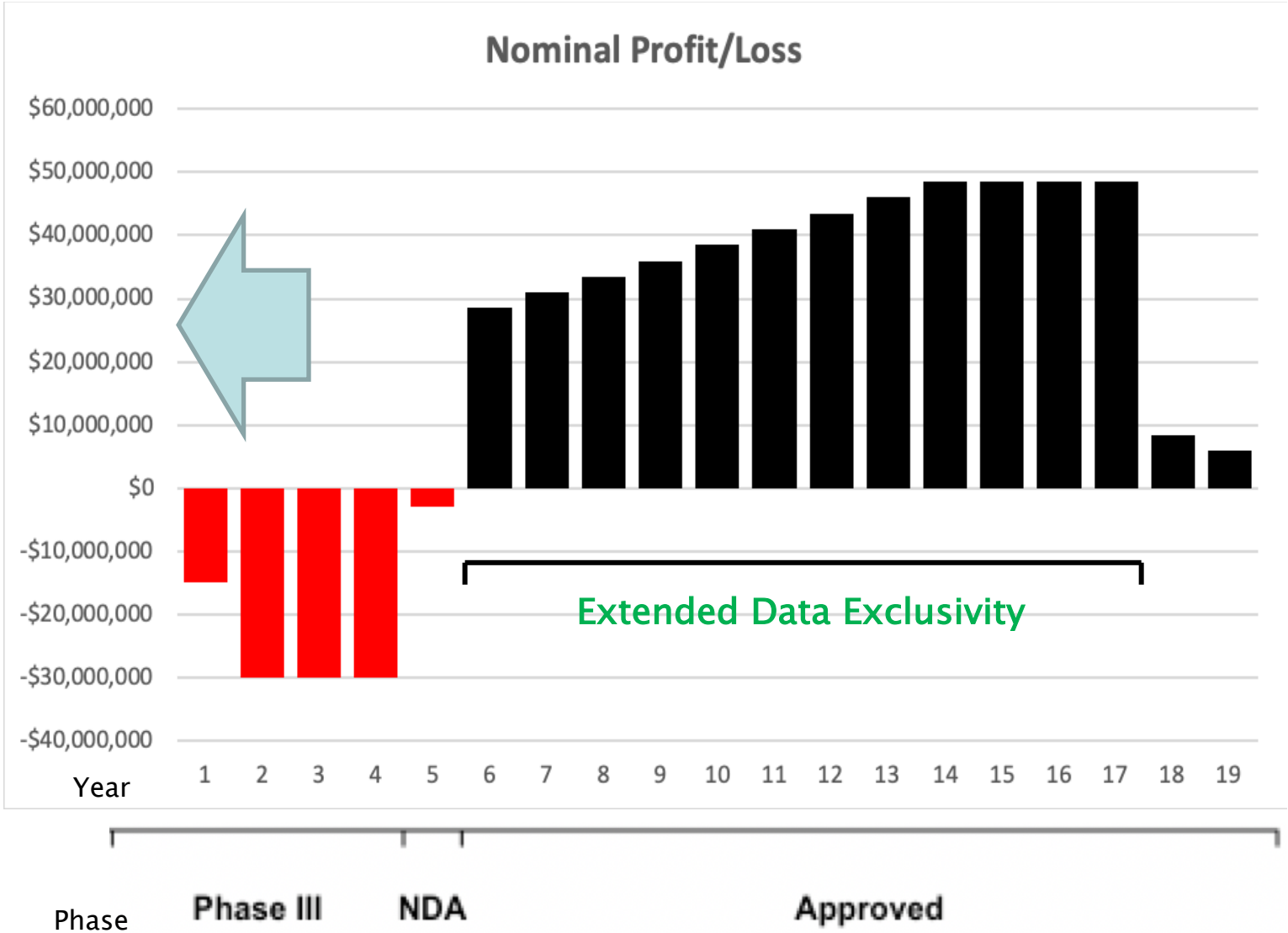


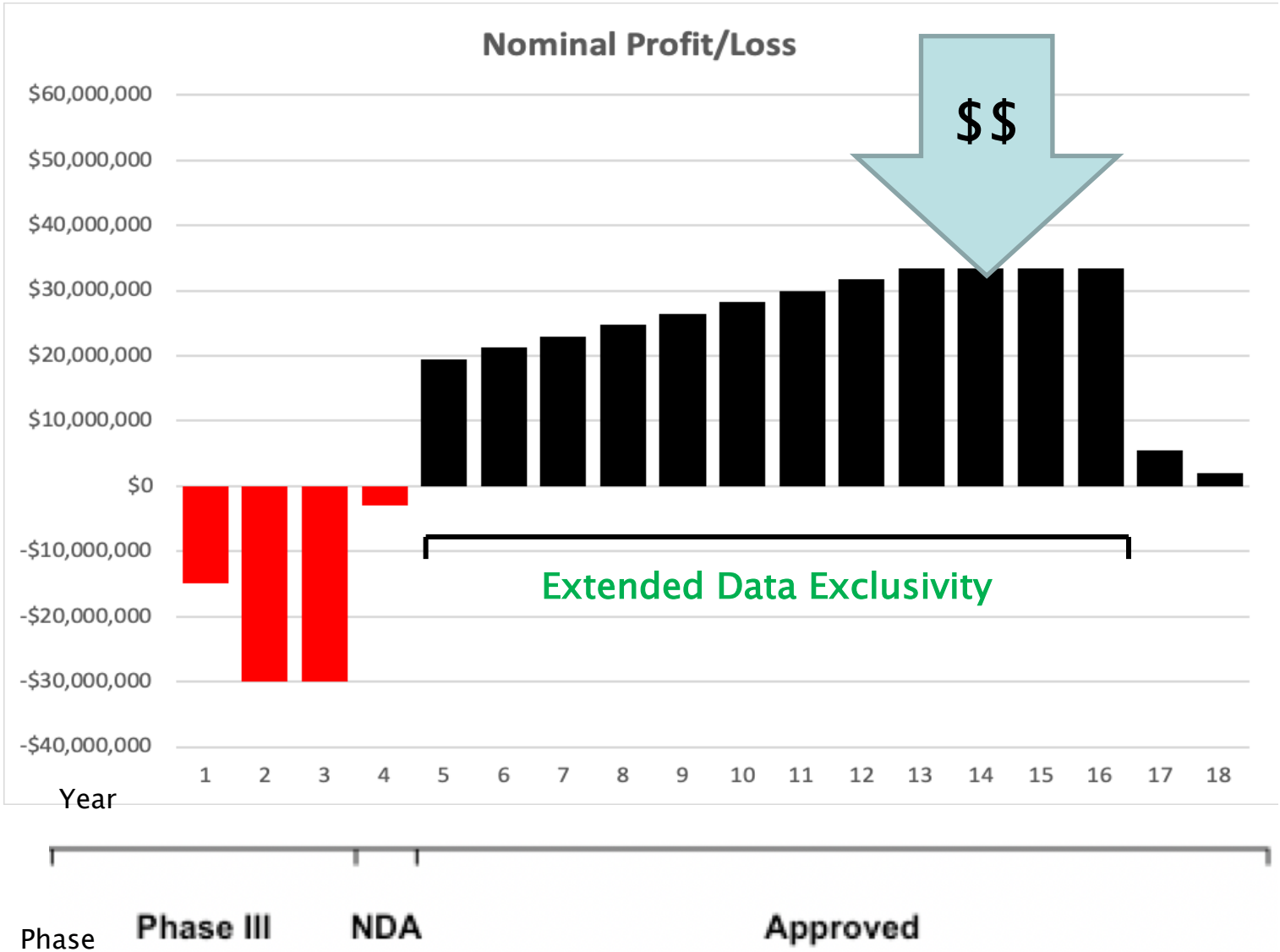
ASSUMPTIONS	
Patient Population	2,000
Market Exclusivity	8 years (data exclusivity only)
Price Per Patient Per Year	$25,000
Investment Decision Point	Mid-Phase III
OUTCOMES	
Nominal Profit/Loss	$248,500,000
Net Present Value	$13,806,000
Business Case for Investment?	YES

Effect of Extended Data Exclusivity for OS?
Attract Earlier Investment …



ASSUMPTIONS	
Patient Population	2,000
Market Exclusivity	12 years (data exclusivity with OS extension)
Price Per Patient Per Year	$25,000
Investment Decision Point	Early Phase III
OUTCOMES	
Nominal Profit/Loss	$398,500,000
Net Present Value	$10,476,500
Business Case for Investment?	**YES**

… OR … Enable Negotiation of Further Pricing Concessions!



ASSUMPTIONS	
Patient Population	2,000
Market Exclusivity	12 years (data exclusivity with OS extension)
Price Per Patient Per Year	$16,000
Investment Decision Point	Mid-Phase III
OUTCOMES	
Nominal Profit/Loss	$268,500,000
Net Present Value	$678,500
Business Case for Investment?	**YES**

Exclusivity extensions have been used to incentivize desirable pharma R&D investments

- Pediatric studies
  - 6-month extensions in Canada and US
  - 2-year extension for orphan exclusivity in EU

- Generating Antibiotic Incentives Now (GAIN) Act
  - 5-year extension for new antibiotics treating serious or life-threatening infections

Proposal: Extend C.08.004.1 Innovative Drug Status by 4 Years

- Condition 1: Data from preclinical and clinical studies made available for research use within specified period
  - Reduces R&D redundancy; improves reproducibility and public trust
  - More rapid secondary and meta-analyses, new hypothesis generation
  - Input to HTA and reimbursement decision-making; improves knowledge base for prescribers and patients.
- Condition 2: No patents on medicine; submission of patent list precluded; patent suits waived
  - Enhances the public domain and precludes evergreening tactics
- Condition 3: Price ceiling based on cost effectiveness in HTA or independent pharmacoeconomic analysis
  - E.g. CADTH; Leeway for HC to develop Guidance
  - Improve system uptake and access for patients; reduce financial burden

Proposed Mechanism

- Submission of information or certification to Minister
  - (4.1)(a)–(c)
- Ministerial determination of compliance at 6 years
  - (4.1)(d)
- Extension revoked if Minister later determines that compliance has ceased
  - (5.1)(a)–(c)
- Minister granted authority to require relevant information and documents
  - (5.1)(d)

The Minister Has Statutory Authority

- *Canadian Generic Pharmaceutical Assn. v. Canada (Minister of Health)*, 2010 FCA 334
  - Parliament has broad power to delegate to Governor in Council within enabling legislation (para. 63)
  - "Very broad latitude" to enact regulations Governor in Council "deems necessary" to implement s. 30(3) of Food & Drugs Act (NAFTA, TRIPS) (para. 64, 85)
  - Unless "bad faith", Courts will not second guess Governor in Council's means to implement
  - Not limited to trade secrets; instead protects against "unfair commercial use" of data created by innovators (para. 73-74)

The Minister Has Statutory Authority

Other sub-sections of s. 30 of the Food & Drugs Act

30(1) The Governor in Council may make regulations ...

(r) respecting marketing authorizations, including establishing the eligibility criteria for submitting an application

(1.2)(a) respecting the issuance of authorizations ... [for] the ... sale … of a therapeutic product

(1.2)(b) authorizing the Minister to impose terms and conditions on [such] authorizations

(1.2)(d.1) specifying the business information obtained under this Act in relation to an authorization … that is not confidential business information.

The Minister Has Constitutional Authority

- *Canadian Generic Pharmaceutical Assn. v. Canada (Minister of Health)*, 2010 FCA 334
  - Pith and substance of C.08.004.1: market protection exists "to encourage the development of new drugs, which … constitutes a valid public health and safety purpose" (para. 113)
  - This is a valid exercise of federal criminal law power under 91(27) of the *Constitution Act*
    - "The scope of the federal power to create criminal legislation with respect to health matters is broad, and is circumscribed only by the requirements that the legislation must contain a prohibition accompanied by a penal sanction and must be directed at a legitimate public health evil" (para. 119)
  - Food & Drugs Act s. 31 creates an offence if a person sells or advertises a new drug without authorization

Let's Continue to Lead in Canada!

SGC

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OPEN SCIENCE TRUST

M4K PHARMA
Open Science for Children's Health

Mission

At M4K Pharma, we are using open science to revolutionize how affordable new treatments are discovered and developed. Through our Medicines4Kids program, we will aggregate and align the work of global academics, foundations and industry researchers to advance new cures for childhood diseases not well-served by current business models.

neuro
Institut et hôpital neurologiques de Montréal
Montreal Neurological Institute and Hospital

Innovation Through Open Science

The Montreal Neurological Institute and Hospital is embarking on a journey to become the first Open Science Institute in the world. The objective is to expand the impact of brain research and accelerate the discovery of ground-breaking therapies to treat patients suffering from a wide range of devastating neurological diseases.

Open For Rare

GOAL OF OPEN FOR RARE

The Open For Rare research program applies open science principles to develop and operate a high-output, low-cost biomarker and drug discovery pipeline for rare genetic diseases.

Contact

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