

Today's path ...



Stories of patients and advanced therapies
The social difficulties in accepting scientific innovations
The paradigm shift of new therapies
What are the ATMPs: Medicinal Products for Advanced Therapies
Therapeutic efficacy and safety (regulatory aspects)
The innovative payment methods
From Patients to Citizen & Institutional Advocacy

INSIGHTS



POLICY FORUM

SOCIAL SCIENCE

The science of fake news

Addressing fake news requires a multidisciplinary effort

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gated about topics such as vaccination, nutrition, and stock values. It is particularly pernicious in that it is parasitic on standard news outlets, simultaneously benefiting from and undermining their credibility.

Some—notably First Draft and Facebook—favor the term “false news” because of the use of fake news as a political weapon. *Q*

sciencemag.org **SCIENCE**

It is necessary to know in order to deliberate correctly

"We have discovered that falsehoods spread faster than the truth"

surprise and

while the true character

<http://sc>



POST-TRUTH, FAKE NEWS AND DISINFORMATION

Oxford Dictionaries 2016

post-truth: «Relating to or denoting circumstances in which objective facts are less influential in shaping public opinion than appeals to emotion and personal belief.»

CRISIS OF CONFIDENCE IN SCIENCE AND EXPERTS

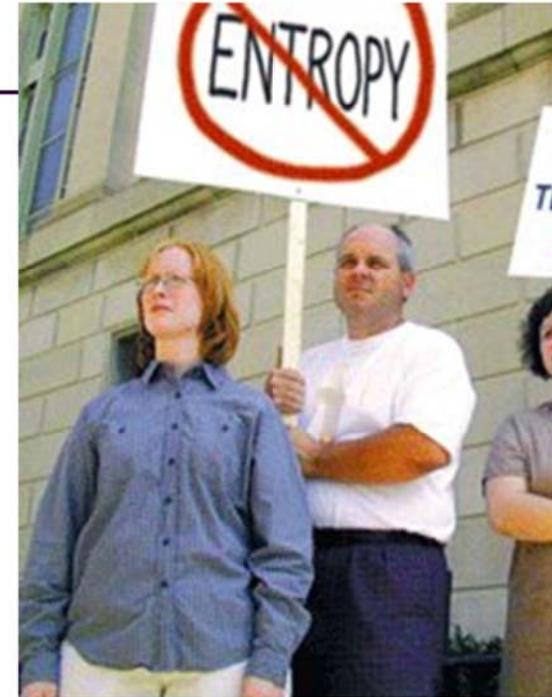
SCIENTISTS VS. CITIZENS

90% of scientists consider **GMOs** to be safe, compared to 35% of citizens

For **vaccines** the ratio is 95% vs 70/50%

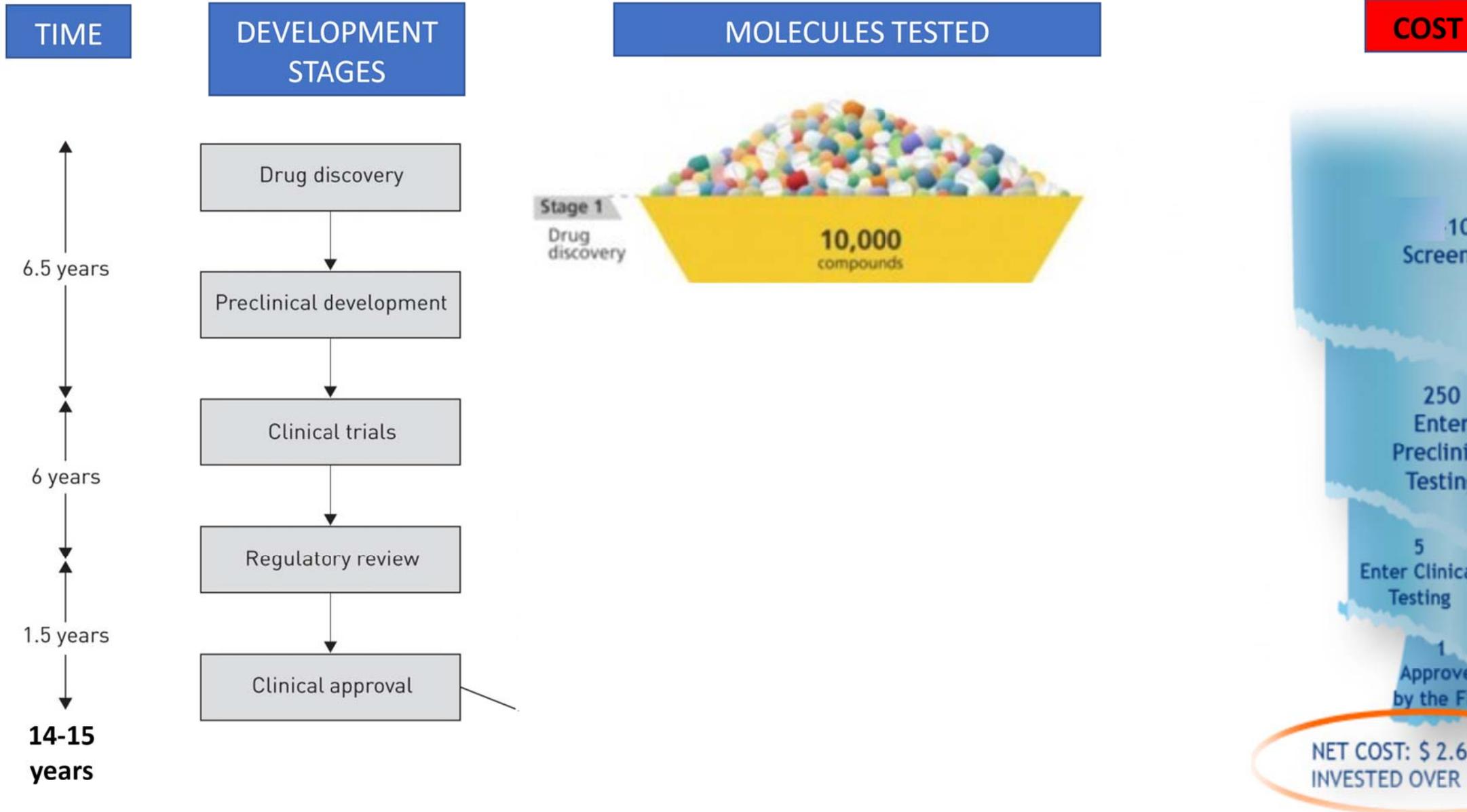
On **climate change** we are at 95% against 65%

On the theory of **evolution** the ratio is 98% vs 65% (USA)

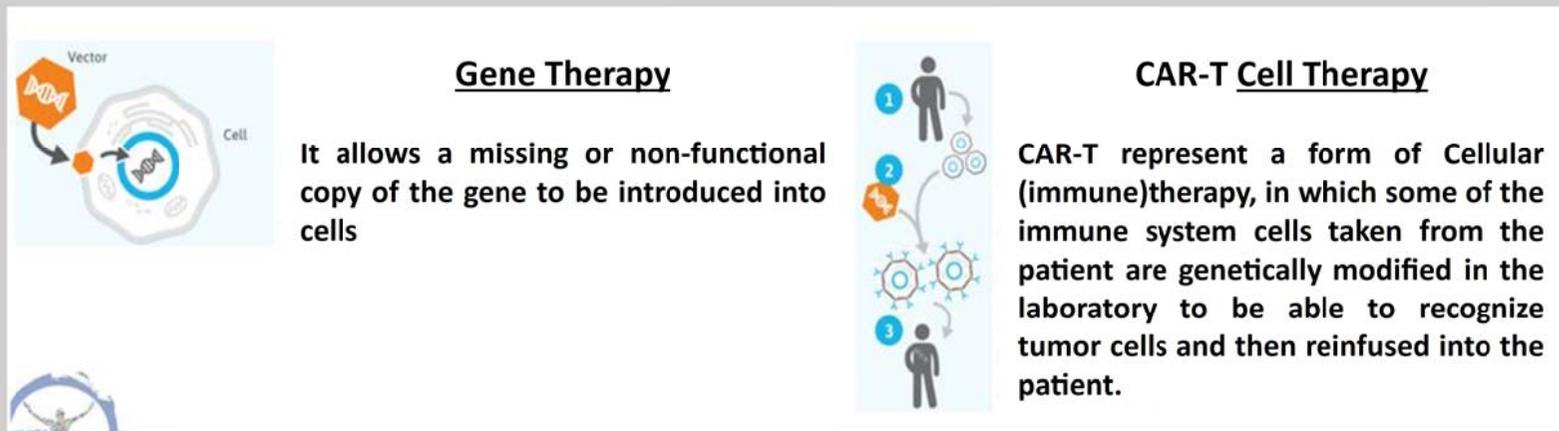
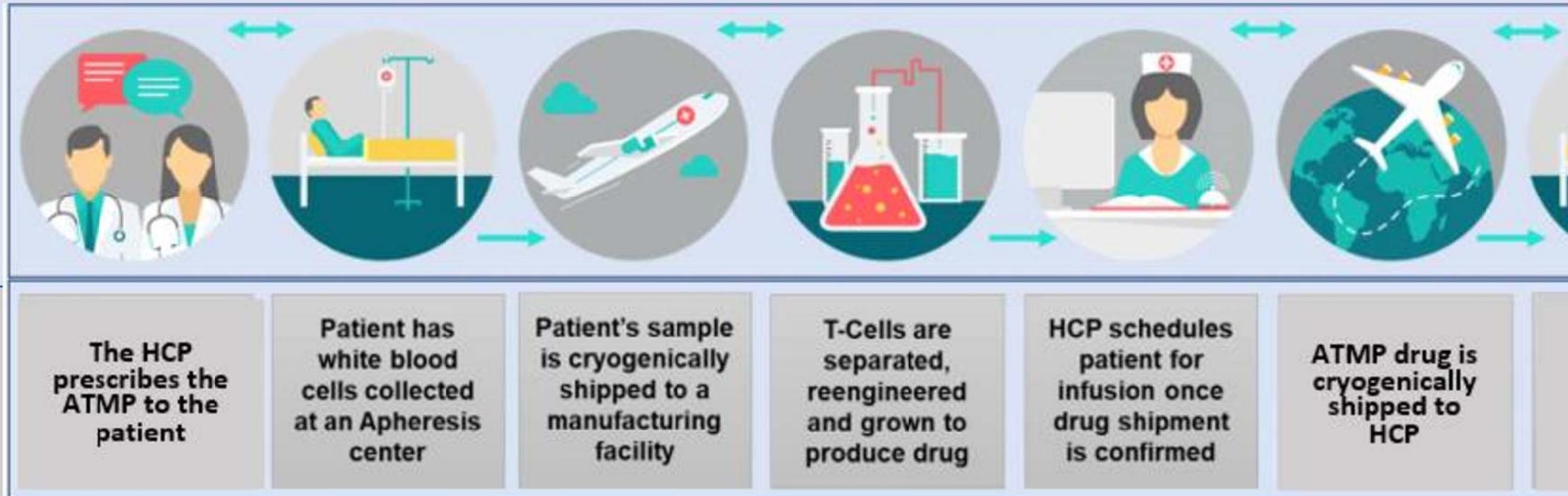


RESEARCH AND DEVELOPMENT IN 'CLASSIC' DRUGS (acute and recurrent pathogens)

A long, complicated and very expensive process



As in the case of **CAR-T** (a type of **ATMPs**), the production process is much **more** and **expensive** than for classic drugs.



If compared with "classic therapies", Advanced Gene and Cellular Therapies are more complicated, because:



They can **restore, correct or modify impaired physiological or pathological functions** in humans, including by correcting **mutations** acquired on a **genetic basis**.



The often target rare (orphan or ultra-orphan) diseases ($5 \leq 10,000$)



Often provide a **single assumption** (one shot), and not extended over time



They have **significant costs** in the current and **benefits that extend** over a long period of time (**temporal mismatch** between **current costs and future benefits**)

Advanced Gene and Cellular Therapies (ATMP) are more complicated, because



They provide for **cell production/culture laboratories** in which the genetic engineering phase takes place (with **complex storage problems**), which are both **limited and grouped** on national territories (intercontinental material shipment)



They provide for clinical procedures **treatment/infusion** (in-vivo or ex-vivo) that involve **few hospital centers of ex-advanced treatment** (facility/non-drug) are often those already **equipped for** (with GMP / JACIE and specialized personnel)



They have a **very complex evaluation process** and a "**traditional reimbursement** and budget schemes" **for cost coverage** are not able to harmonize the value of **ATMP** (impacting patients' lives)



Health mobility between territories equipped for ATMPs and those equipped on the national territories increase: to date, the special GMP/JACIE certified **treatment** are generally **grouped in some**

To guarantee patients this important therapeutic innovation
..... new management models are needed

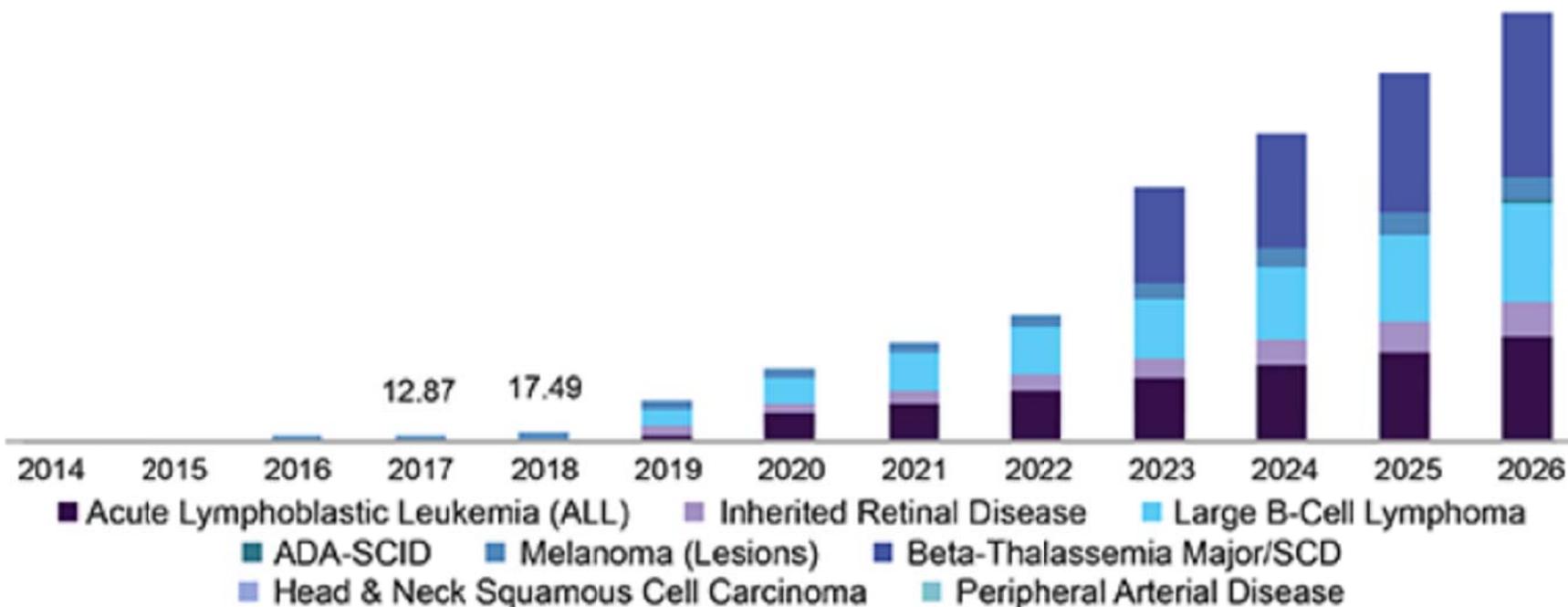
Durable Therapies Distill Payments U



By 2030, up to 60 new ATMPs will be launched globally

They will treat over 350,000 patients

European Gene Therapies Market by Therapeutic Indication, 2014-2025 (Million USD)



Source: www.grandviewresearch.com

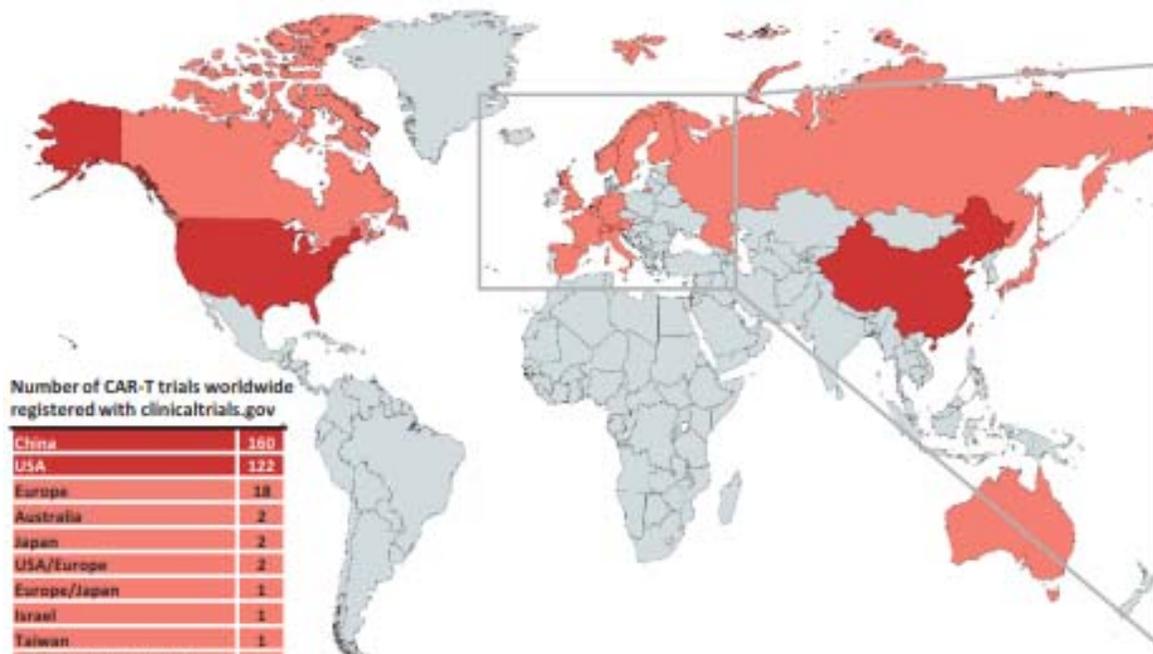
The world market is growing steadily, ranging from 25 year.

In EU, there are **230** therapeutic operating in the ATMP in a 2021 *Regenerative Med* estimated an **invest billion dollars.**

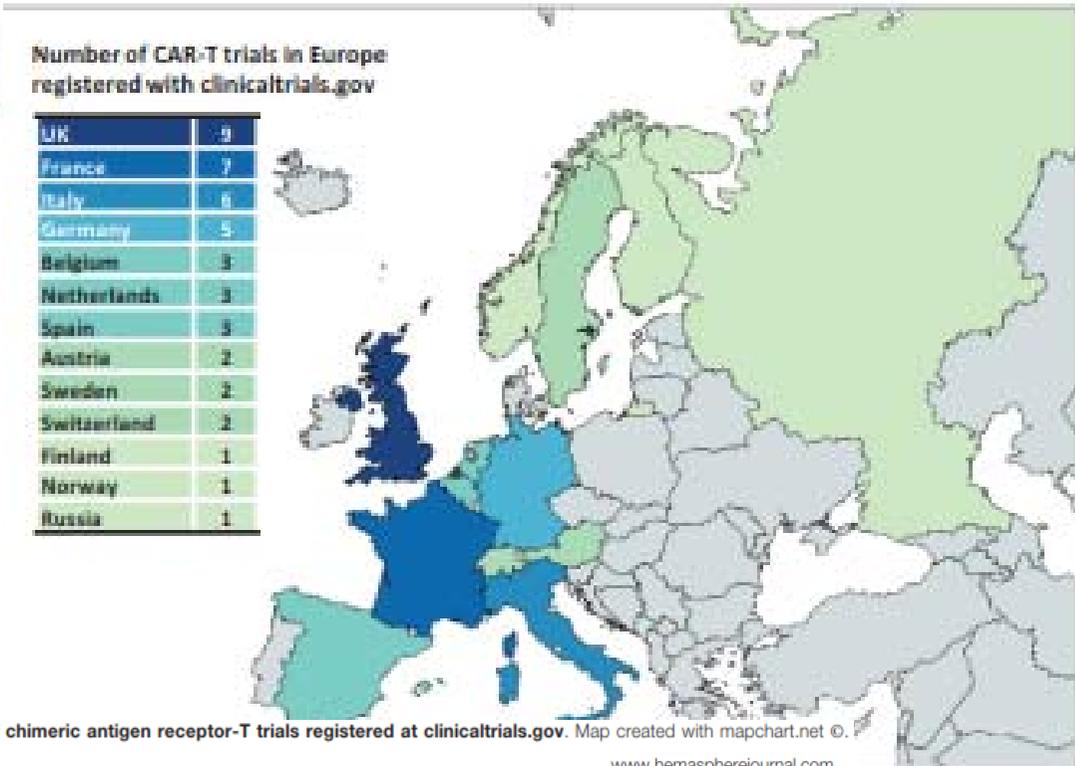
<http://alliancerm.org/wp-content/uploads/2022/03/>

Health is first of citizens' VALUE, an INVESTMENT EXPENDITURE FOR COUNTRY SYSTEMS

TODAY EUROPE IS ONE OF LEADER IN THE

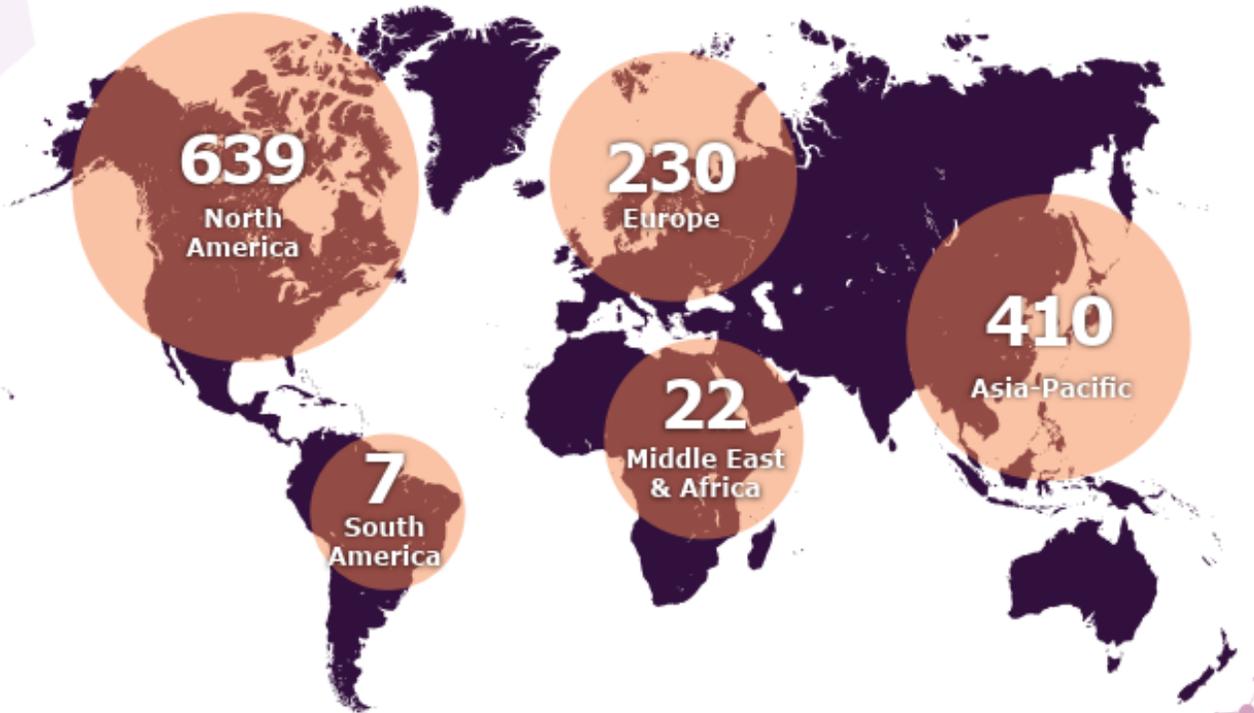


EU dominates the production market. For this reason, several treatment centers are being set up in Europe for ATMP. Countries such as Australia and Japan are also adapting.



Nowadays, there are 14 Advanced Therapeutic Medicines (ATMP) already on the market in Europe for various indications. Significant increase in such therapies is expected in the next decade: by 2030, up to 60 new cell therapies could be launched globally, which could benefit a total of approximately 350,000 patients.

The Global Regenerative Medicine Sector



1,308
 Total gene, cell, and tissue-based
 therapeutic developers worldwide
 Up 19% Year-Over-Year

THE GLOBAL COMPETITION TO DEVELOP NEXT-

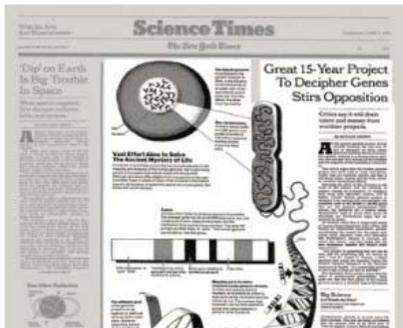


The history of medicine suggests that **innovations and paradigm shifts** often struggle to make their mark

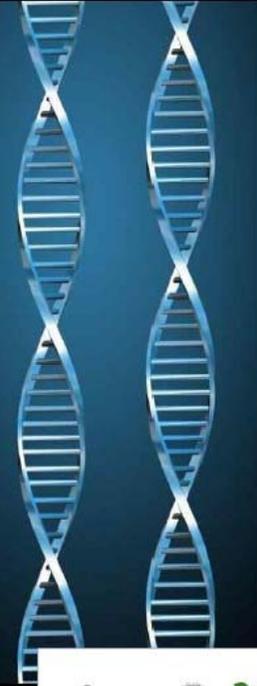
Great 15-Year Project To Decipher Genes Stirs Opposition

By NATALIE ANGIER

June 5, 1990



AS the human genome project drives steadily forward, the vast new effort to delineate all three billion chemical building blocks of humanity's genetic makeup is arousing alarm, derision and outright fury among an increasingly activist segment of the biomedical community.



At the origins of hereditary diseases: Mendel (1865) > Garrod (1923), Pauling & Watson and Crick (1953) > ... > ... > The Human Genome Project (1986)

CHANGE OF PARADIGM of the conc



Renato Dulbecco
tra i promotori del

... is arousing alarm, derision and outright fury among an increasingly activist segment of the biomedical community. The critics argue that the human genome project has been sold on hype and glitter, rather than its scientific merits, and that it will drain talent, money and life from smaller, worthier biomedical efforts. **"Everybody I talk to thinks this is an incredibly bad idea,"** said Dr. M. S. a

