

# Affordability of Curative Therapies

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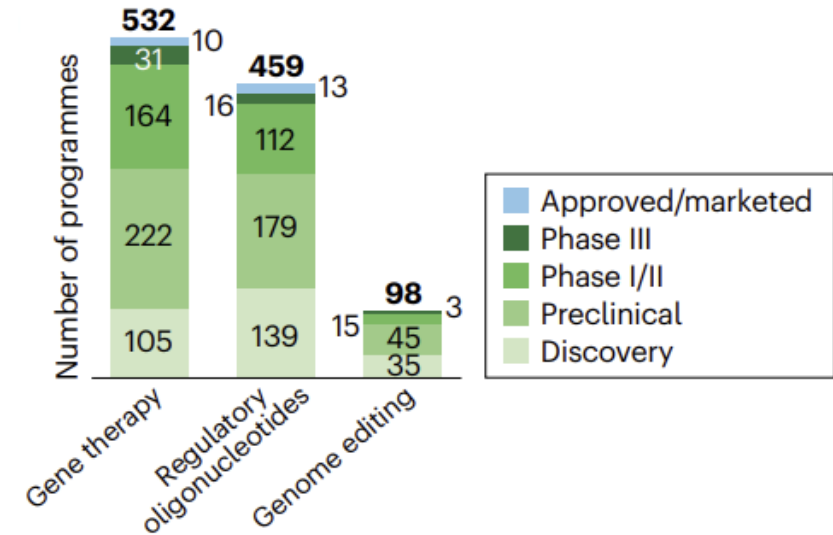
University of Washington, Seattle, WA

**ISPOR EUROPE 2023**

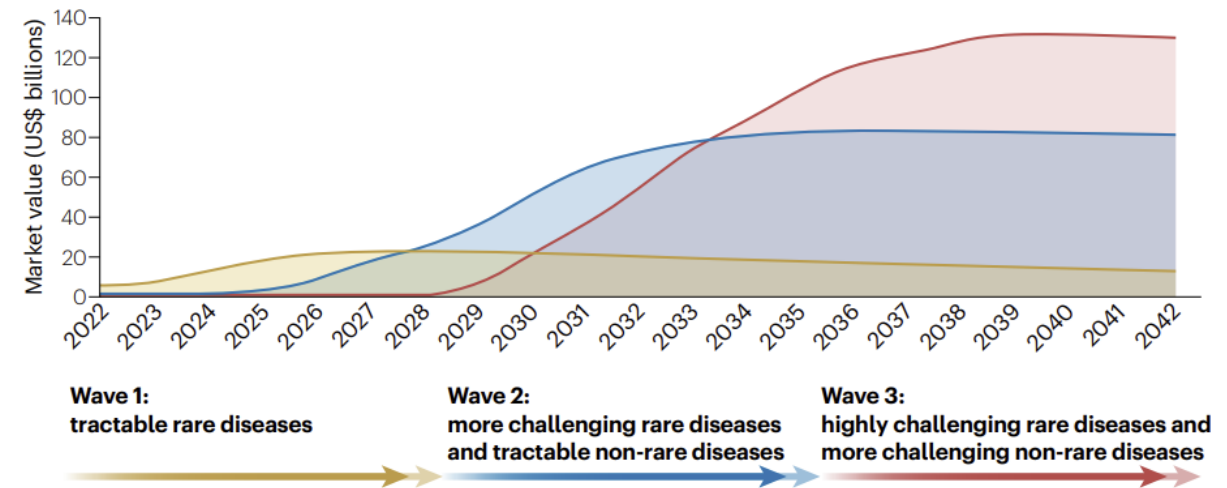
# Disclosure

- This work funded by the National Heart, Lung, and Blood Institute (OT3HL152448)
- Consulting with Salutis Consulting LLC outside the scope of this work.

# Advent of Genomic Medicine



- Only ~20 genomic medicine products have been approved so far
- These treatments currently target <0.1% of the global population with genetic disorders
- There are ~1,100 active clinical trials of genomic medicines



# Assessment & Affordability of Genomic Medicine

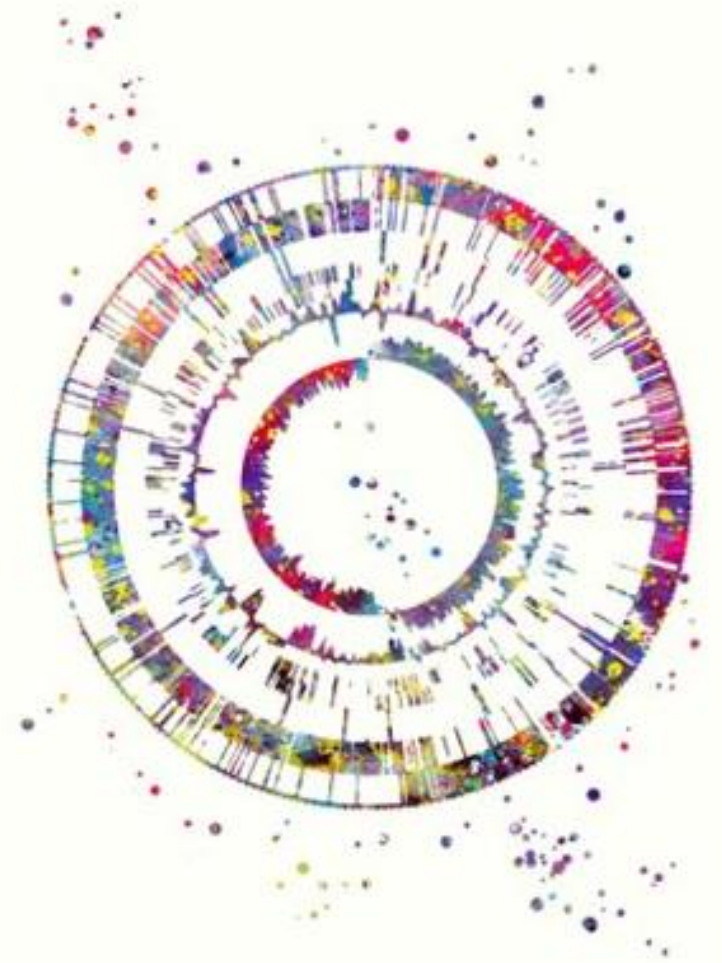
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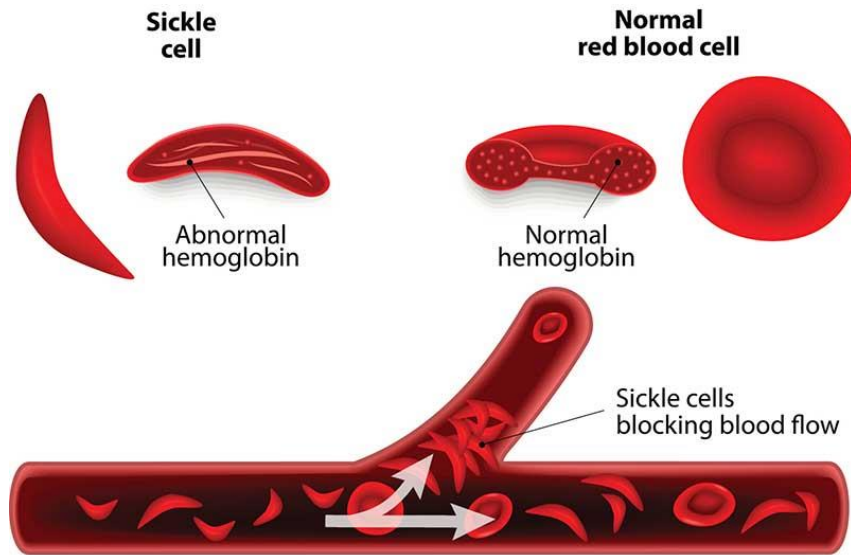
Genetic Cure

Sickle Cell Disease

US Context

Pricing & Budget Impacts





## Sickle Cell Disease

- Most prevalent genetic blood disorder in the United States ~ about 100,000 patients
  - Disproportionately affects African heritage and Latino populations
- Characterized by deformed ("sickle"-shaped) red blood cells that fail to adequately deliver oxygen to body tissues and cause blockages in blood flow
- Causes extraordinarily painful and severe attacks, known as crises, and a high burden of other health conditions.

# Genomic Cures

Undergoing FDA priority review in December 2023

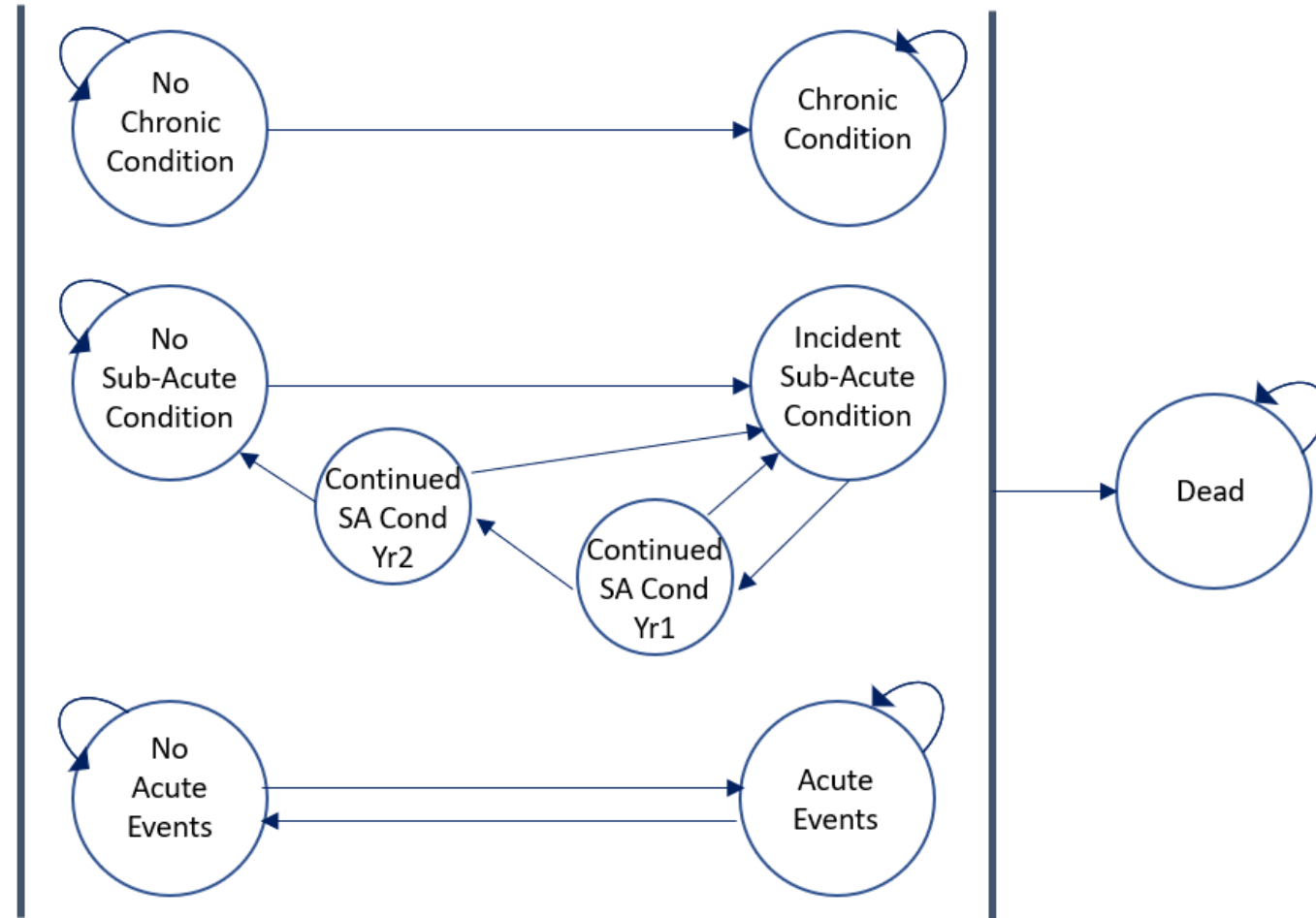
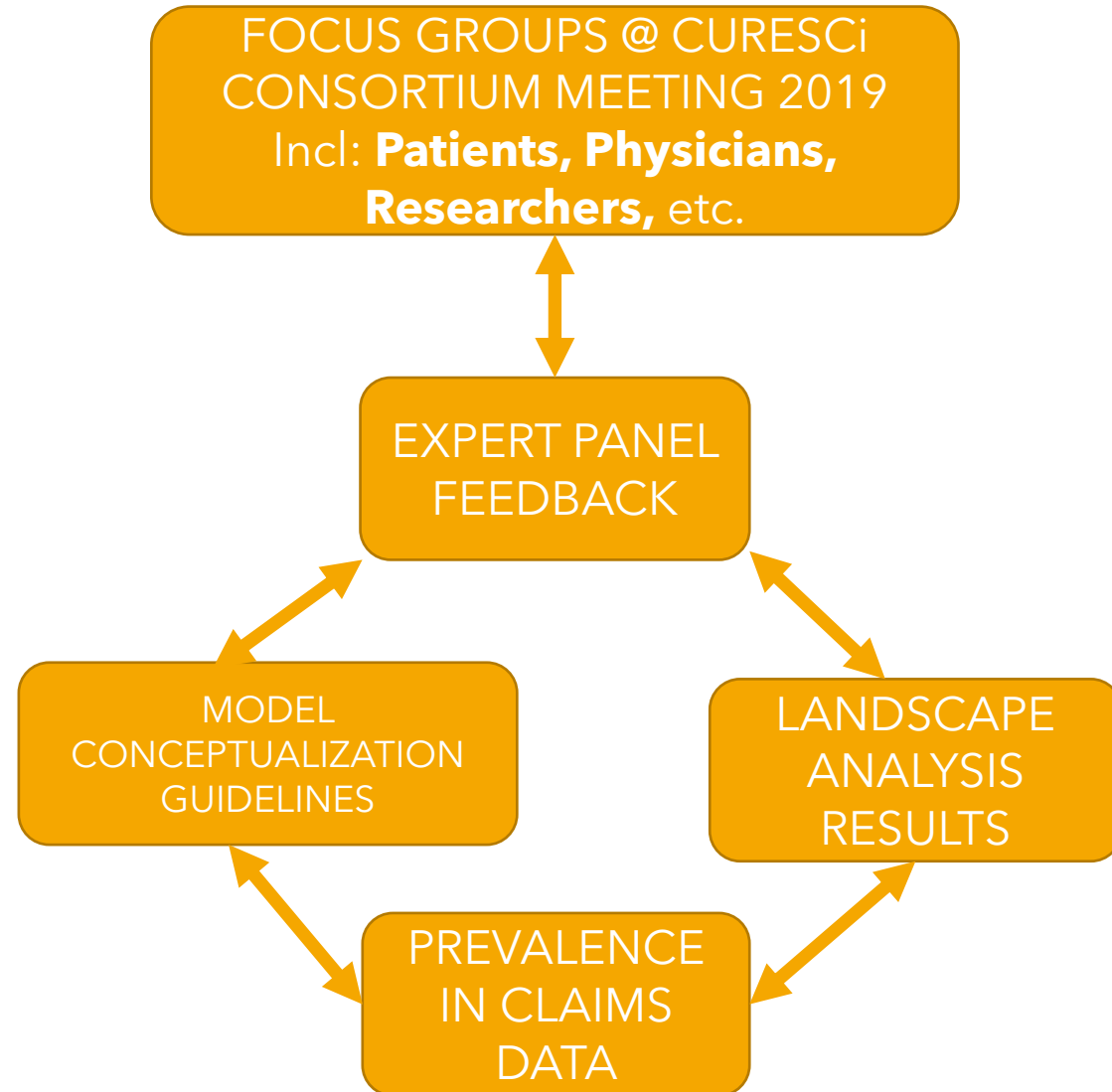
## **lovo-cel (Blubird Bio)**

Treat the underlying cause of SCD through the addition of functional copies of a modified form of the  $\beta$ -globin gene ( $\beta$ A-T87Q-globin gene) into a patient's own hematopoietic (blood) stem cells (HSCs)

## **exa-cel (CRISPR & Vertex)**

Patient's own hematopoietic stem cells are edited to produce high levels of fetal hemoglobin (HbF; hemoglobin F), the naturally occurring form of the oxygen-carrying hemoglobin in fetal stage, in red blood cells.

# UW MODEL for Economic Analysis of Sickle Cell Cure (UW-MEASURE) Conceptualization



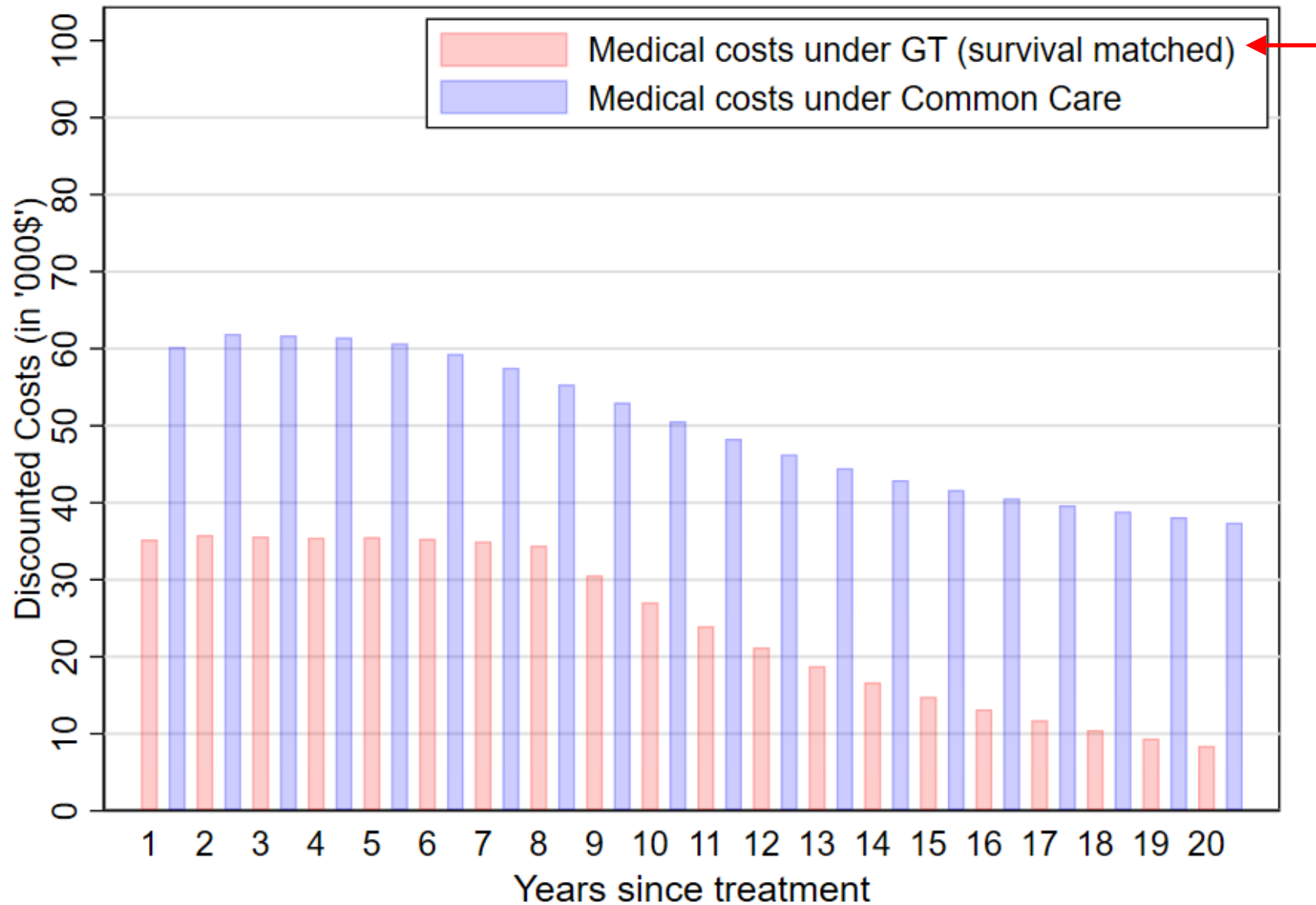
| Outcomes+                      | LentiGlobin | Common Care | Incremental       | 7              |
|--------------------------------|-------------|-------------|-------------------|----------------|
| Life Expectancy (undiscounted) | 30.8 (3.7)  | 13.4 (2.1)  | 17.4 [10.5, 24.3] |                |
| Healthcare Sector Perspective  | LentiGlobin | Common Care | Incremental       | ICER (\$/QALY) |
| QALYs (P+F)                    | 30.6 (2.9)  | 18.7 (1.9)  | 11.9 [8.5, 15.3]  |                |
| Total Medical Costs            | \$3.50M     | \$1.20M     | \$2.30M           | \$193,175      |
| Societal Perspective           | LentiGlobin | Common Care | Incremental       | ICER (\$/QALY) |
| QALYs (P+F)                    | 30.6 (2.9)  | 18.7 (1.9)  | 11.9 [8.5, 15.3]  |                |
| Total Costs                    | \$2.04M     | \$0.54M     | \$1.50M           | \$125,964      |
| Healthcare Sector Perspective  | LentiGlobin | Common Care | Incremental       | ICER (\$/HYT)  |
| Health Years in Total          | 48.7 (4.1)  | 29.0 (3.0)  | 19.7 [14.4, 25.0] |                |
| Total Medical Costs            | \$3.50M     | \$1.20M     | \$2.30M           | \$116,689      |
| Societal Perspective           | LentiGlobin | Common Care | Incremental       | ICER (\$/HYT)  |
| Health Years in Total          | 48.7 (4.1)  | 29.0 (3.0)  | 19.7 [14.4, 25.0] |                |
| Total Costs                    | \$2.04M     | \$0.54M     | \$1.50M           | \$76,089       |



# Cost Breakdown

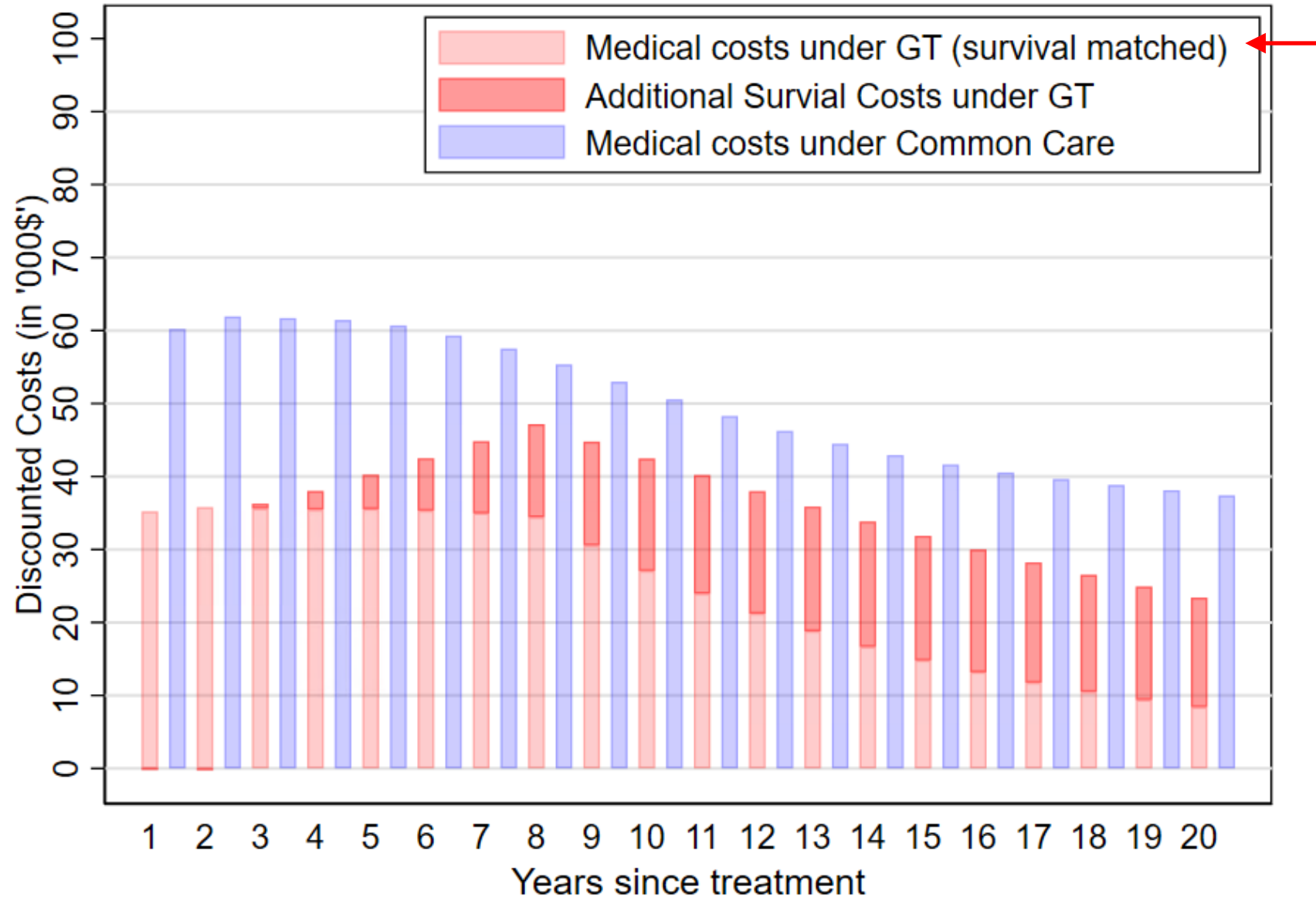
| Cost Components             | Gene Therapy                   | Common Care        | Difference         |
|-----------------------------|--------------------------------|--------------------|--------------------|
| LentiGlobin Drug Price      | \$2,000,000                    | -                  | \$2,000,000        |
| LentiGlobin Adm & Monit     | \$ 470,809                     | -                  | \$ 470,809         |
| Other Medical Costs         | \$1,025,095                    | \$1,197,111        | -\$ 172,016        |
| <b>TOTAL MEDICAL COSTS</b>  | <b>\$3,495,891</b>             | <b>\$1,197,111</b> | <b>\$2,298,780</b> |
|                             |                                |                    |                    |
| Medical Time Costs          | \$ 17,633                      | \$ 46,253          | -\$ 28,620         |
| Caregiving Time Costs       | \$ 44,499                      | \$ 63,534          | -\$ 19,035         |
| Net Productivity            | -\$1,519,296                   | -\$ 767,459        | -\$ 751,837        |
| <b>TOTAL SOCIETAL COSTS</b> | <b>\$2,038,410</b>             | <b>\$ 539,439</b>  | <b>\$1,498,971</b> |
|                             |                                |                    |                    |
|                             | Genomic Medicine Affordability |                    |                    |

# Average discounted costs in first 20 years



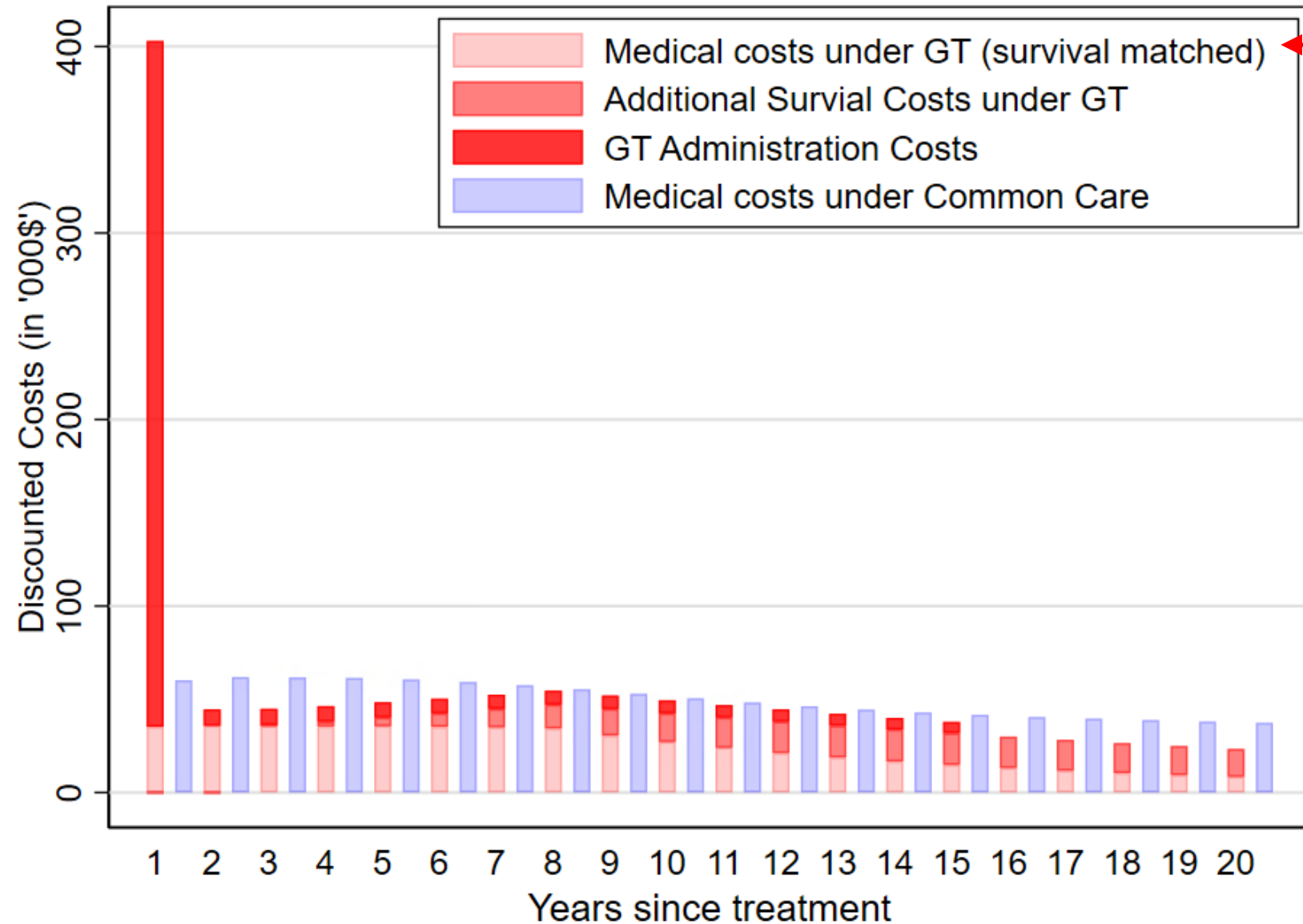
- Survival matched
- No administrative costs
- No gene therapy costs

# Average discounted costs in first 20 years



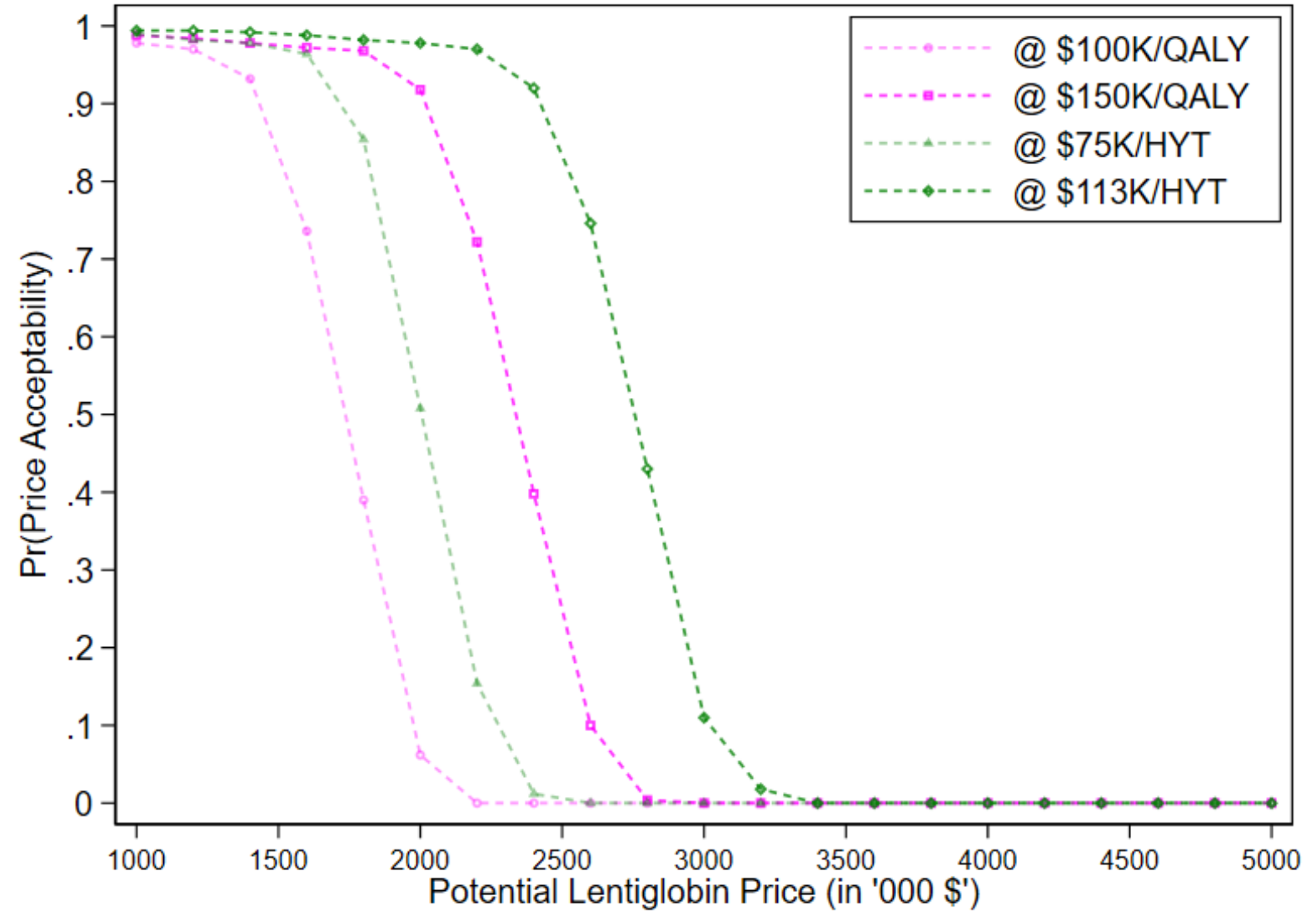
- No administrative costs
- No gene therapy costs

# Average discounted costs in first 20 years



- No gene therapy costs

# Price Acceptability Curves





# Budget Impact



**CMS  
Perspective**

# Cohorts

| Insurance cohorts     | Eligible for GT | Without exclusion criteria |
|-----------------------|-----------------|----------------------------|
| Medicaid              | 2322 (49)       | 4343 (44)                  |
| Dual Eligible         | 2376 (50)       | 5320 (54)                  |
| Medicare (Disability) | 64 (1)          | 113 (1)                    |
| Total                 | 4,762           | 9,776                      |

Trial Target  
Population



Likely FDA-  
labeled Target  
Population



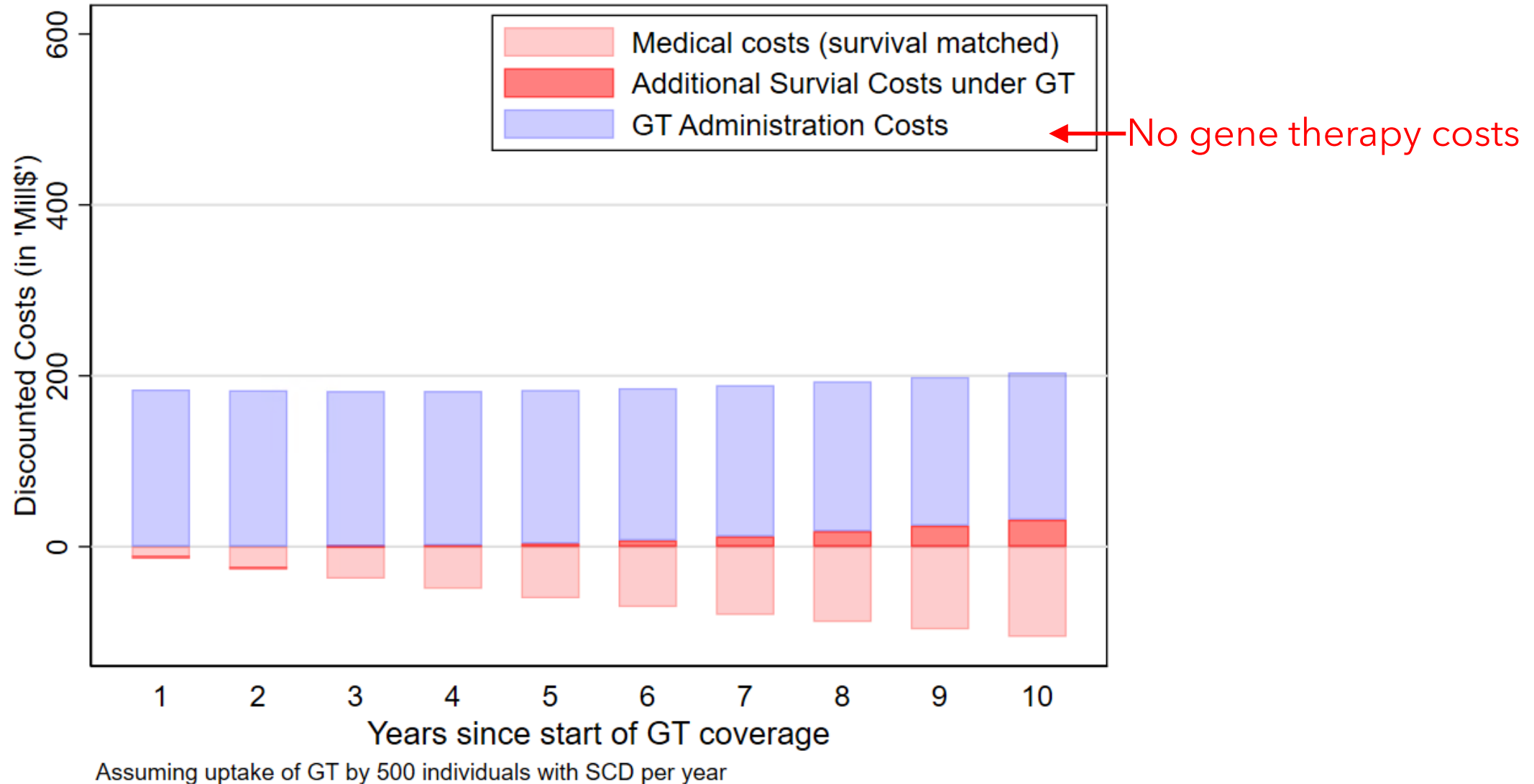
Assume uptake by  
500 individual/year

**CMS BUDGET  
IMPACT**



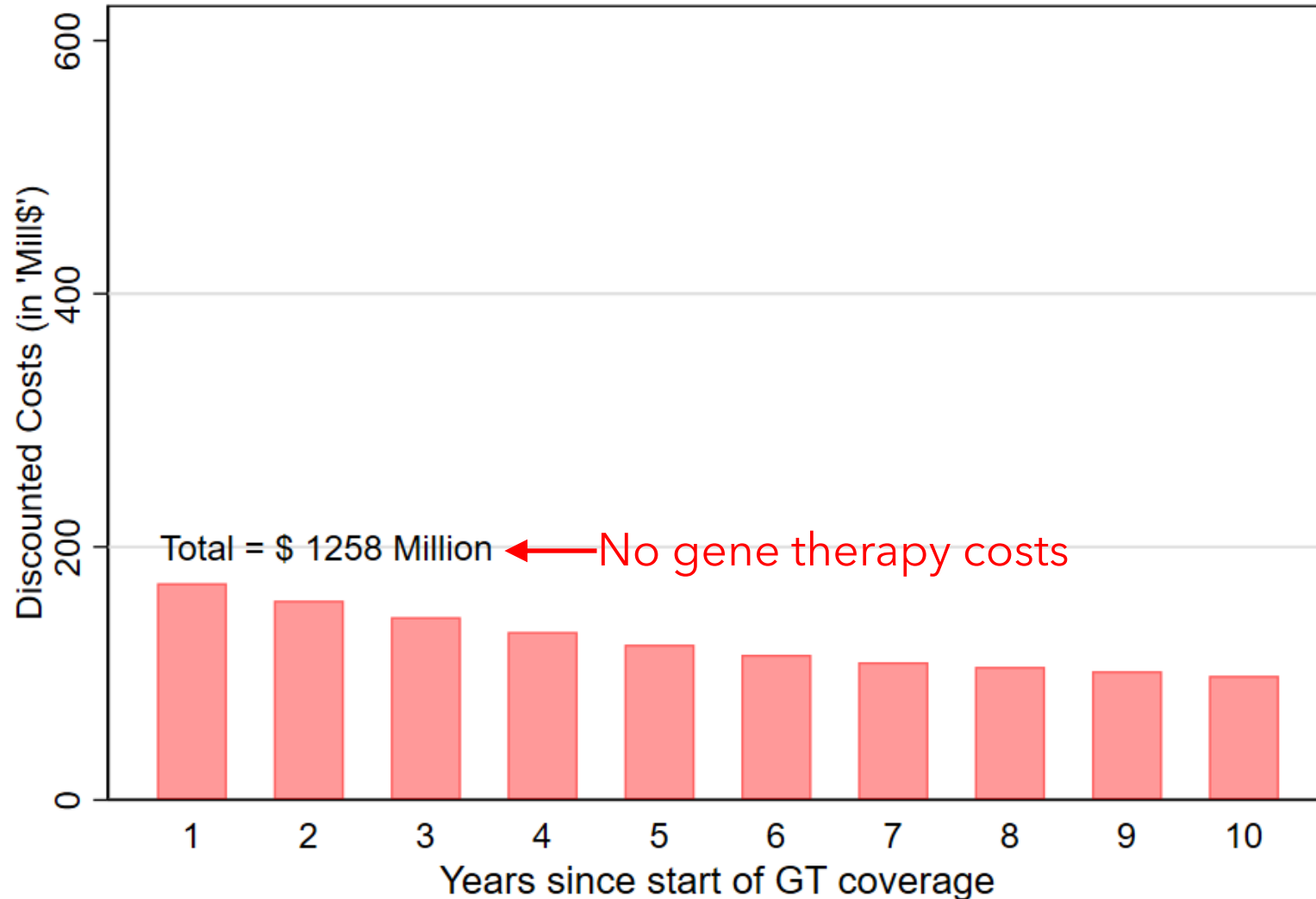
# CMS Budget Impact Components in first 10 years of coverage

16



# CMS Net Budget Impact in the first 10 years of coverage

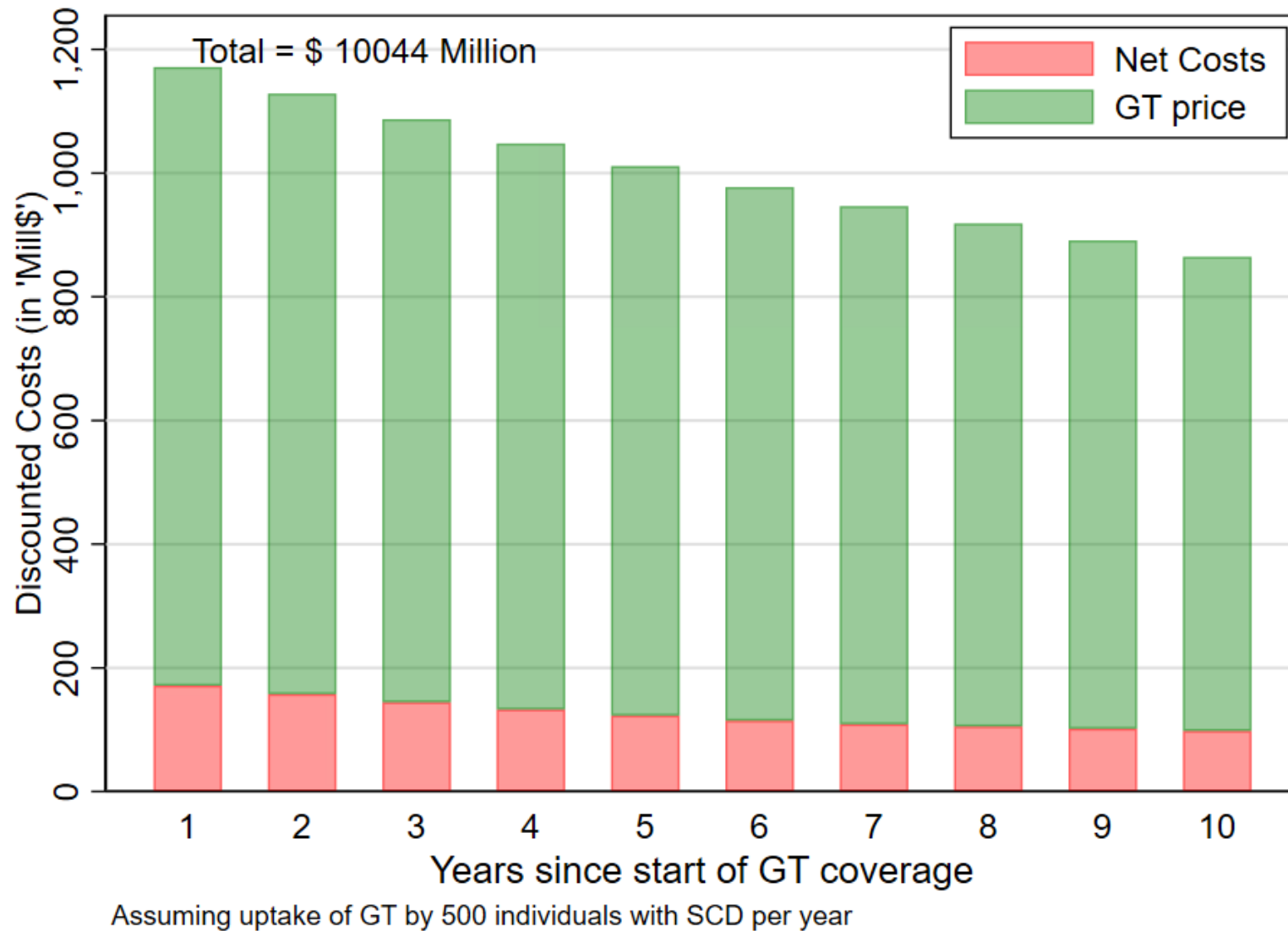
17



Assuming uptake of GT by 500 individuals with SCD per year

# CMS Net Budget Impact with front-loaded GT price in the first 10 years of coverage

18



Total # of Medicaid enrollees = 93 Million

% of enrollees who benefit annually: .0005%

Total Annual Spending by Medicaid: \$750 Billion

% of Annual Total Spending going to this cure: 0.1%

GT price assumed to be \$2 million

If genomic cures were used by 0.1% of the Medicaid population annually, Medicaid annual budget increases by 25% to \$1 Trillion

**Unsustainable Impact**

# Alternate Financing

- **Up-front**
- **Annuity**
- **Outcomes-based**

# Alternate Financing

## Traditional Up-Front

- \$2 Mill Price
- 10 -year NPV = \$10 Billion
- Year 1 NPV: \$1.2 Billion
- Year 5 NPV: \$1.0 Billion
- Year 10 NPV: \$865 Million
- Doesn't include the cost of borrowing

## Annuity

- \$234K/year annuity assures NPV of \$2 Mill price
- 10-year NPV = \$7.2 Billion
- Year 1 NPV: \$289 Mill
- Year 5 NPV: \$675 Mill
- Year 10 NPV: \$1.2 Billion

## Outcomes-based

- Pay full price in year 5 if the beneficiary is alive.
- \$2.25 Million price assures NPV of \$2 Mill price
- 10 -year NPV = \$6.8 Billion
- Year 1 NPV: \$171 Mill
- Year 5 NPV: \$1.1 Billion
- Year 10 NPV: \$962 Million

# Subscription-based

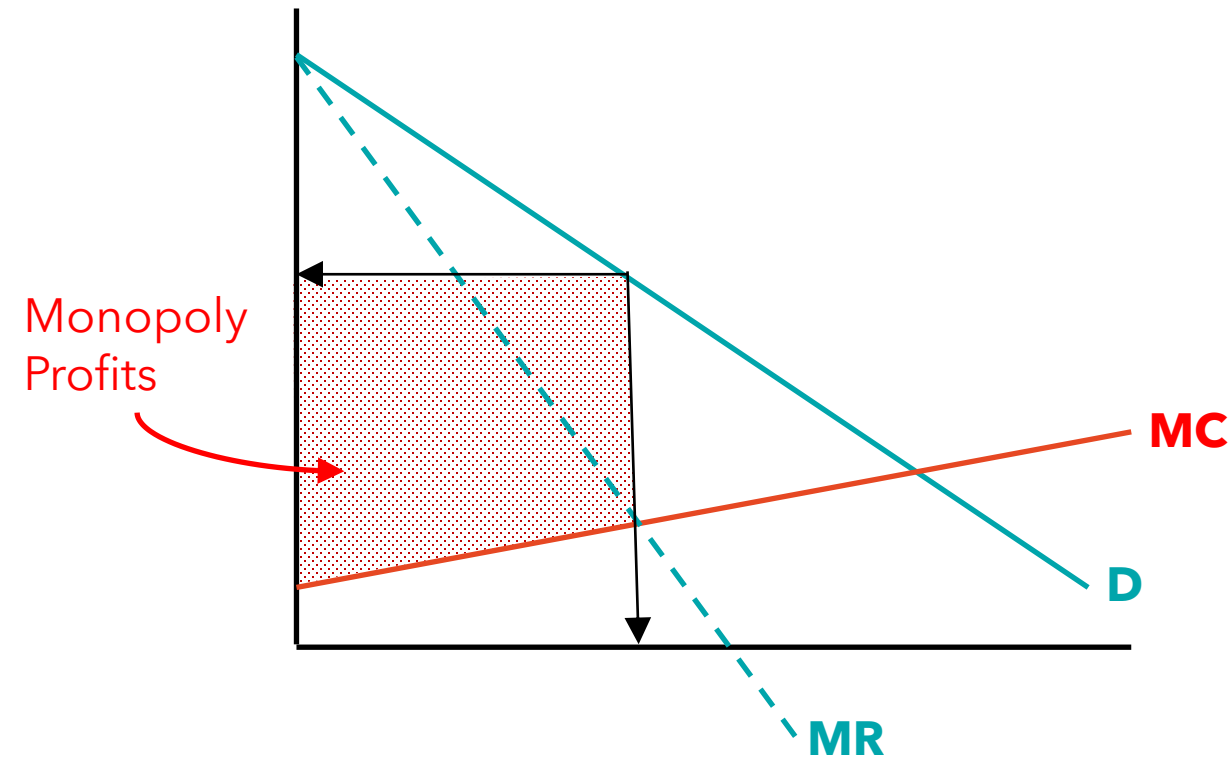
In the presence of competition

**True Netflix**

**National-auction**

**State-level auction**

# True Netflix



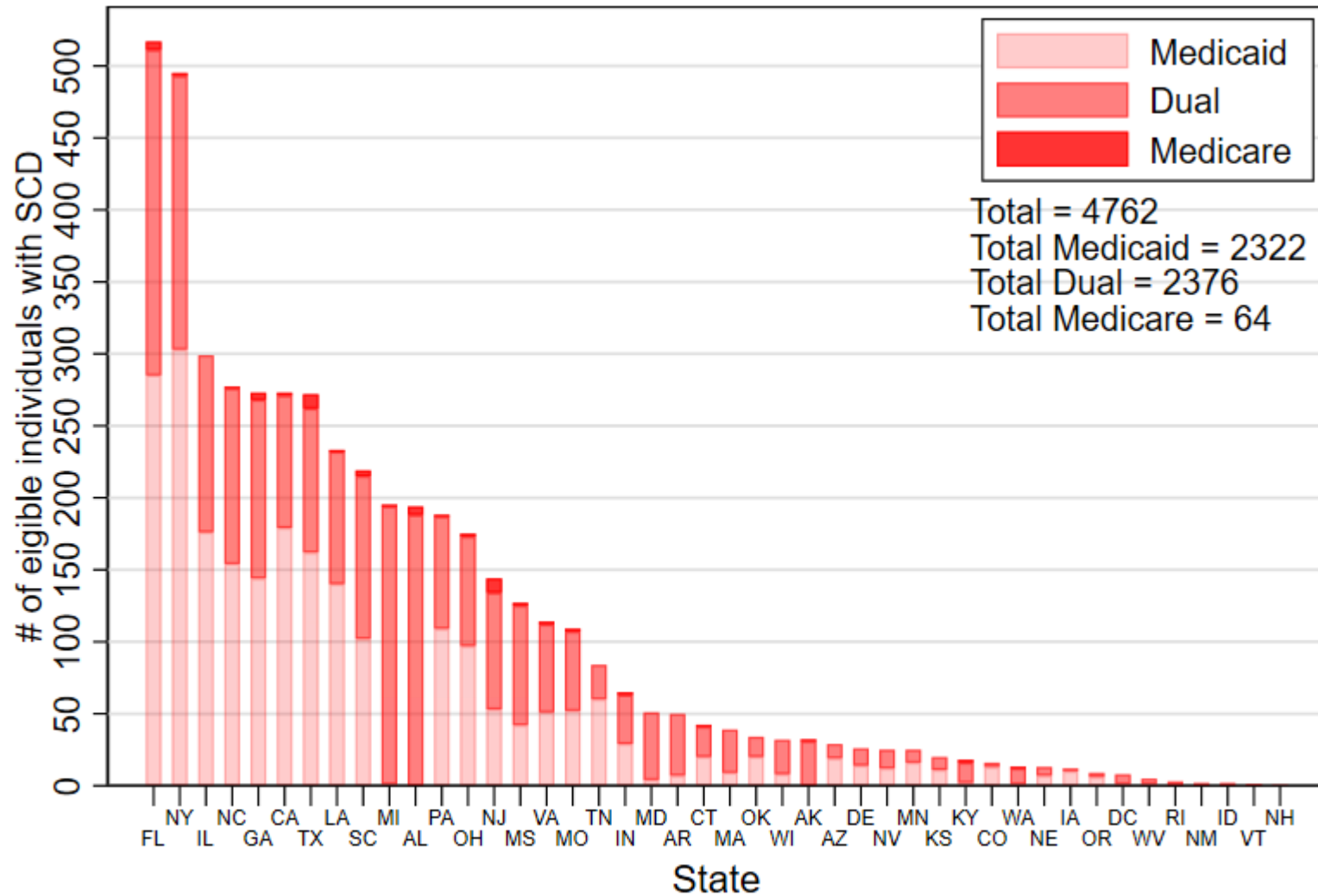
- Pay best estimate for monopoly profits upfront
- Get unlimited therapies at marginal costs over the next 10 years
- Shortages?



# National Auction with competitors

- e.g. CMS DME competitive bidding process
- Choose a winner based on the best price
- Generates very high risk for competitors (especially in duopolistic/oligopolistic markets) - unless the auction is repeated annually
- Incentive for reducing costs of manufacturing gene therapies

# Distribution of eligible population by state



Based on 2015/2016 Population

# National State-level Auctions

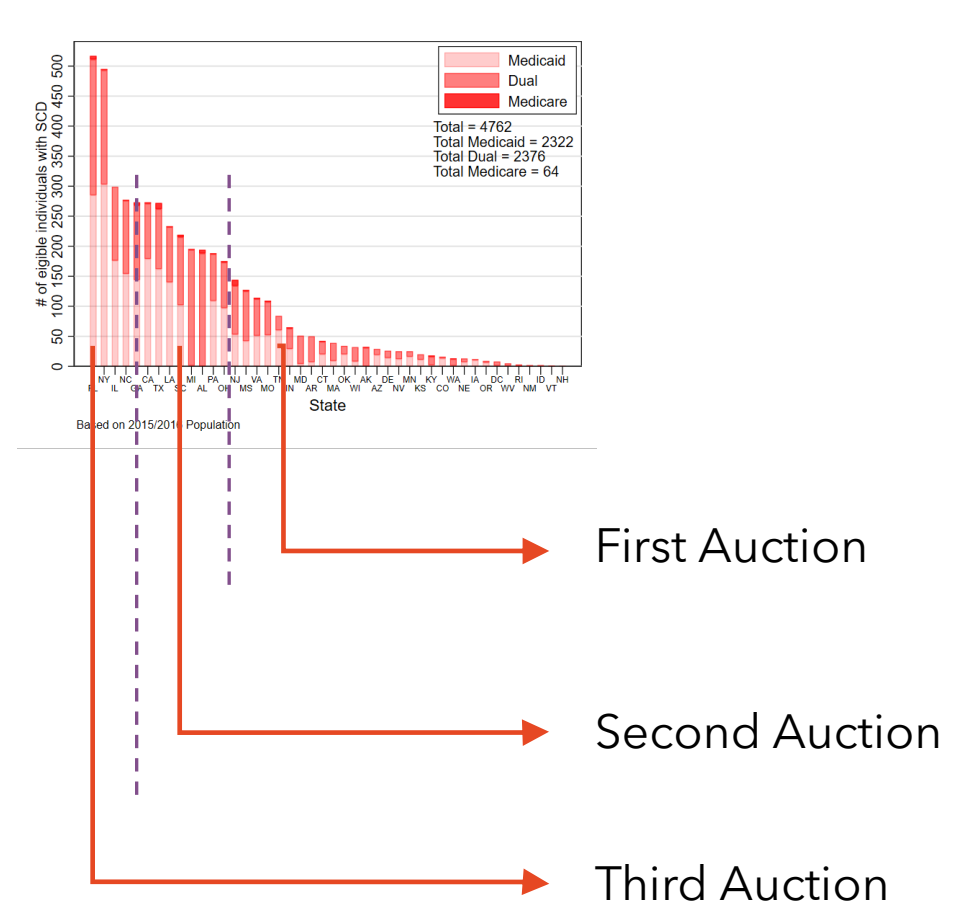
Multi-phases, closed-bids

Offer multi-year contracts to winners

Opportunity for losing firms to learn and update bid estimates for the next auction

Collusion? Give concessions to previous losers

Reward Pie increases with subsequent auctions



# Conclusions

- Likely to be a surge of genomic cures available in the next decade
- In most cases, value is unambiguous
- Affordability is questionable
- A sustainable healthcare system needs to come to terms with the opportunity costs of paying for genomic cures at the societal level.