

# Innovative Payment Models for Transformative Therapies



Biotechnology  
Innovation  
Organization

The **HUMAN GENOME** has approximately **20,000** genes



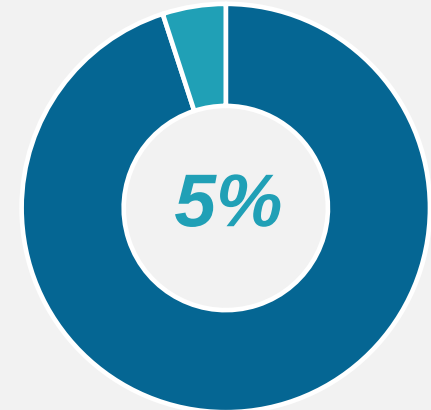
More than **6,000** diseases are caused by genetic mutations



Many are **RARE** diseases affecting 30 million Americans (*1 of 10*)



ONLY 5 percent of these diseases have **APPROVED** therapies



**CBS NEWS**

NEWS

SHOWS

VIDEO

CBSN

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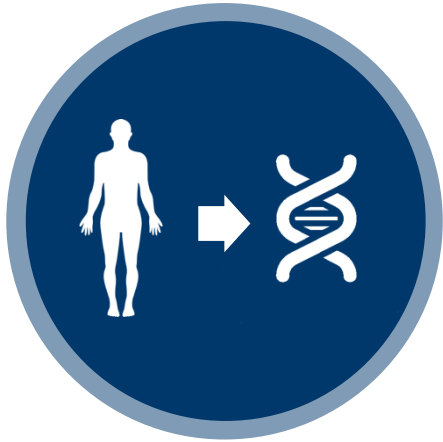
**FDA approves gene therapy for rare form of blindness**

**FORTUNE**

**The Way We Treat Cancer Will Be Revolutionized as Gene Therapy Comes to the U.S.**

# Broken Genes Lead to Broken Proteins

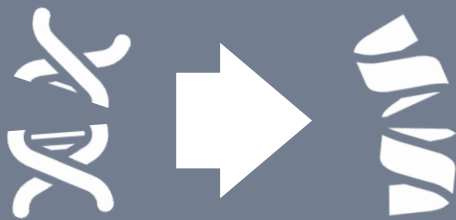
Humans are made of cells that contain our **genes**



**Genes** are instructions for **proteins**



**Proteins** help **cells** function in a healthy manner

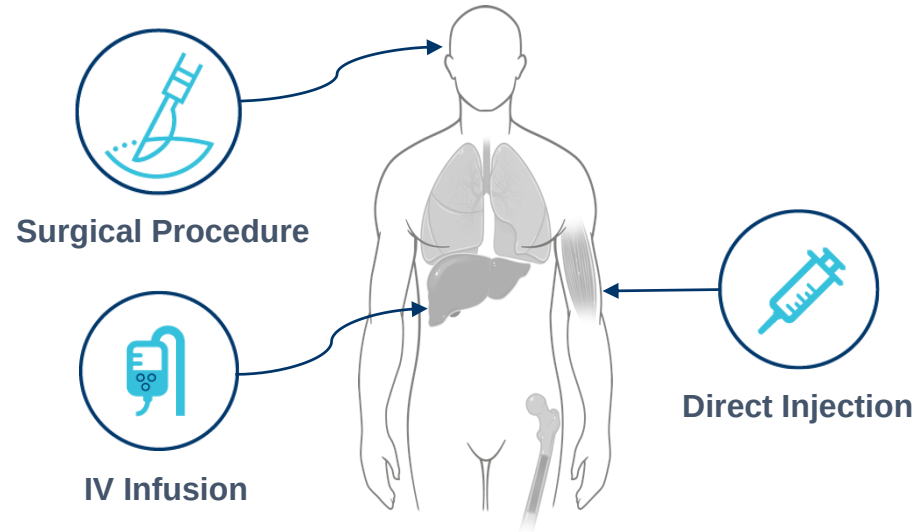


Abnormalities in genes can result in proteins that do not function properly

# Gene Therapy: Treating Patients

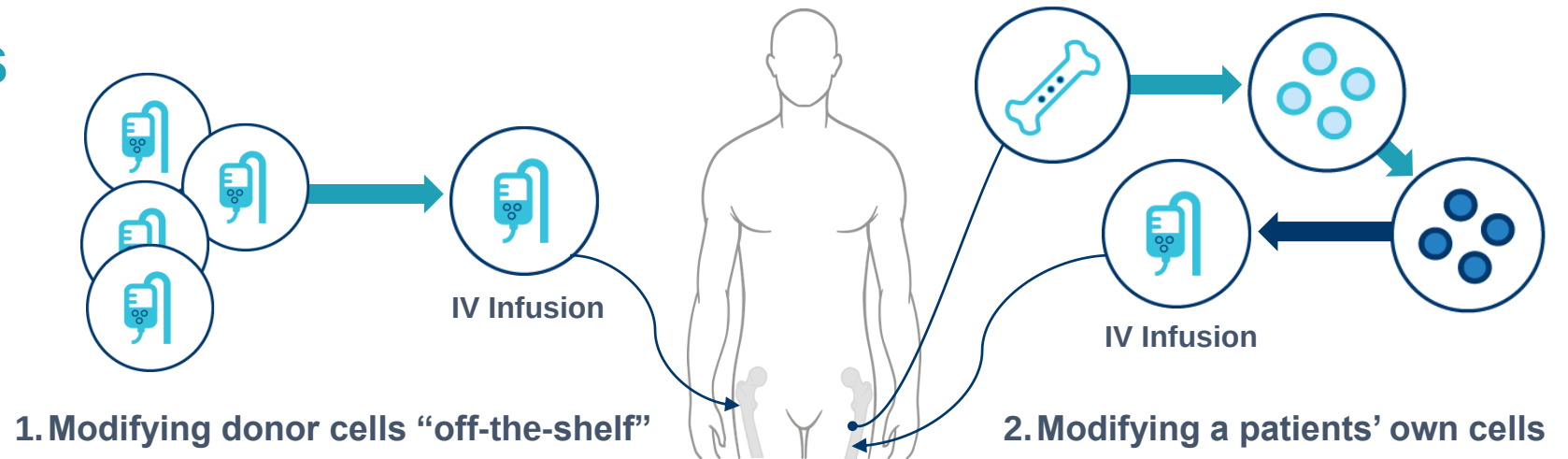
## *In Vivo* Approaches

- Intervene inside the body
- Genome modifications within target cells after individuals receive a genomic medicine
- e.g., surgery, injection



## *Ex Vivo* Approaches

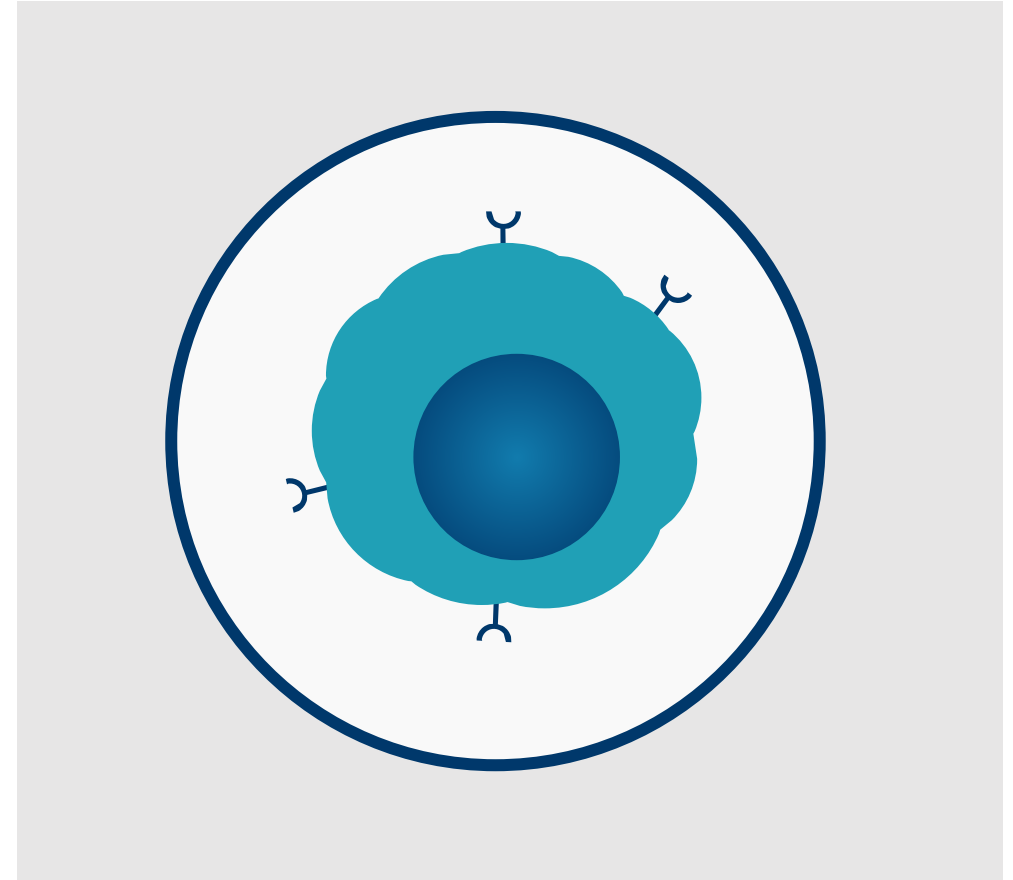
Outside the body,  
create cell medicines by:



# Cell Therapy

- Cell therapy refers to the use of whole cells to treat disease
- Includes **replacing** or **repairing** tissue and /or cells damaged by disease, or **attacking** cancer cells
- Hematopoietic stem cell transplantation (bone marrow transplant) is the most frequently used cell therapy and is used to treat a variety of blood cancers and hematologic conditions.
- Potential applications\*:
  - Treating cancers
  - Treat autoimmune disease
  - Rebuilding damaged cartilage in joints
  - Repairing spinal cord injuries
  - Improving weakened immune systems
  - Treating neurological disorders

\* **Facts About Cellular Therapy, AABB at [www.aabb.org](http://www.aabb.org)**



# Clinical Trials in Genomics Medicine

|                           |                         |                 |                                 |
|---------------------------|-------------------------|-----------------|---------------------------------|
| Oncology                  | Infectious Disease      | Musculoskeletal | Gastroenterology                |
| Central Nervous System    | Genitourinary Disorders | Respiratory     | Endocrine / Metabolic Disorders |
| Ophthalmology             | Geriatric Diseases      | Hematology      | Lymphatic Diseases              |
| Immunology / Inflammation | Ear Disorders           | Dermatology     | Surgery                         |

# New Therapies Require Novel Approaches to Patients Access



**SCIENCE** and the  
**REGULATORY** ✓  
**APPROVAL POLICY**  
for Genomics Medicine...



Have  
**ACCELERATED...**



**REIMBURSEMENT**  
**POLICY**  
**LAGS BEHIND!**

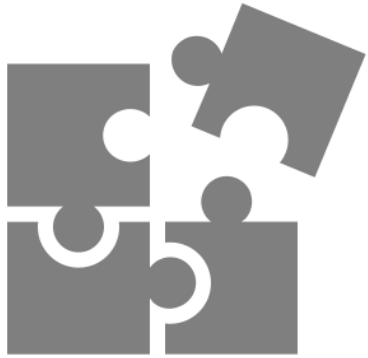


Short—even single dose—treatment regimens yield lasting health benefits, but large single payments will challenge the current reimbursement system



# New Therapies Require Novel Approaches to Patients Access

## Challenges:



- Different from traditional drugs
  - ✓ Usually very small patient population
  - ✓ Often single episode of care instead of chronic therapy
  - ✓ Expensive to administer
  - ✓ Potentially curative
- Further exacerbated by structural challenges
  - ✓ FFS system
  - ✓ Medicaid budgeting
  - ✓ Limited long-term clinical data on transformative therapies



# Innovative Payment Models

## Outcomes-Based Arrangements



Payor reimburses manufacturers for treatments based on how successful the patient responds to therapy with pre-determined clinical endpoints

## Payments in Installments



Payor reimburses manufacturers over a predetermined period of time to avoid significant up-front costs

## Outcomes-Based with Installments



Payor reimburses the manufacturer over time and the payments are portioned out based on how successful the patient responds to therapy

## Warranty Model



Manufacturer pays for follow-up treatments if they become necessary because treatment fails to deliver on promise

## Risk Pooling / Reinsurance



Payors and government set aside a portion of a healthcare budget into a dedicated fund that covers both government and non-government payors

## Subscription Model “Netflix Model”

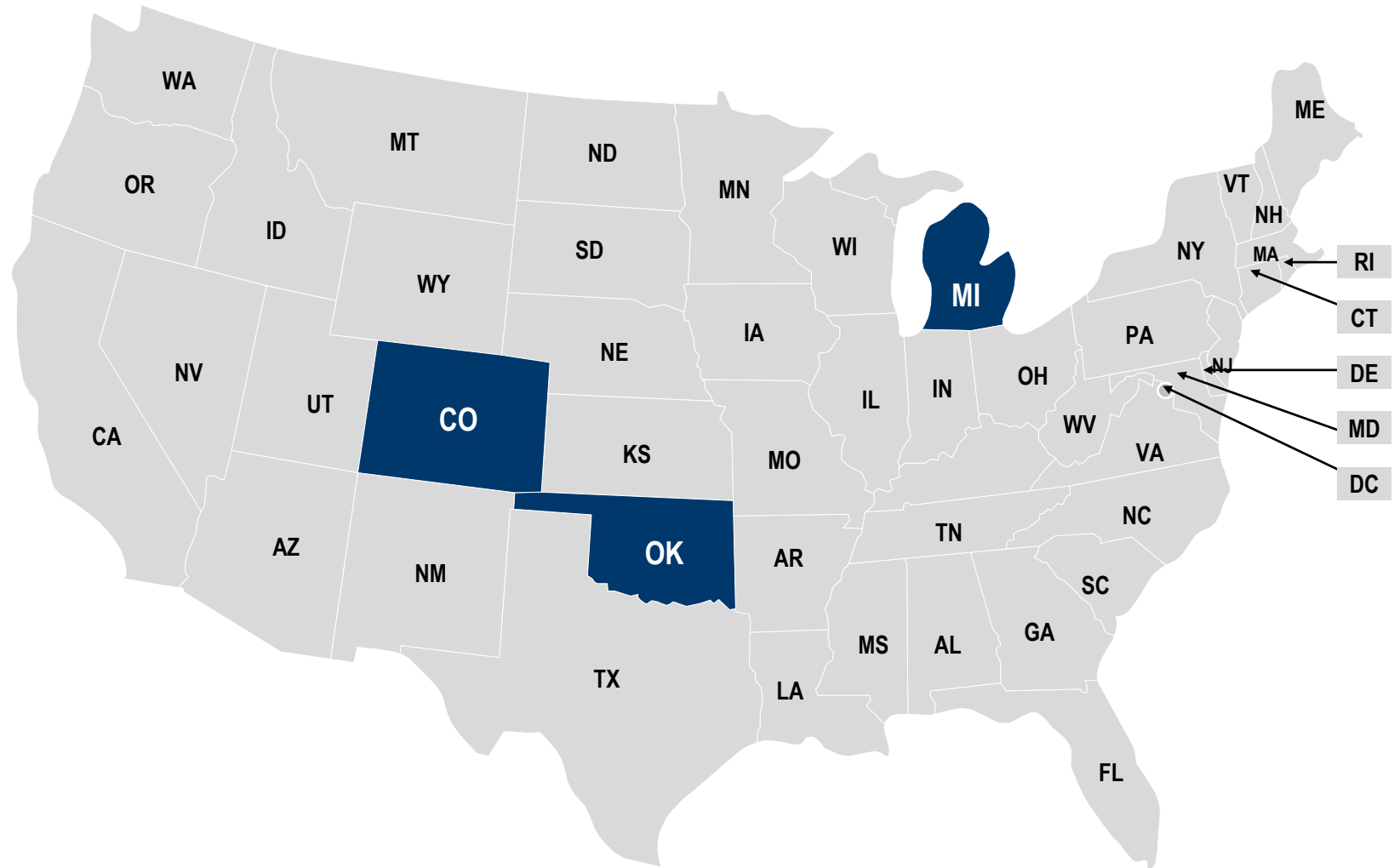


Payor reimburses the manufacturer a set fee for unlimited access to a therapy

# State Approaches

## OUTCOMES-BASED ARRANGEMENTS

Some predetermined clinical endpoints include reduced hospitalization costs and greater patient adherence

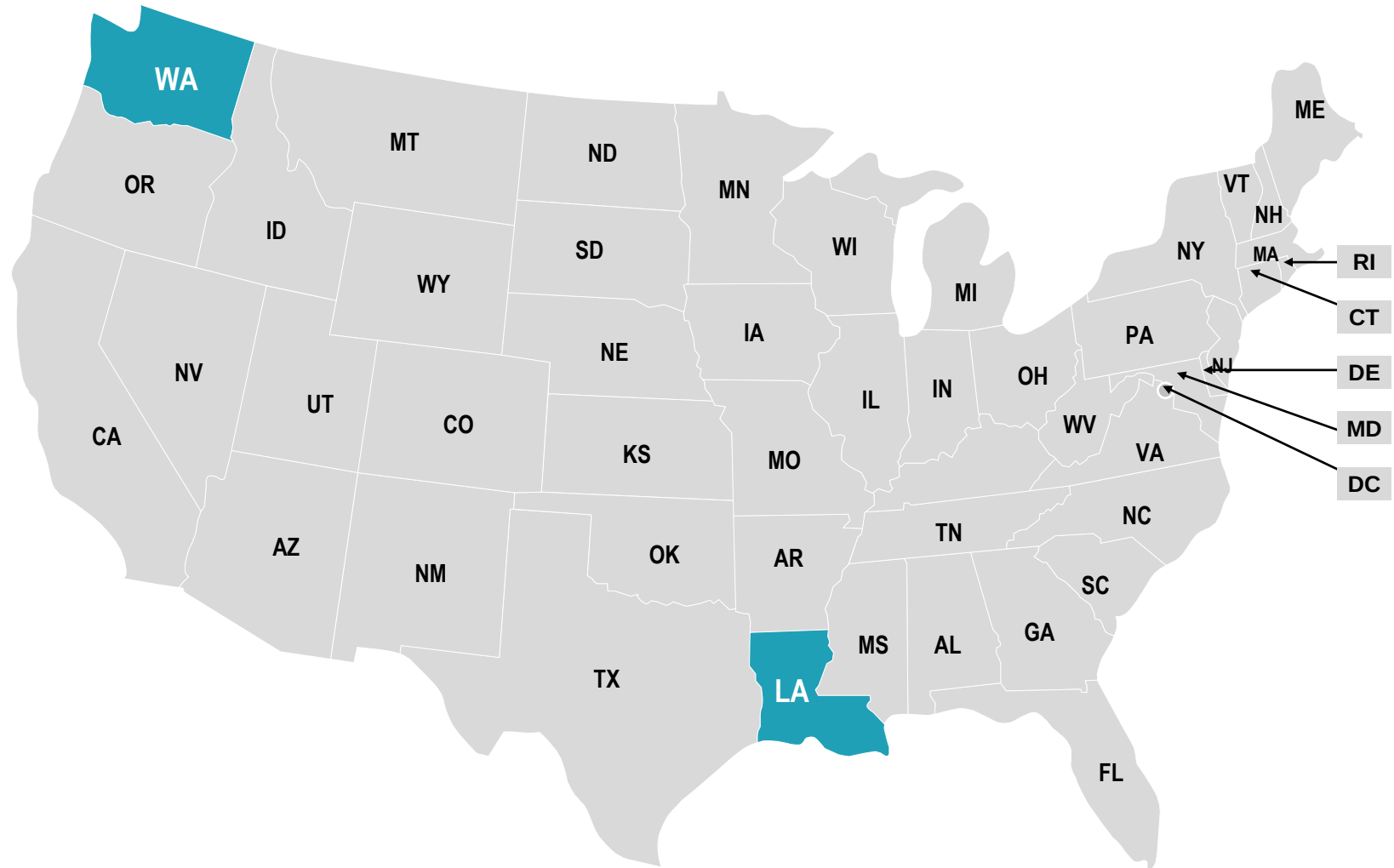


# State Approaches

# SUBSCRIPTION MODEL

State pays an established fee per month for unlimited amount of a drug

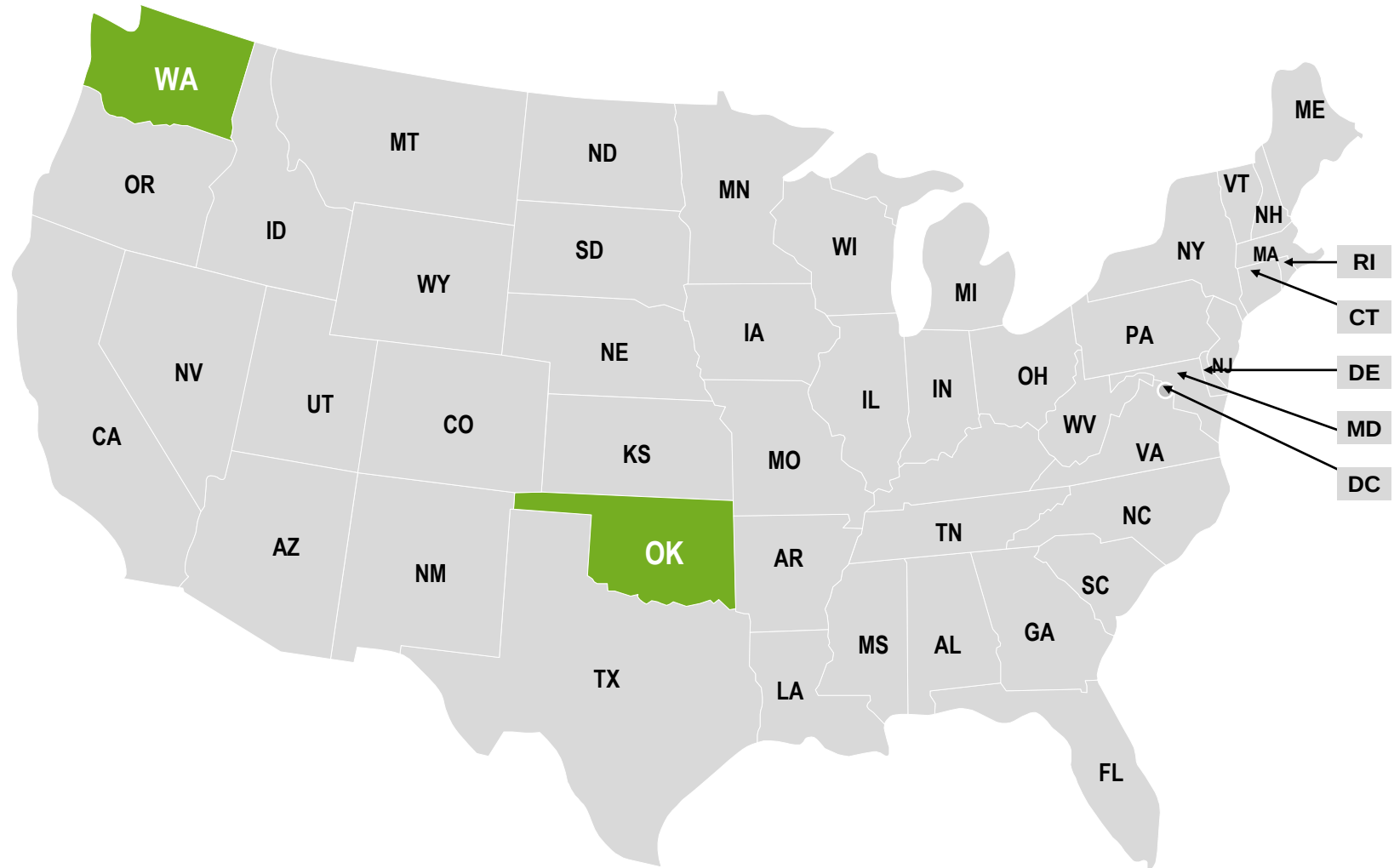
(ideal for curative therapies)



# State Approaches

## HYBRID APPROACHES

States also exploring hybrid approaches such as outcomes-based models and payments over time



# Opportunities for New Mexico

- Implement innovative payment models in Medicaid and other state-run health coverage.
- Ensure no state anti-kickback laws prevent biopharmaceutical manufacturers and health plans from entering into innovative payment models.

# BIO Member Companies Support Innovative & Alternative Payment Structures

- Biopharmaceutical companies and payers are already coming together in creative ways to address the challenges of a 21<sup>st</sup> Century innovative health ecosystem.
- BIO strongly supports innovative and voluntary alternative payment structures between biopharmaceutical companies and payers (commercial & public).
- BIO believes value, outcomes, and/or indication-based arrangements, and alternative payment structures all have merit.
- Policymakers and regulators must understand the variety of these arrangements and provide flexibility to ensure new models can be designed as healthcare evolves and new therapies are developed.

# Resources To Help You Better Understand Genomics Medicine

## General Information on Genomics Medicine

- US Food & Drug Administration- <https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/>
- Biotechnology Innovation Organization (BIO)- <https://bio.org/genome-editing-toolkit>
- Alliance for Regenerative Medicine (ARM)- <https://alliancerm.org/>

## Alternative Payment Models

- M.I.T. New Digs- <https://newdigs.mit.edu/papers-publications>
- Duke Margolis Center for Health Policy- <https://healthpolicy.duke.edu/>





## Biotechnology Innovation Organization

- World's largest trade association of biotechnology companies, academic institutions, state biotechnology centers and related organizations
- Based in Washington, D.C. with members across the US and in over 30 countries
- Membership includes large multinational companies, as well as small-to-medium size companies
- Members are involved in three core areas:
  1. Healthcare
  2. Food & Agricultural
  3. Industrial & Environmental Bio-based Products