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# Translational Science at NCATS

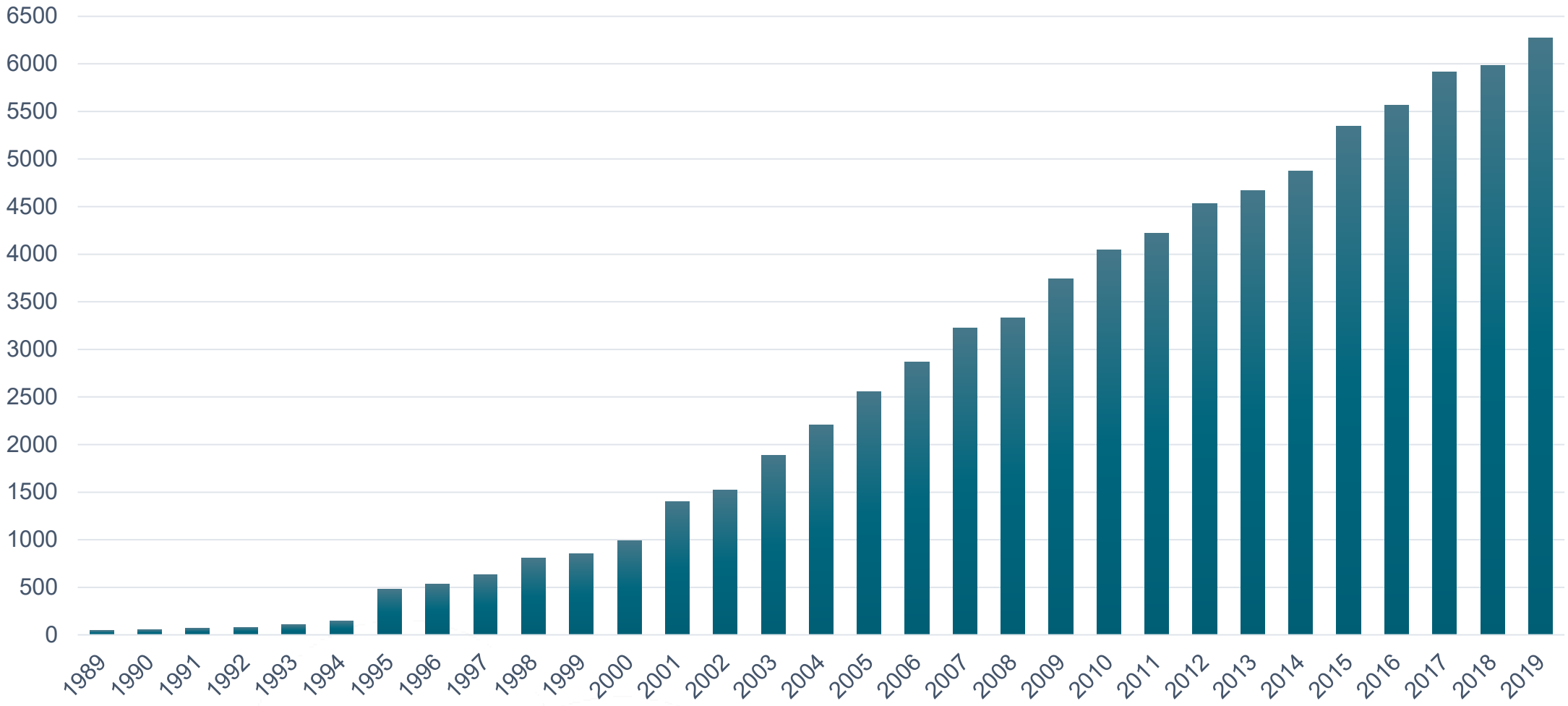
Joni L Rutter, PhD

*National Center for Advancing Translational Sciences*

*Acting Director*

NIH National Center for Advancing Translational Sciences

# Diseases with Known Molecular Basis




Source: Online *Mendelian Inheritance in Man*, Morbid Anatomy of the Human Genome

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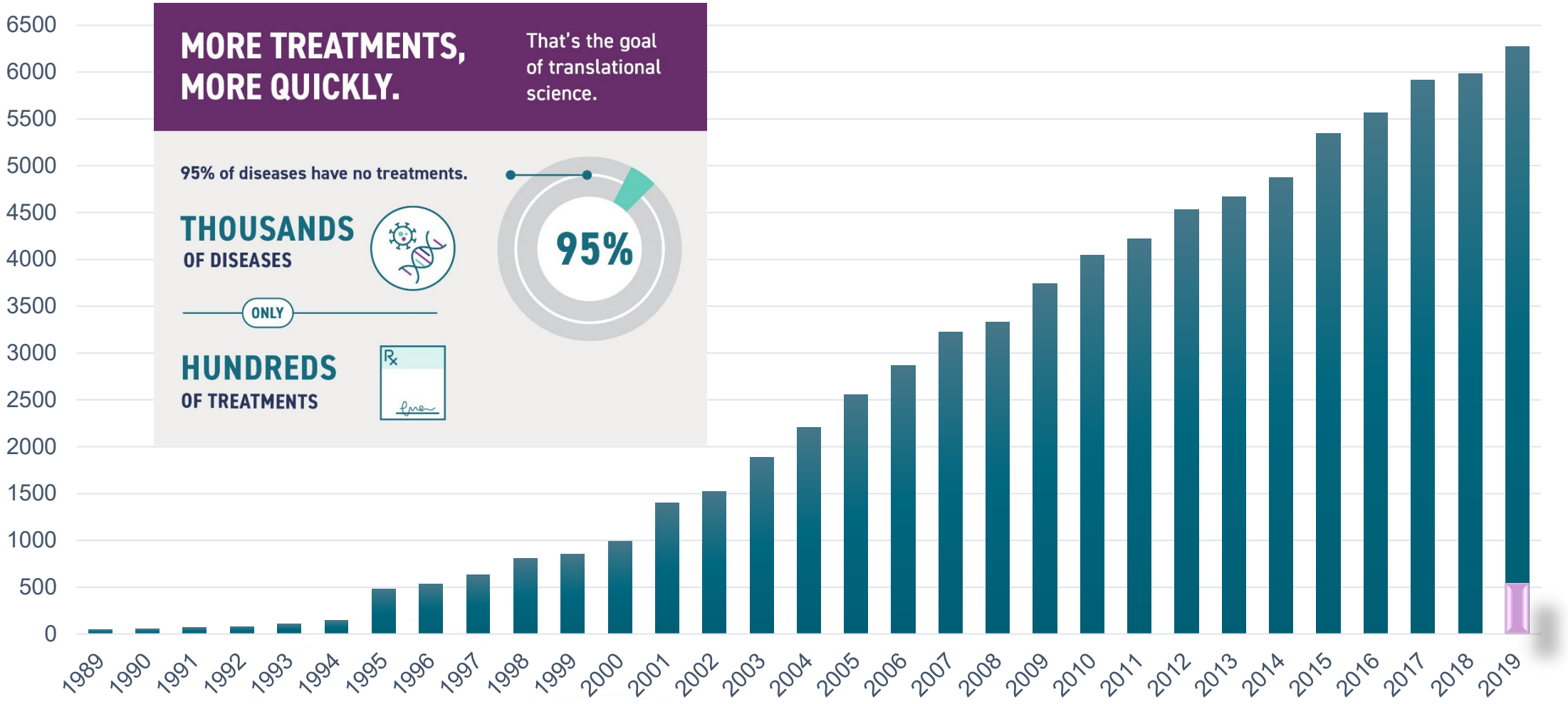
# Diseases with Treatments




Source: Online *Mendelian Inheritance in Man*, Morbid Anatomy of the Human Genome

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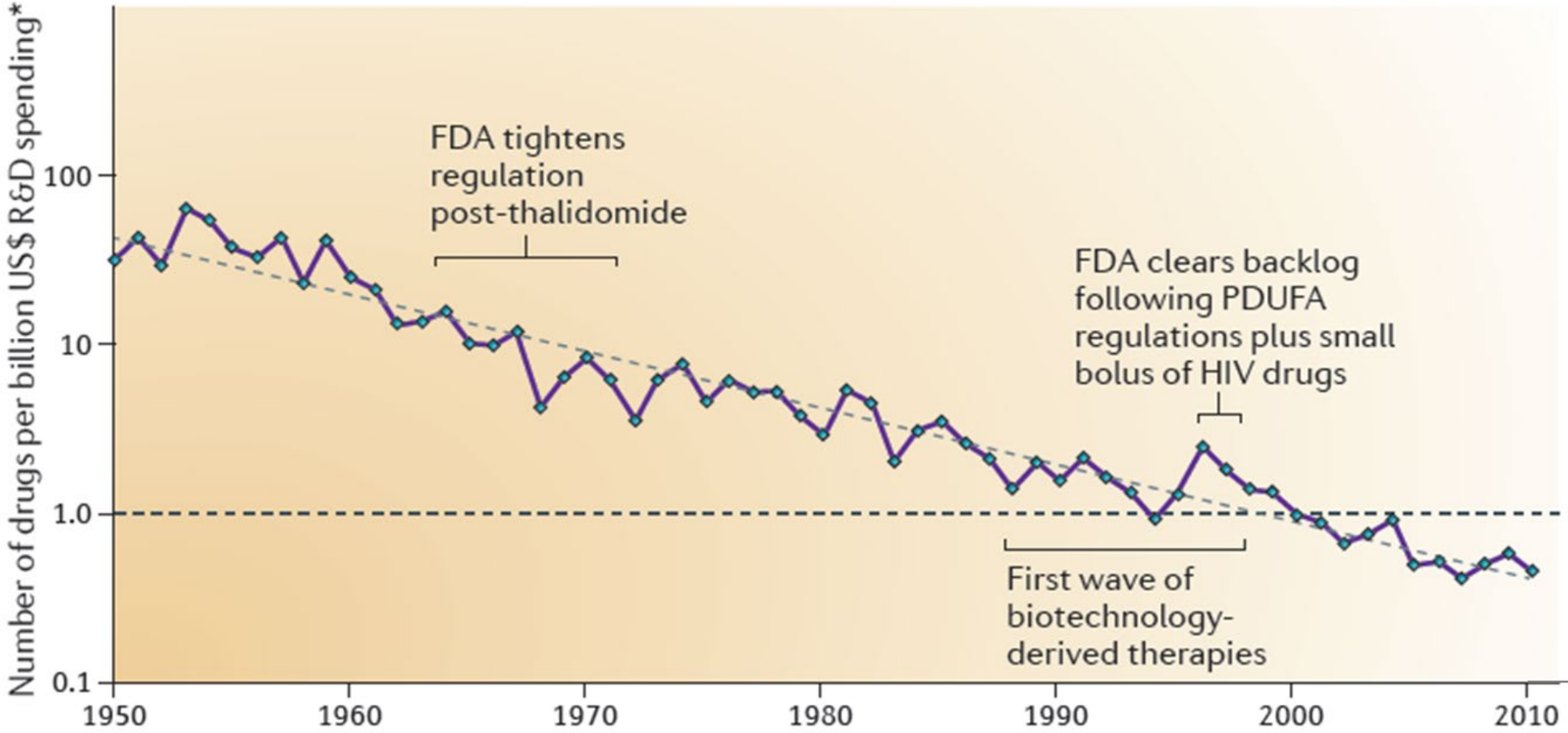
# The Drug Development Problem

- **The number of new drugs approved by the FDA per billion US dollars (inflation-adjusted) spent on research and development (R&D) has halved roughly every 9 years since 1950.**




"The promise of mRNA vaccines: a biotech and industrial perspective" Jackson, nAC et al., npj Vaccines 5 (2020).

Scannell et al., *Nature Reviews Drug Discovery* 11:191, 2012

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NCATS MISSION

# Turn basic science observations into health solutions through translational science

**MORE TREATMENTS, MORE QUICKLY.**

That's the goal of translational science.

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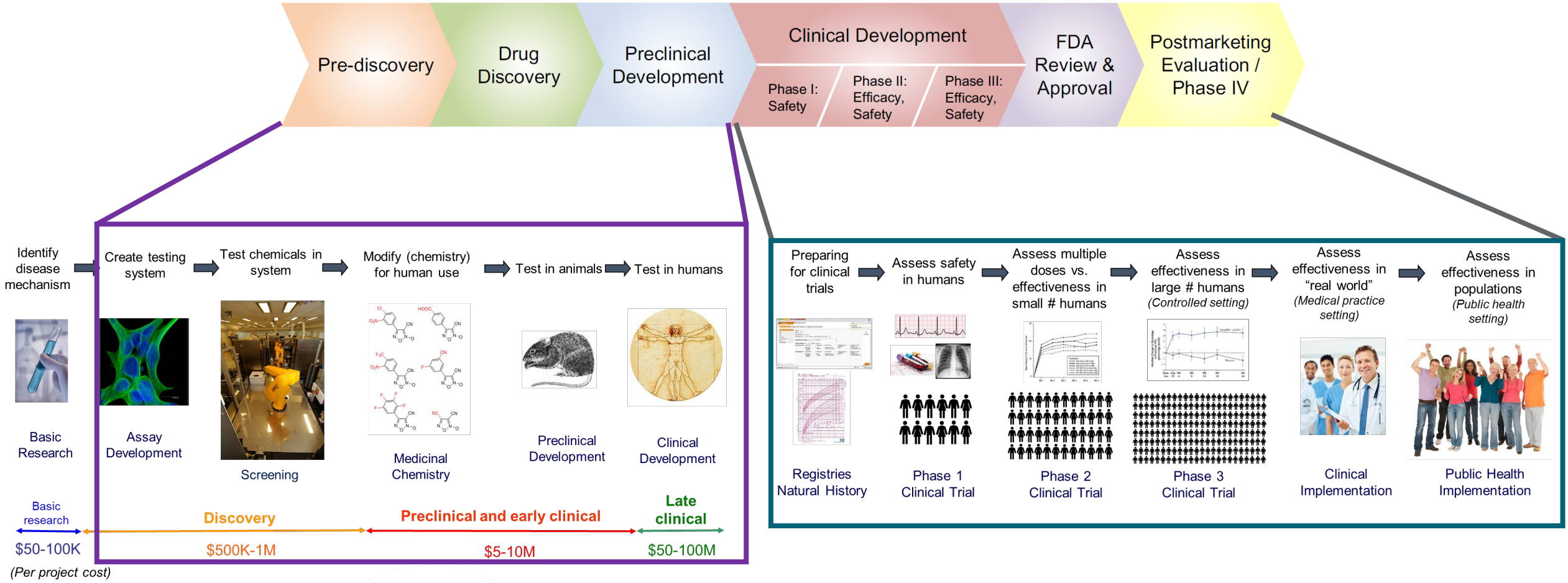
# The "Translational Pipeline" Illustration Part 1: Bottlenecks

1. Translational Research (**Evolutionary**)



2. Translational Science (**Revolutionary**)

# The “Translational Pipeline” Illustration Part 2: Pre-clinical & Clinical



# The “Translational Pipeline” Illustration Part 3: It’s complex!

## *The Drug Discovery, Development and Deployment Map (4DM)*


Technologies

Platforms

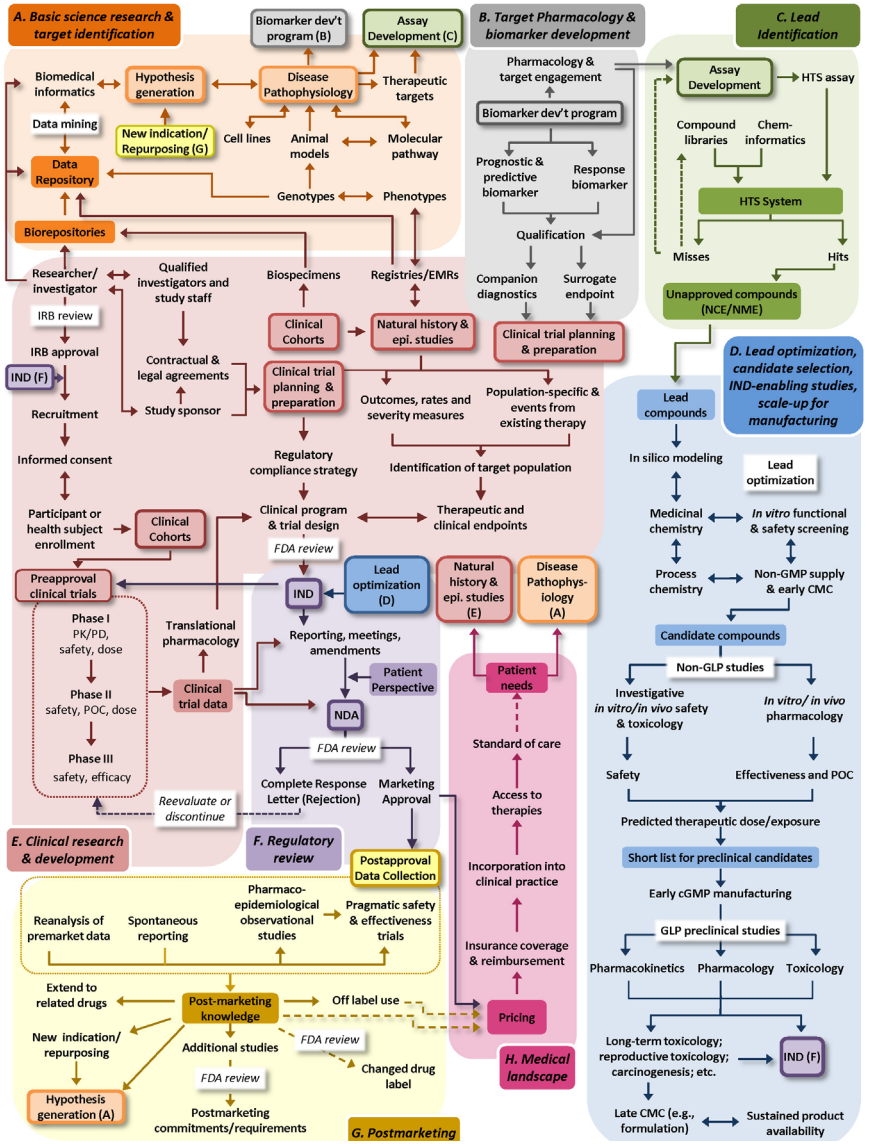
Tools

Resources

Training & Education

Collaborations & Team Science

Public – Private Partnerships




*Nature Reviews Drug Discovery* 15:150, 2018

https://ncats.nih.gov/translation/maps

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# Translational Science

The **field of investigation** focused on understanding the **scientific and operational principles** underlying each step of the translational process.

Requires:

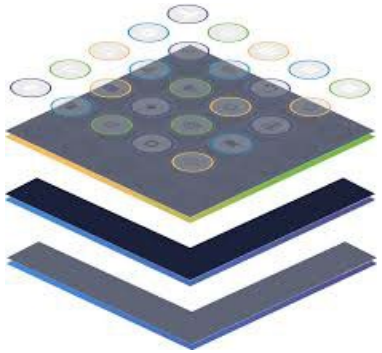
- Understanding common challenges or roadblocks to translation
- Determining the scientific and operational principles that can be utilized to remove the roadblocks
- Developing solutions that employ these principles and will be applicable to many research areas, diseases, and conditions.

# Our Approach: More than 1 Disease at a Time

- **NCATS uses two main tactics**

## *Collaborative Development of Treatments*

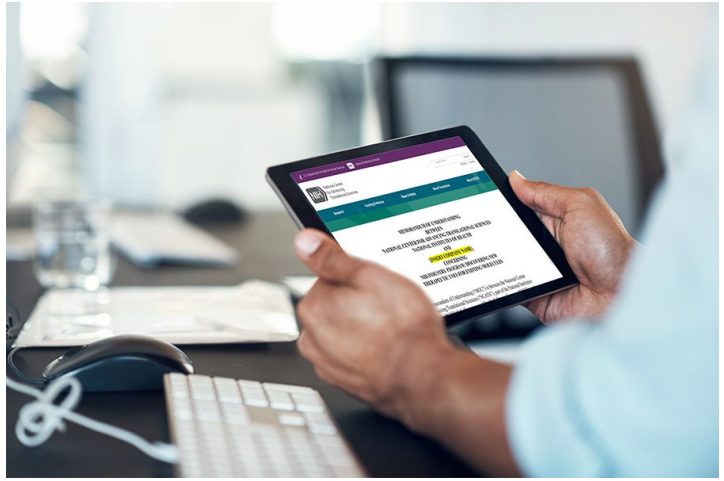

**"Platform":** a scientific/operational infrastructure to support testing of diseases simultaneously



**Goals:** Decrease time to development
Lower costs
More therapies for more diseases faster

# Templates for Success: Speeding the Formation of Public-Private Partnerships

NCATS is speeding the formation of innovative private-public partnerships through the development, demonstration and dissemination of template agreements, to get things done faster.



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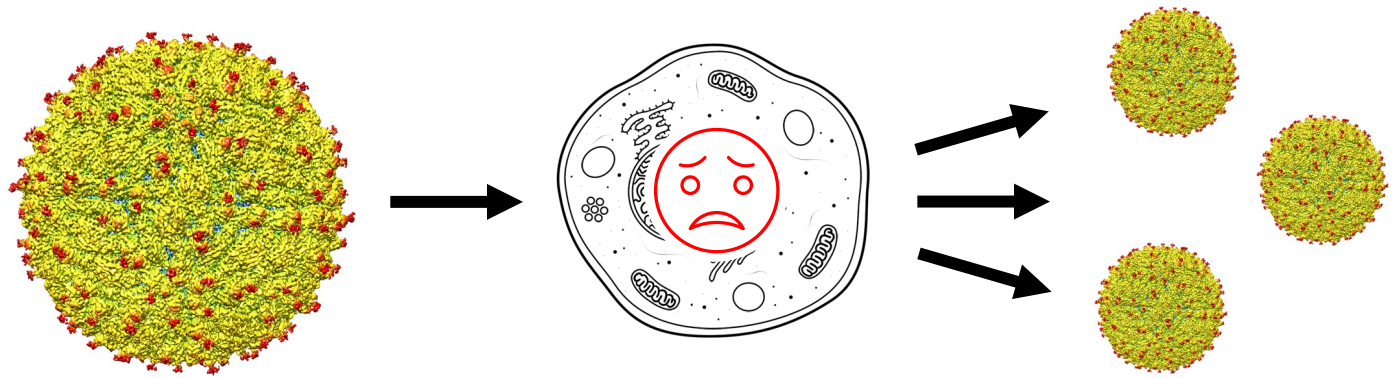
# The Promise of Translational Science for Rare Diseases

- **>7000 rare diseases** (new rare diseases identified each year)
  - ~80% Mendelian genetic
  - ~50% onset in childhood
- **Population prevalence -** ~8-10% (US ~30M; EU ~30M, World ~350M)
- **Definition of rare disease varies by country**
  - Absolute prevalence: US <200,000; Japan <50,000; S. Korea <20,000
  - Percentage prevalence: EU <5 in 10,000; Australia <1 in 20,000
- **Diagnosic odyssey** typically requires multiple specialists and 5-15 years
- **Less than 5%** of rare diseases have an approved treatment
  - At current pace, it will take THOUSANDS of years to have treatments for all

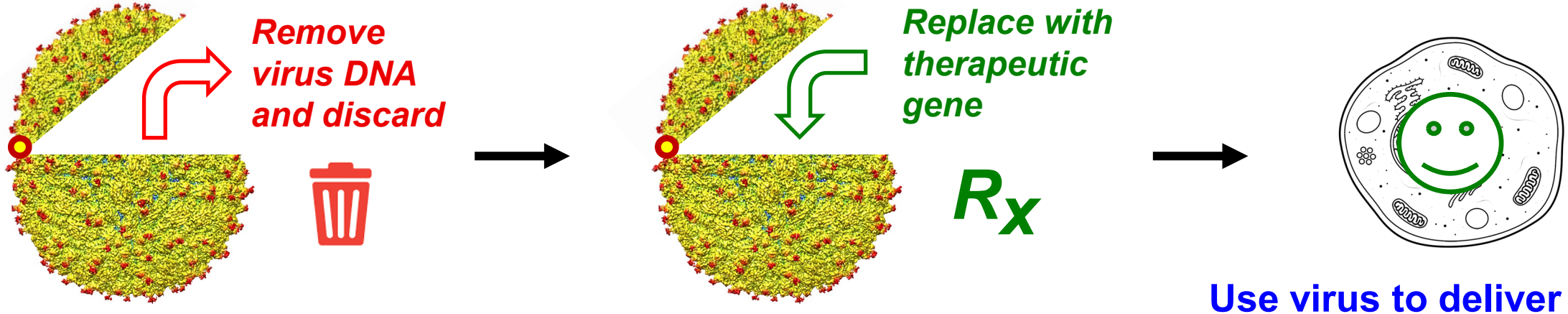
Normally, a adeno-associated virus injects its DNA (= genes) into a human cell to make more virus:



AAV

*human cell*

In **gene therapy**, we replace the viral DNA with a human therapeutic gene:




AAV

**Use virus to deliver therapeutic gene to the patient's cells!**

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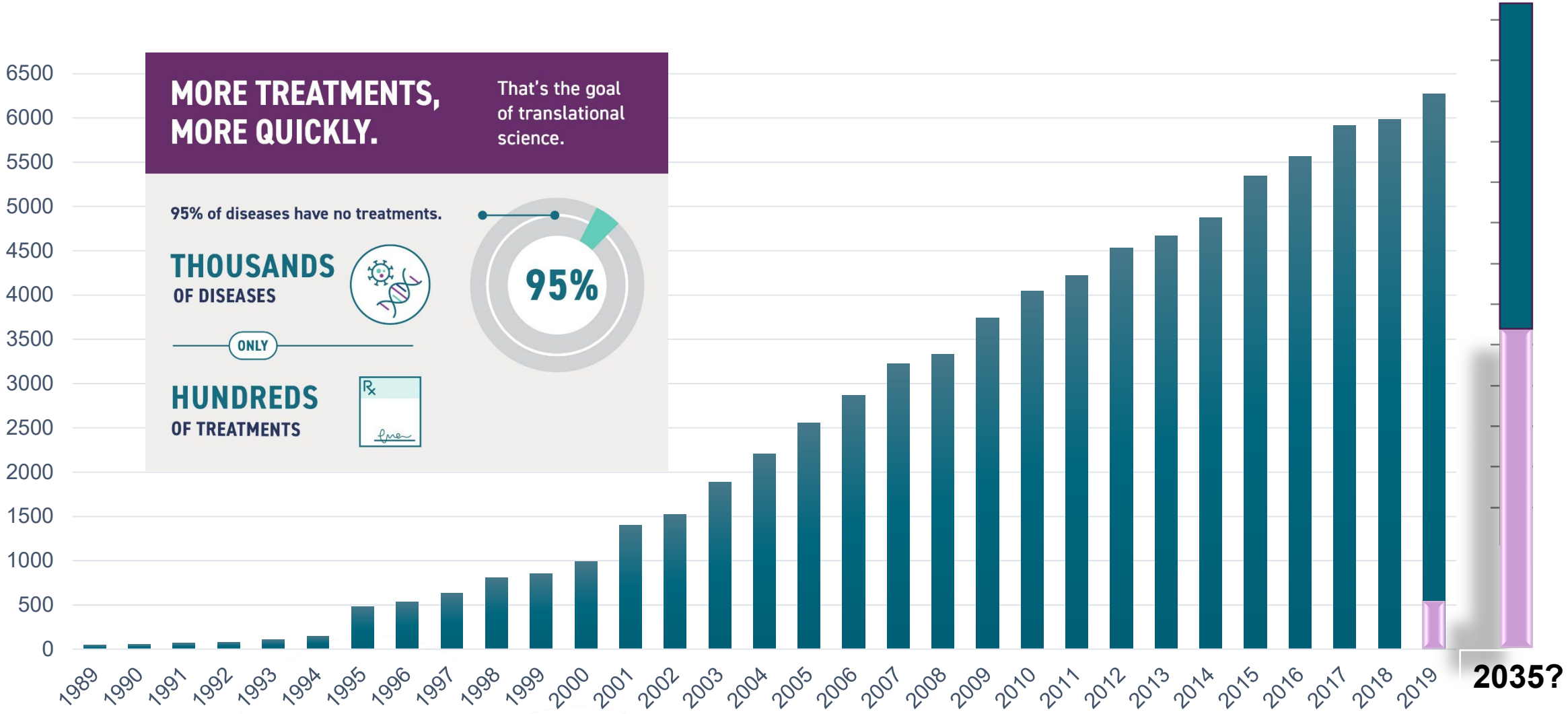
# Diseases with Treatments




Source: Online *Mendelian Inheritance in Man*, Morbid Anatomy of the Human Genome

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# Conclusions

- Translational science is team science.  It is an evidence-based field that seeks to develop and speed solutions for many diseases at a time through identifying and overcoming persistent challenges
- The solution-focus of translational science highlights the centrality of identifying scientific and operational principles underpinning successful translation efforts
- Formalizing our translational science knowledge is essential for distinguishing this area of research
- Conveying this knowledge to others will lead to more successful and impactful translation

# Your Charge – Possibility thinking – innovating on training the next generation of translational scientists

- How would teaching the principles of translational science augment core competencies that have been identified
- How do we turn core competencies and teaching the principles of translational science into consistent components for training the next generation of translational scientists
- How do we share knowledge that addresses the vast pre-clinical and clinical translational science pipeline
- How do we innovate on training and coordinate training better
- How do we reach the full breadth of the translational science workforce with our education activities

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