farmaindustria

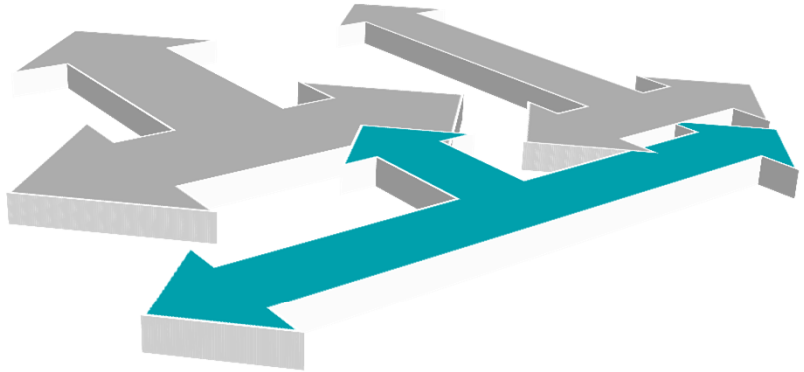
Universidad Carlos III de Madrid

UIMP
Universidad Internacional Menéndez Pelayo

ferrer

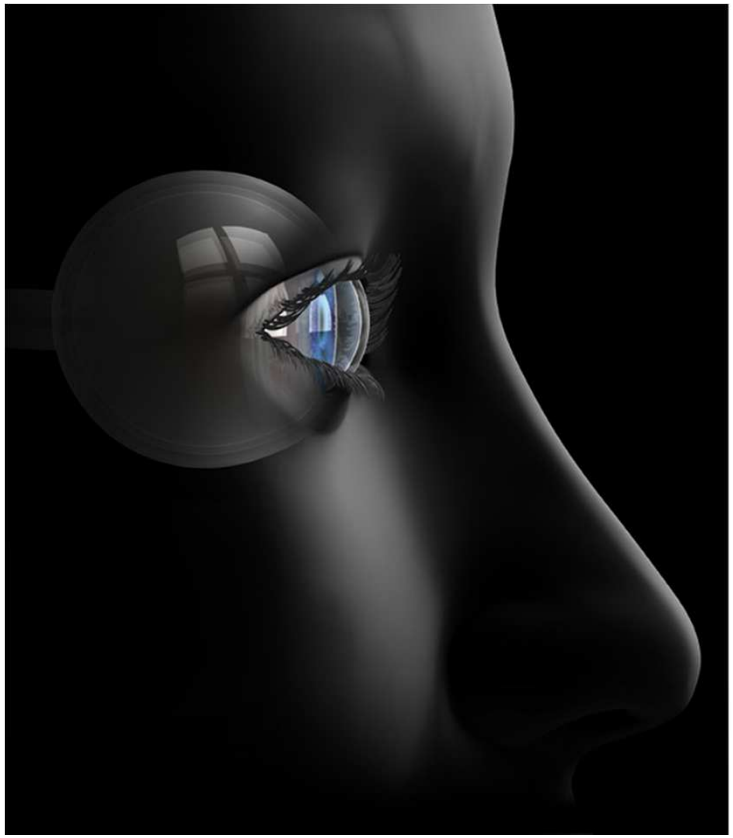
Contribución a la mesa redonda:

Medicamentos biológicos y terapias avanzadas: revolución terapéutica y económica.



Dr. Andrés G. Fernández
Ferrer Advanced Biotherapeutics, Director
Ferrer

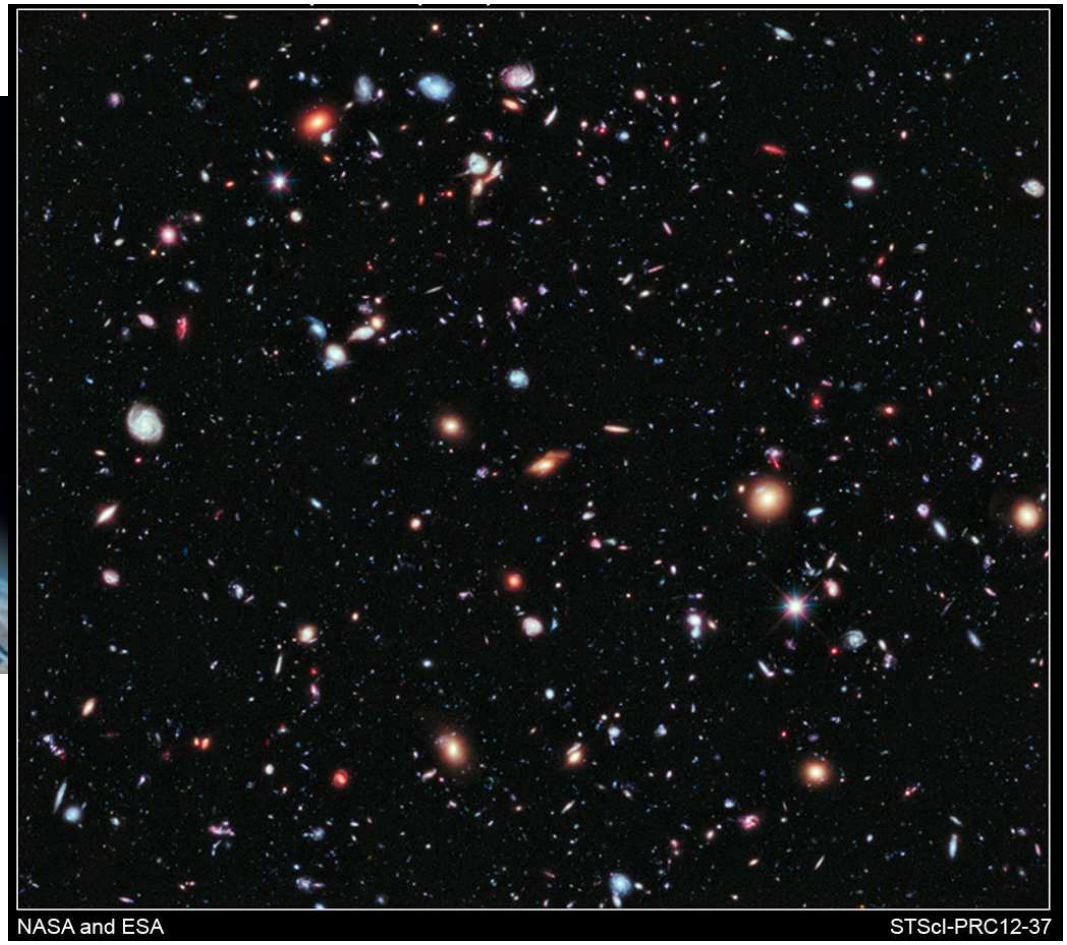
4 y 5 de Septiembre de 2014



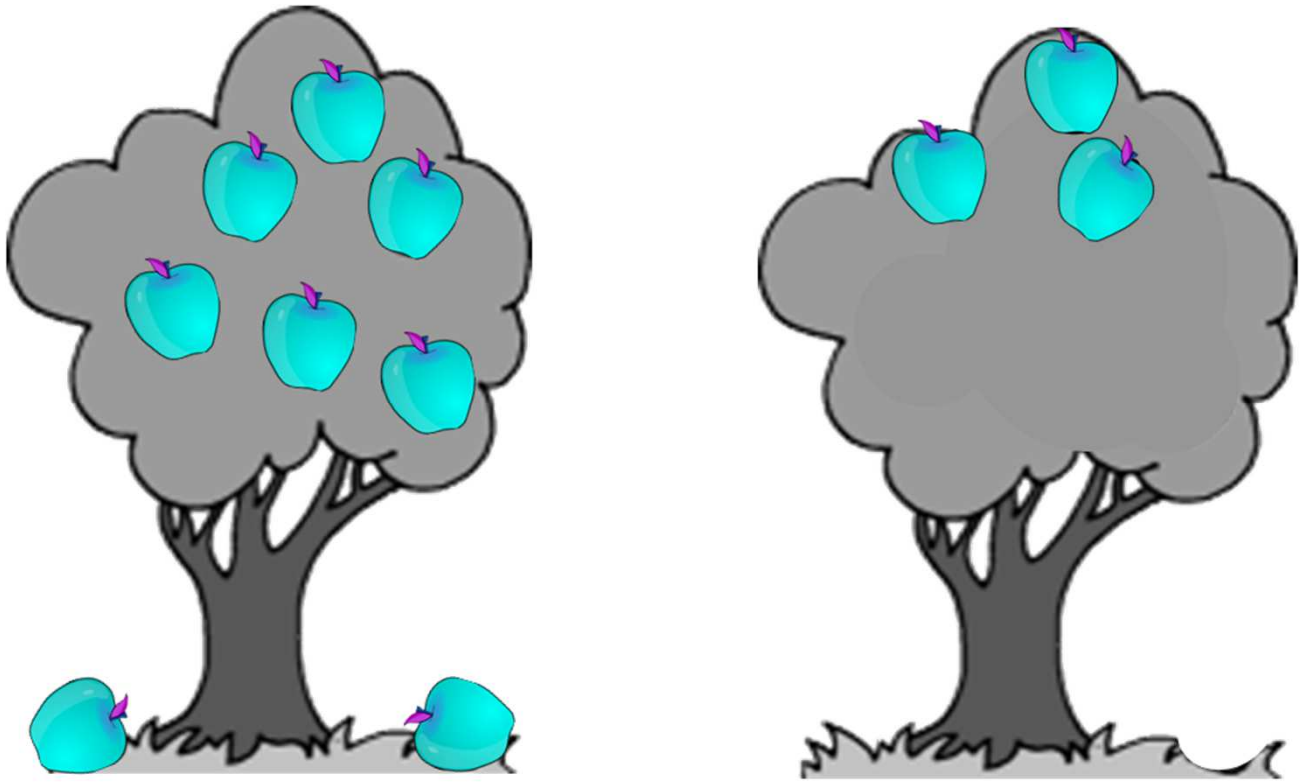
Connecting celestial bodies and drugs…

Discovery & Platforms

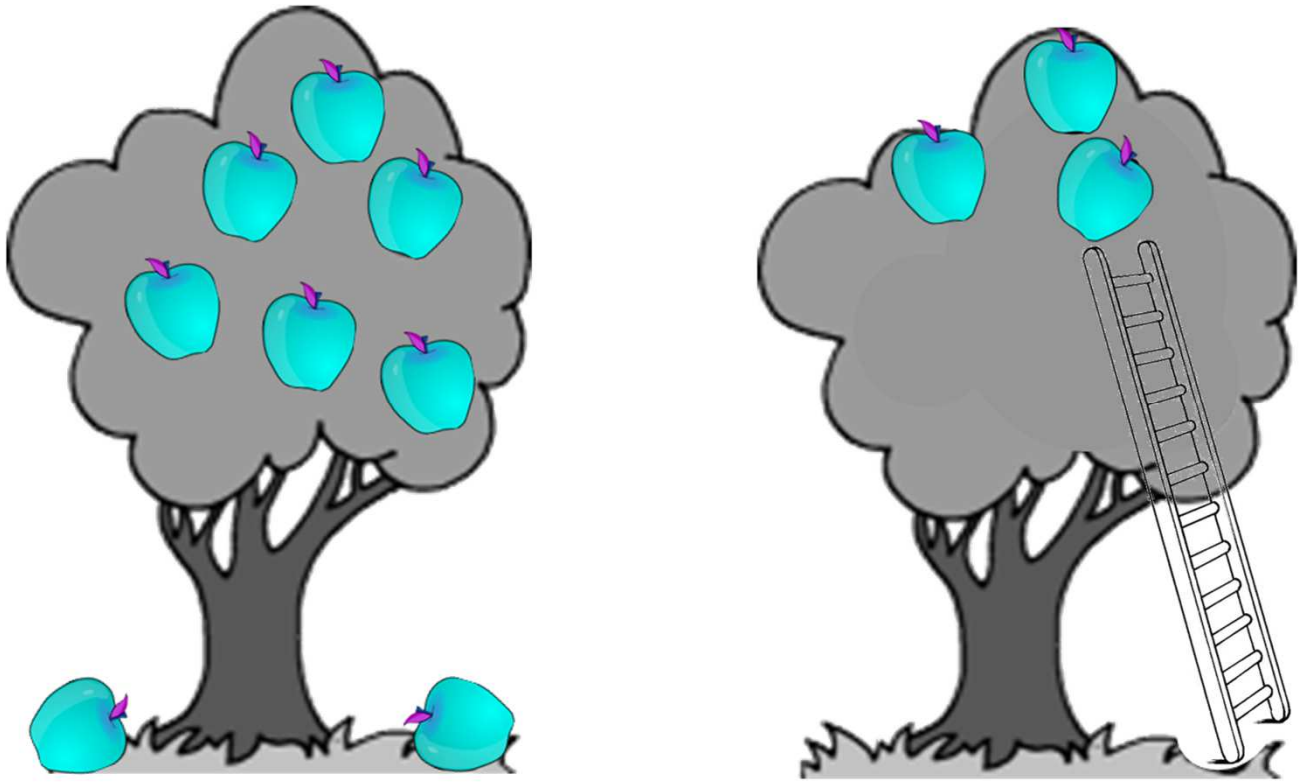
Discovery & Platforms



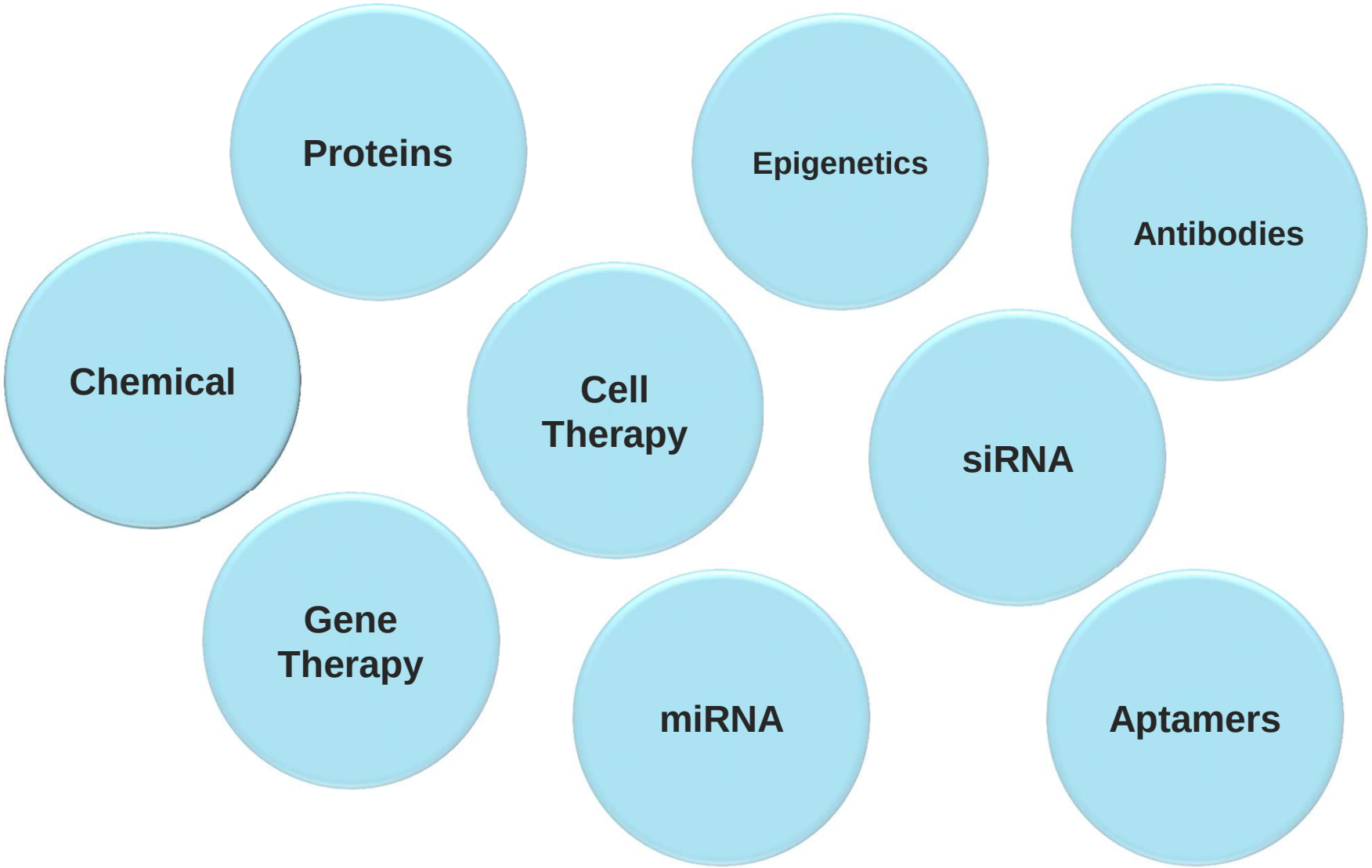
Technological **Platforms**: their pivotal importance



Technological **Platforms**: their pivotal importance



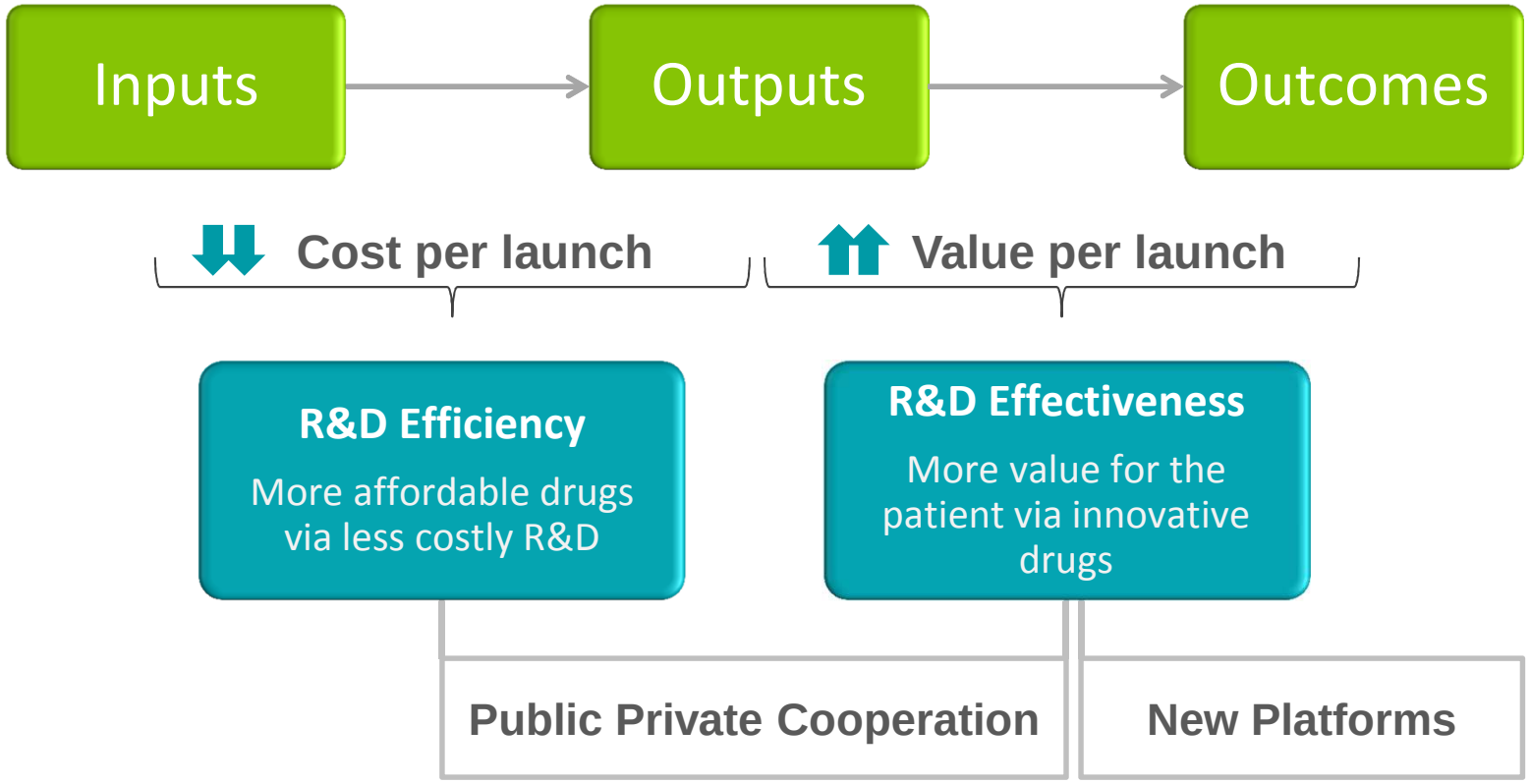
Platform **proliferation...**



Platforms **complementarity**

... but who can afford so many platforms?

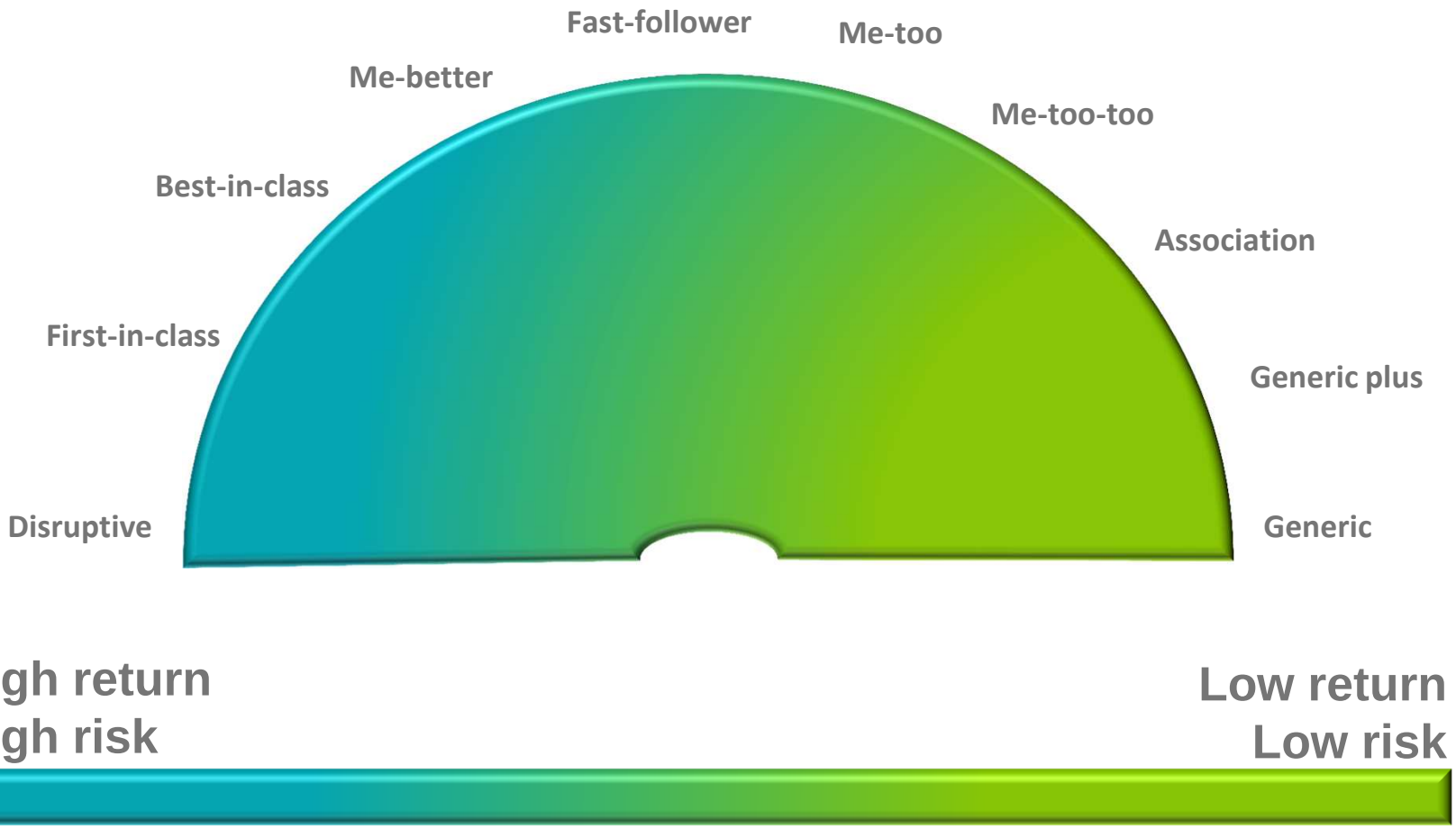
R&D sustainability: Challenges



Modified from S.M. Paul et al. (2010)

R&D **Effectiveness**

The **innovation** field



R&D **Effectiveness**

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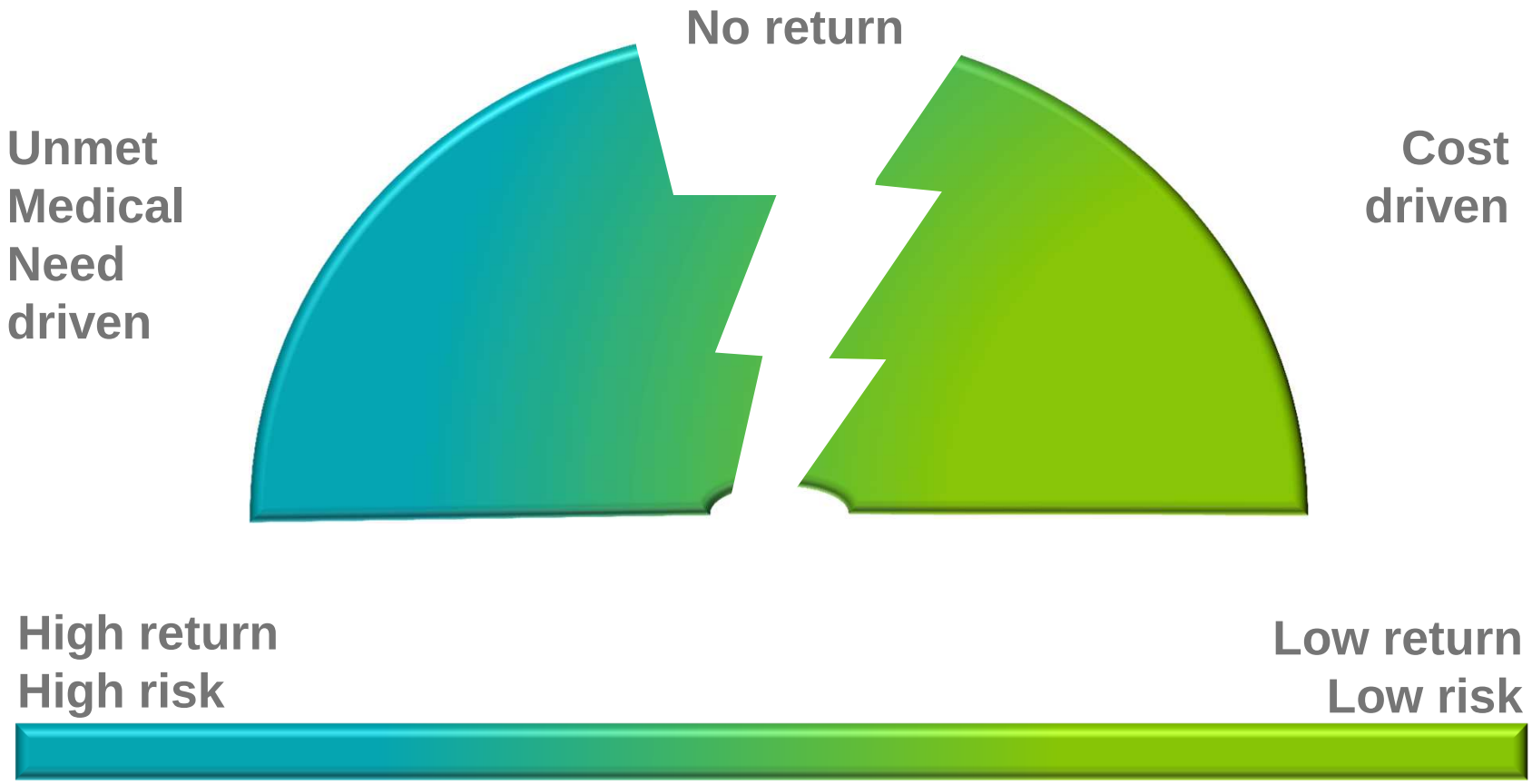

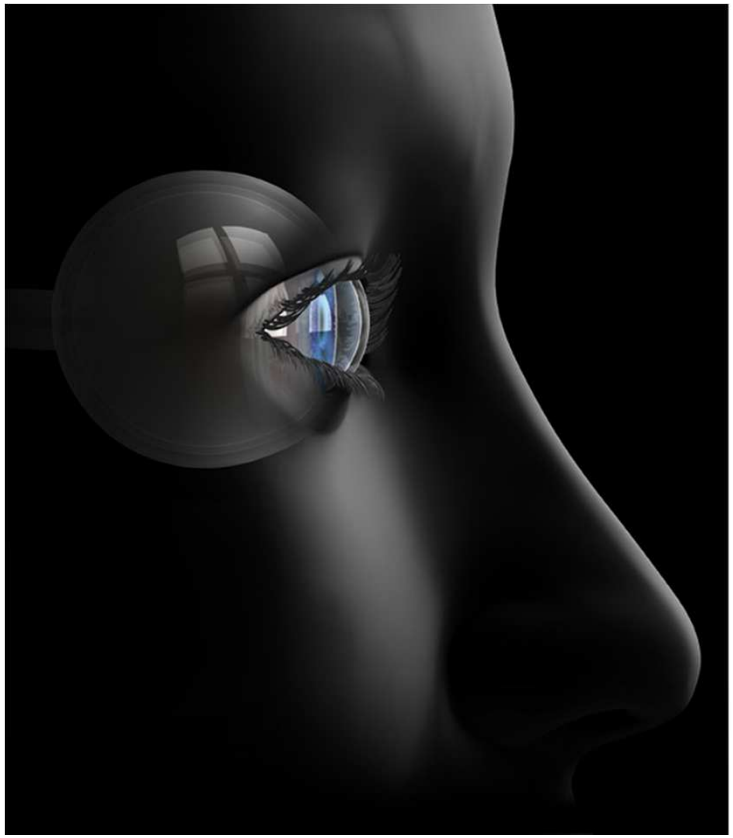

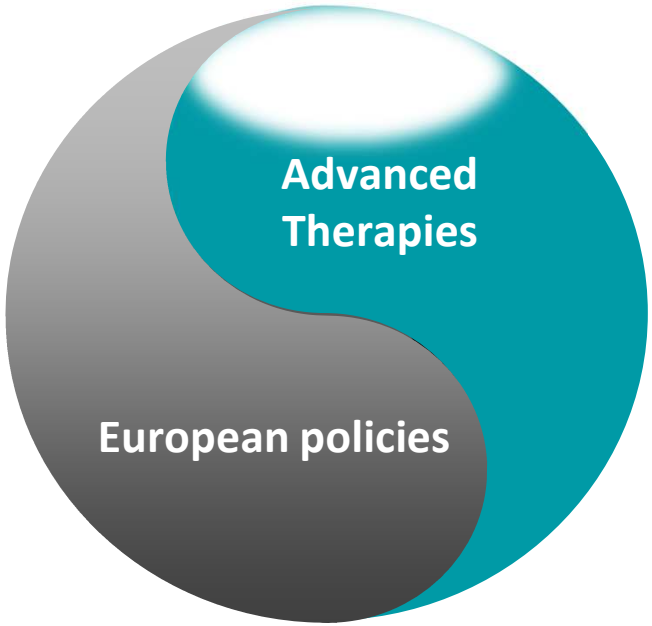

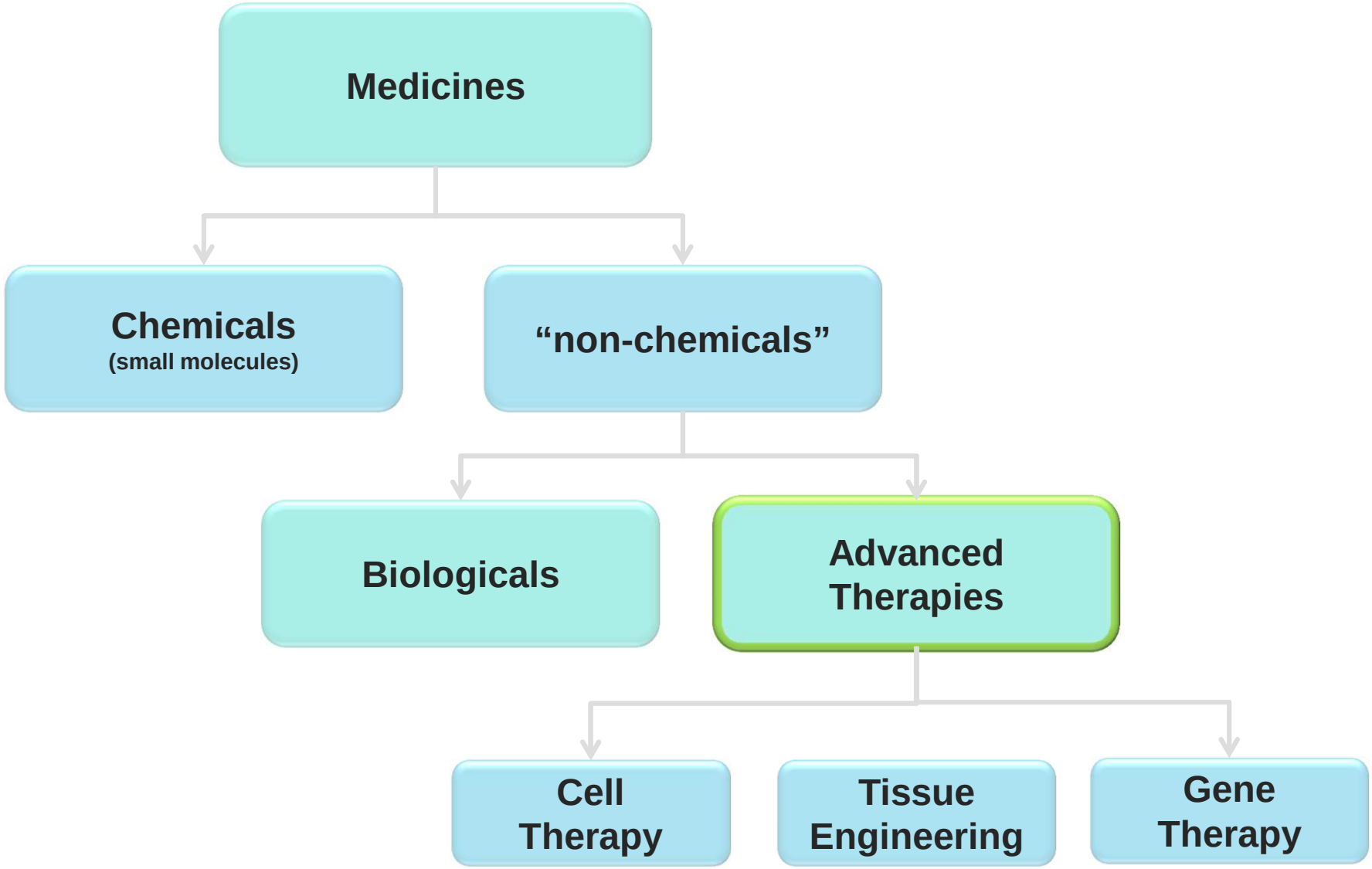
Medicines
Chemicals
(small molecules)
“non-chemicals”
Biologicals
Advanced Therapies
Cell Therapy
Tissue Engineering
Gene Therapy

Gene Therapy

Only one drug approved…

Glybera *(UniQure)*

July 20, 2012 8:40 pm

Gene therapy wins Europe approval

FT FINANCIAL TIMES

By Andrew Jack in London

Europe's regulators have authorised the western world's first gene therapy, paving the way for a breakthrough treatment for an extremely rare genetic disease that**, at more than €1m per patient**, will be the most expensive medicine on the planet.

Gene Therapy

but… No single dose sold after 2 years

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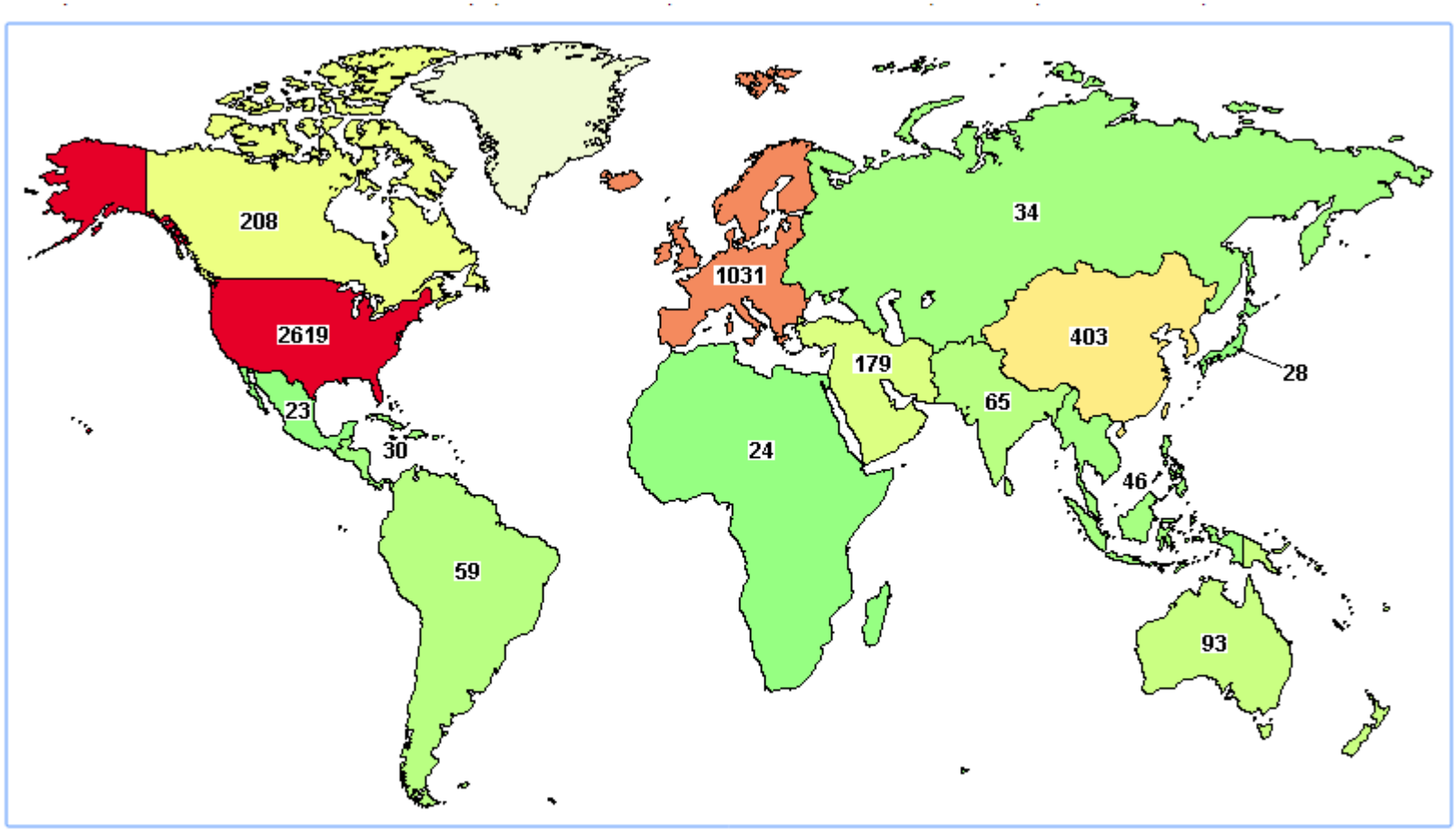
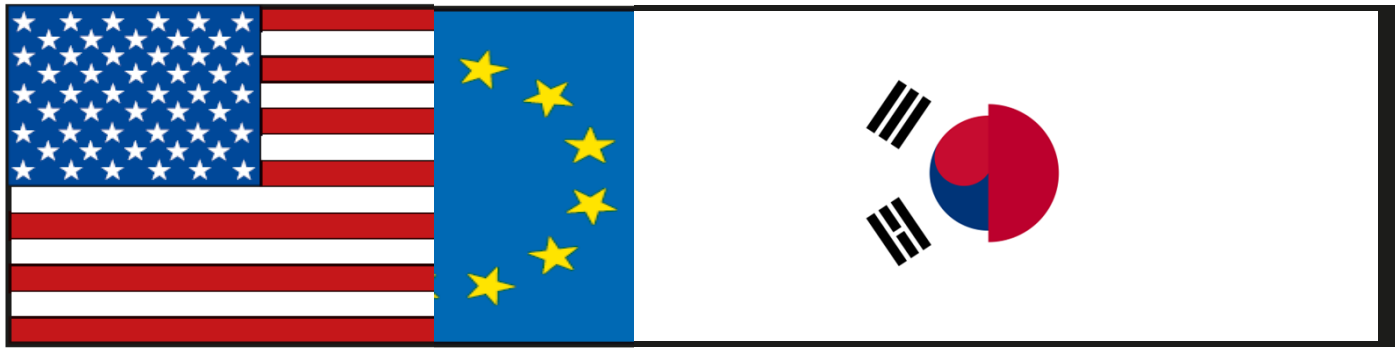
The European paradox



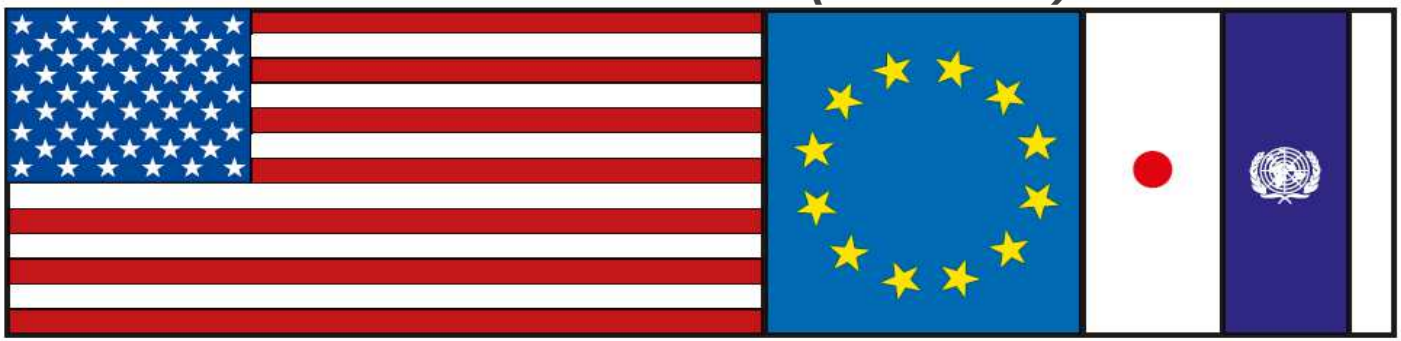
Quo vadis Europe?

Advanced Therapies approved per territory (up to 2013)



Source: FDA, EMA & www.celltherapyblog.blogspot.com.es

Sales of new medicines launched (2009-2013)



Source: EFPIA 2014

March 10, 2014 7:00 am JST

Japan's stem-cell therapy progress accelerates

DAISAKU YAMASAKI and SHIGENORI ARAI, Nikkei staff writers

TOKYO -- Japan's iPS revolution continues to gather pace.

Medical research projects and clinical test plans involving induced pluripotent stem cells are proliferating. **Japanese pharmaceutical companies are racing to claim a piece of the promising market for regenerative medicine treatments**.

New discoveries and potential clinical applications concerning stem cells frequently make headlines in Japan.

The latest came Thursday, when a team of researchers led by Kyoto University's Jun Takahashi announced they have devised a technique to create neurons from iPS cells. The team expects to be able to conduct clinical testing to treat Parkinson's Disease as soon as 2016.

The technique developed by Takahashi's team at the Center for iPS Cell Research and Application involves using blood from patients with the degenerative brain disease to create iPS cells. Those cells are then used to create neurons that are inserted into patients' brains.

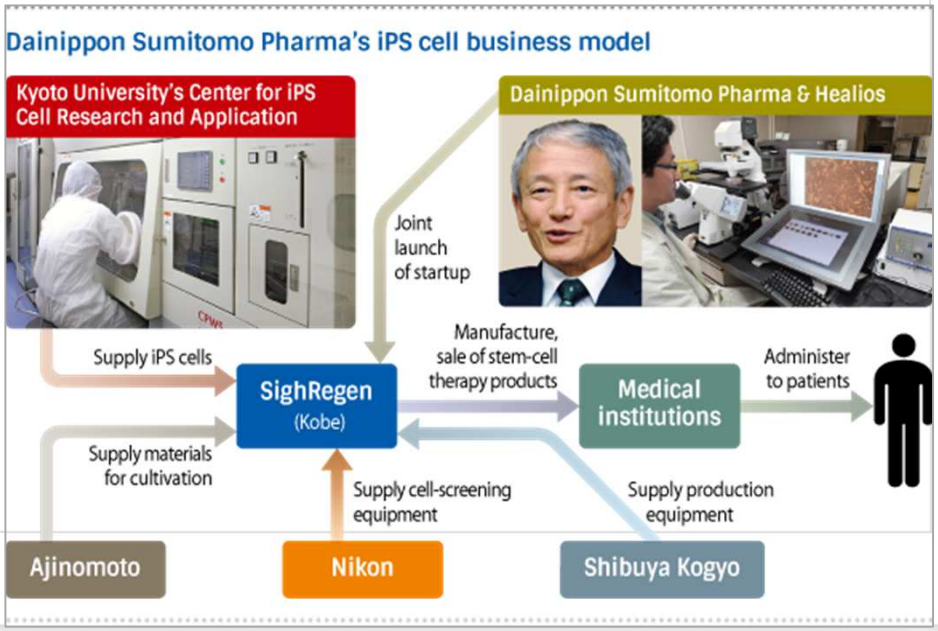
The huge growth potential of the market for regenerative medicine treatments and related equipment has attracted the attention of pharmaceutical companies. **Dainippon Sumitomo Pharma on Thursday set up a joint venture named SighRegen with Healios, a Riken-approved startup based in Kobe.**

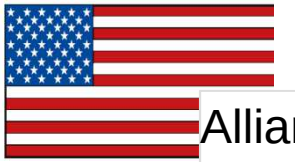
The new venture **will develop iPS cell-derived** retinal pigment epithelial cells for the treatment of age-related macular degeneration and other eye diseases.

"Although this is not an area we are familiar with, we hope to make regenerative medicine a new core business," said President Masayo Tada of Dainippon.

The joint venture aims to develop, manufacture and market Japan's first regenerative medicine treatment, which may be approved as early as 2018. **Dainippon will invest 5.2 billion yen ($50.2 million)** in the development of the therapy. Healios will be responsible for obtaining the Ministry of Health, Labor and Welfare's approval for production and sales.

Dainippon Sumitomo can obtain the vital biotechnology expertise needed to enter the regenerative medicine market from Healios.





Alliance for Regenerative Medicine Announces

Introduction of Regenerative Medicine Promotion Act of 2014 in the Senate

March 13, 2014 1:17 PM

•WASHINGTON, DC--(Marketwired - Mar 13, 2014) - The Alliance for Regenerative Medicine (ARM), the national voice for regenerative medicine, today announced the introduction of the Regenerative Medicine Promotion Act of 2014 in the U.S. Senate and expressed appreciation for the efforts of its lead sponsors, Senators Barbara Boxer (D-CA) and Mark Kirk (R-IL).

"Regenerative medicine represents the single most promising new approach to mitigating the human and economic costs of disease, and changing the course of human health," said Michael Werner, Esq., Executive Director of ARM "Medical innovation and economic growth are important to all of us, regardless of political affiliation, and we are grateful to our lead sponsors, Senators Boxer and Kirk, for making this bill a bipartisan effort. The bill launches a national effort to support these technologies."

The field of regenerative medicine is of national significance because of its acknowledged potential to cure or dramatically diminish the effects of many serious and economically burdensome conditions -- including diabetes, cardiovascular and neurodegenerative disease, cancer and traumatic injury -- by harnessing the restorative properties of living cells and bioactive materials.

"Regenerative medicine offers hope for millions of Americans living with debilitating conditions or diseases," said Amy Comstock Rick, J.D., CEO, Parkinson's Action Network. "By establishing national priorities and coordination of federal agencies around regenerative medicine, this Act paves the way for our families to benefit from breakthrough scientific developments, secures a leading role for the U.S. in the global medical economy of the future, and creates possibilities for new solutions to the healthcare financing crisis."

Major provisions of the bill include **creation of a multi-agency Regenerative Medicine Coordinating Council within the Department of Health & Human Services (HHS); and calling for a detailed assessment of federal activities in regenerative medicine as well as progress compared to national programs in other countries.**

"Multiple federal agencies fund or conduct regenerative medicine research and recognize its promise to transform medical care," said Morrie Ruffin, Managing Director of ARM. "A coordinated effort, such as the one outlined in the bill introduced today, will allow us to advance toward innovative, life-saving therapies and create the regulatory infrastructure necessary to encourage private investment in promising regenerative medicine research," he added.

"Dovetailing with this bill, ARM has outlined a national strategy for regenerative medicine and is seeking rapid implementation of these programs," said Werner. "To date, ARM has worked with the White House, the U.S. Food & Drug Administration (FDA), National Institutes of Health (NIH), National Institute of Standards & Technology (NIST) and members of Congress to further define and promote adoption of this proposed strategy," he added.

Go Europe!

- Regulation (EC) No 1394/2007 of the European Parliament and of the Council of 13 November 2007 on Advanced Therapy Medicinal Products and Amending Directive 2001/83/EC and Regulation (EC) No 726/2004.

Go Europe!

- 28 different Hospital Exemptions
- More than 2/3 still undefined 7 years after…
- “case by case” is the spirit…

european biopharmaceutical enterprises

ebe

EBE position paper on the Hospital Exemption

Conclusions (1)

- The biopharmaceutical industry **appreciates and supports** the therapeutic option for certain patients to receive treatment with a customised innovative product, particularly **in those situations where the disease occurs so rarely that full development and validation of the required therapy is often not feasible**.

european biopharmaceutical enterprises

ebe

EBE position paper on the Hospital Exemption

Conclusions (2)

- However, the HE should be correctly applied and not turn into a parallel circuit for small-scale, locally produced ATMPs competing with centrally authorised products. As a general policy, hospital exemptions should no longer be allowed in those situations where a fully validated, centrally approved ATMP is available for the same indication in the same patient population.

european biopharmaceutical enterprises

ebe

EBE position paper on the Hospital Exemption

Conclusions (3)

- At this moment, there is no European-wide legal certainty on this point. If not addressed, this might lead to undermining the ATMP regulation and ultimately the full clinical development and regulatory control of innovative treatments with important consequences for the patients as well as jeopardising investment by the cell therapy industry as a result of lack of clarity in the regulatory framework.

- **Netherlands:** Case by case assessment. Autologous products, non-autologous products prepared for a single patient, or ATMPs prepared on a small scale (i.e. **maximum 10 treatments for one year**) can be considered non-routine by default. Criteria might evolve in the light of experience.

- **UK**: Case by case assessment based on a set of criteria including the mode of action, the intended use, the manufacturing processes applied and the scale and frequency of the preparation of the specific product. Autologous products are not considered non-routine by default. Criteria might evolve in the light of experience.

- **Germany**: Established definition. ATMPs which are manufactured in small quantities, and in case a routine manufacturing procedure is applied, variations in the procedure are carried out based on a medical justification for an individual patient. Alternatively, **ATMPs which have not yet been manufactured in sufficient quantities to obtain the necessary data to enable a comprehensive assessment are also considered 'non routine'**.

BOLETÍN OFICIAL DEL ESTADO
Núm. 144 Sábado 14 de junio de 2014 Sec. I. Pág. 45068

I. DISPOSICIONES GENERALES

MINISTERIO DE SANIDAD, SERVICIOS SOCIALES E IGUALDAD

6277 *Real Decreto 477/2014, de 13 de junio, por el que se regula la autorización de medicamentos de terapia avanzada de fabricación no industrial.*

CAPÍTULO I

Disposiciones generales

Artículo 1. *Objeto y ámbito de aplicación.*

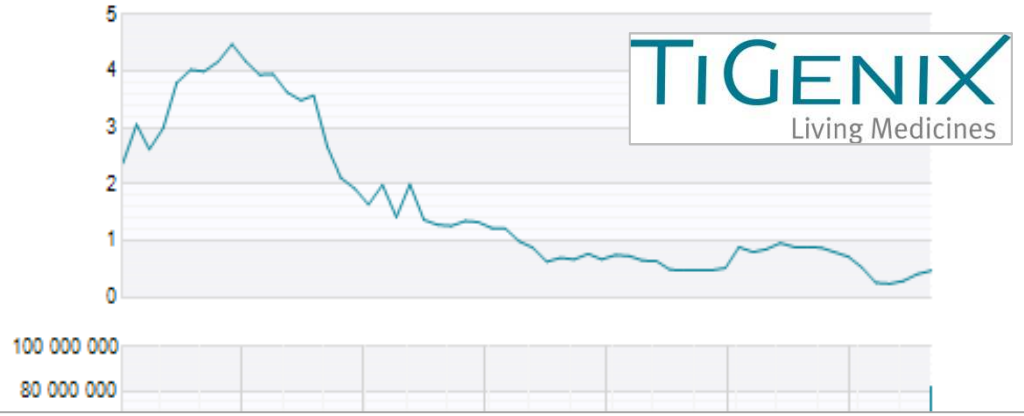
Este real decreto es de aplicación a los medicamentos de terapia avanzada de uso humano que son preparados ocasionalmente, de acuerdo con normas de calidad específicas, y empleados en España, en una institución hospitalaria y bajo la responsabilidad profesional exclusiva de un médico colegiado, con el fin de cumplir una prescripción facultativa individual de un producto hecho a medida destinado a un solo paciente, según definición del Reglamento (CE) n.º 1394/2007 del Parlamento Europeo y

Uso Consolidado	Uso Consolidable?
Cada Hospital su autorización de uso	Pero puede “calcar” el dossier de otro hospital
Cada Hospital manufactura su medicamento	Un hospital puede suministrar a otro
	Un tercero puede suministrar a un hospital
Un producto hecho a medida del paciente	Alogénico

Poor revenues from new arrivals...

Any relationship with the regulatory landscape?

ChondroCelect is only reimbursed in Belgium, The Netherlands and Spain… 4 years after approval



Thousands of Euro (€)	Years ended December 31		
	2013	2012	2011
Belgium	2,023	1,653	591
The Netherlands	1,786	1,949	86
United Kingdom	427	368	213
Other	65	115	256
Total	4,301	4,084	1,146

U.K. Slots over $90M for Large-Scale Cell Therapy Manufacturing Center

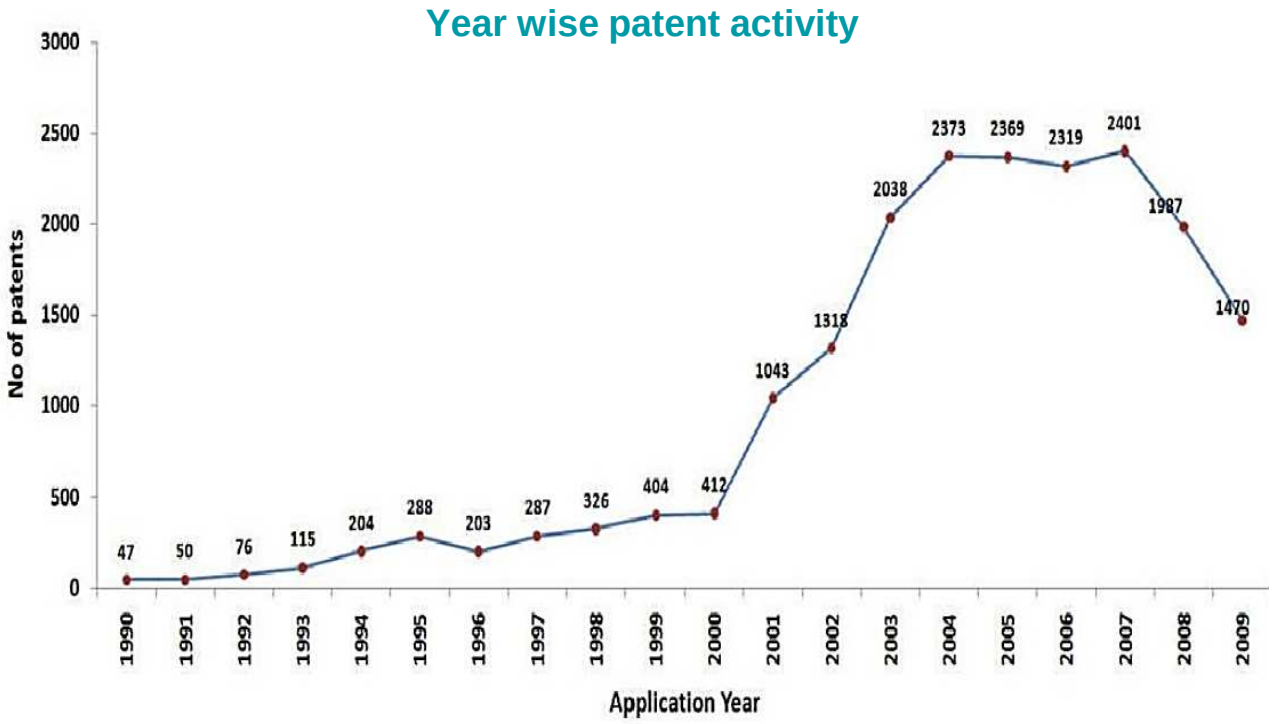
The U.K. government will allot £55 million (around $90.7 million) from its 2014/2015 budget for a new Cell Therapy Manufacturing Center. The Cell Therapy Catapult, a translational center of excellence, will manage the new Center, which is scheduled to open during 2016/17.

Officials at the Cell Therapy Catapult say that an analysis has shown that the U.K.'s small-scale academic facilities are an excellent source of materials for early-stage clinical trials. However, it is expected that this capacity will be full within 3–6 years as the industry's pipeline matures. The Cell Therapy Manufacturing Center will be designed to provide the U.K. with the manufacturing facilities needed for later studies and commercialization, promoting retention of domestic expertise and jobs. In addition, **many global cell therapy organizations with which the CT Catapult has engaged believe that a high-quality EU manufacturing base is essential for bringing their products to the European market**, **and this facility should also help to anchor their activities in the U.K.**, according to a spokesperson for the Cell Therapy Catapult.

The Center will leverage process development expertise at the CT Catapult. It is expected to create up to 100 jobs, and the process for choosing its location will be announced shortly. Forecasts indicate that firms using the center will generate £1.2 billion ($1.98 billion) of revenue by 2020 **(80% via export).**

Cell patenting

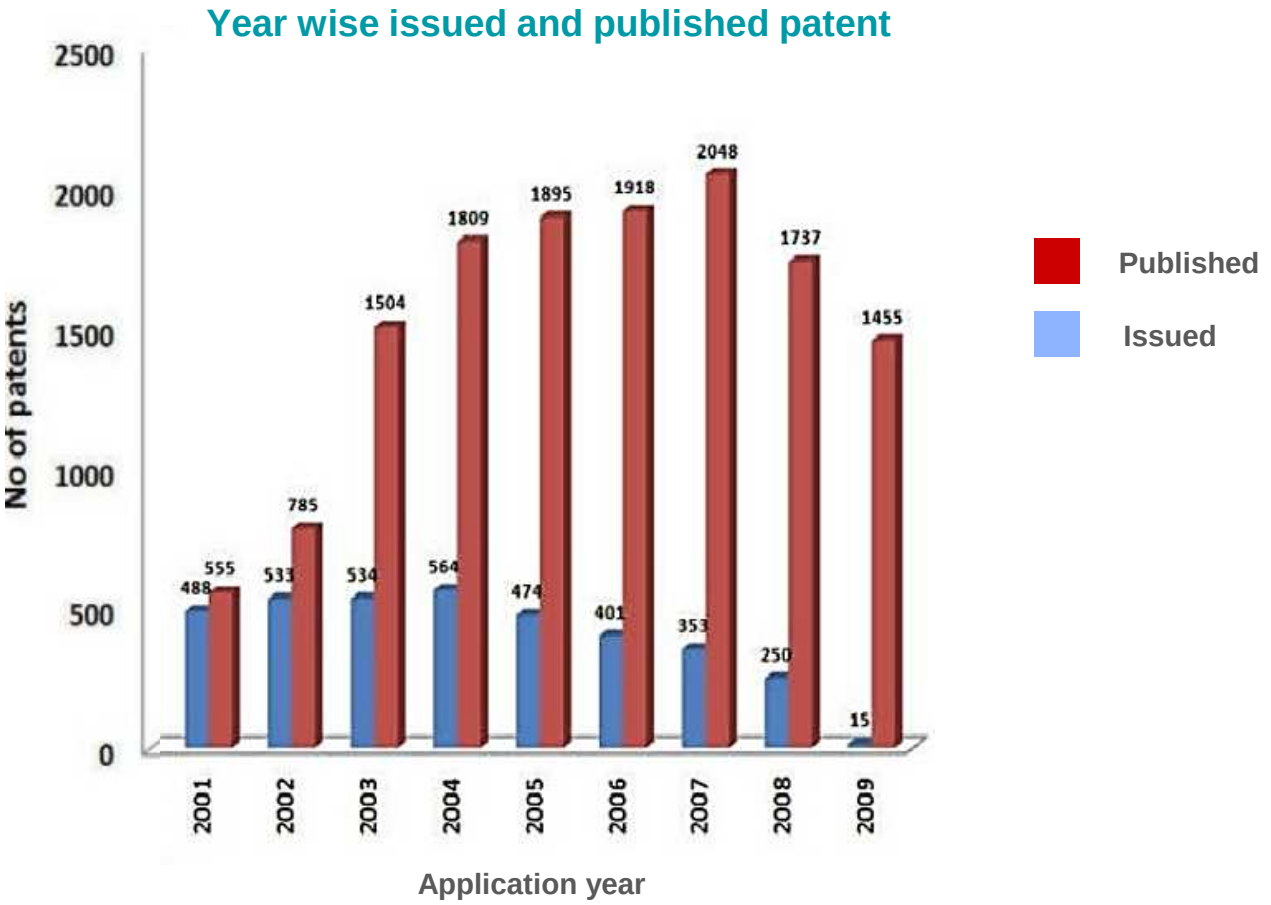
Declining patent activity



Source: ipcalculus 2010

Cell patenting

Low Issue/Published rate



Source: ipcalculus 2010

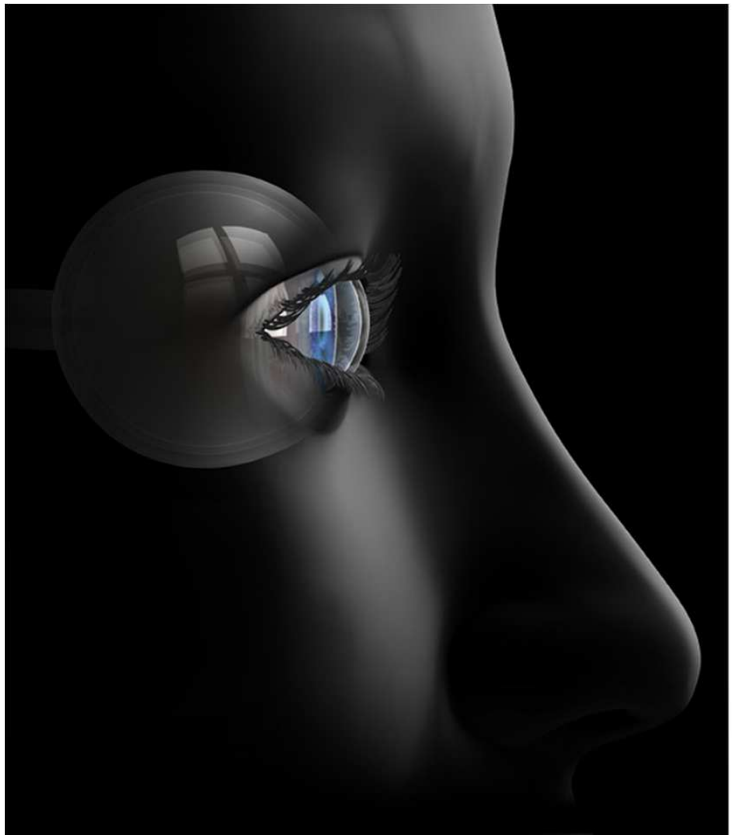
Burnt land danger

What we see is a number of individually *unique* disasters, each marked by a chronic lack of one or another necessity and each, at the end of the day, thereby succumbing to the stem cell entrepreneurs' version of the Field of Dreams Fallacy (*"If you inject them, they will work"*).

It is neither the case that "stem cells work," nor that "they don't work."

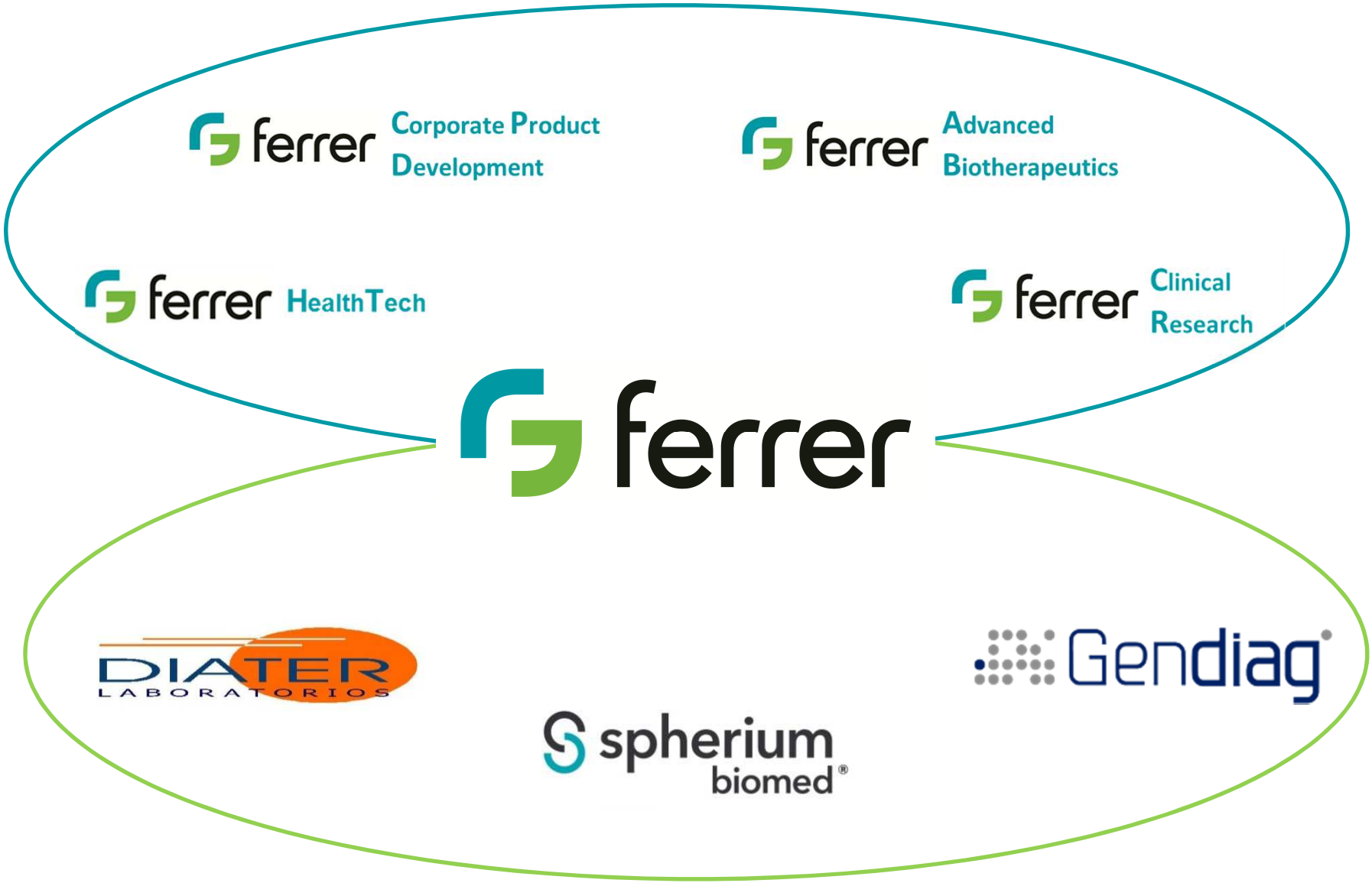
The *right* cell type, applied to the *right* medical condition, delivered to the *right* body compartment via the *right* delivery method, manufactured with the *right* technology and led by the *right* team with the *right* financial resources, behind them will, by definition, 'work.'

Modified from: The Stem Cell Stock Index, October 2013

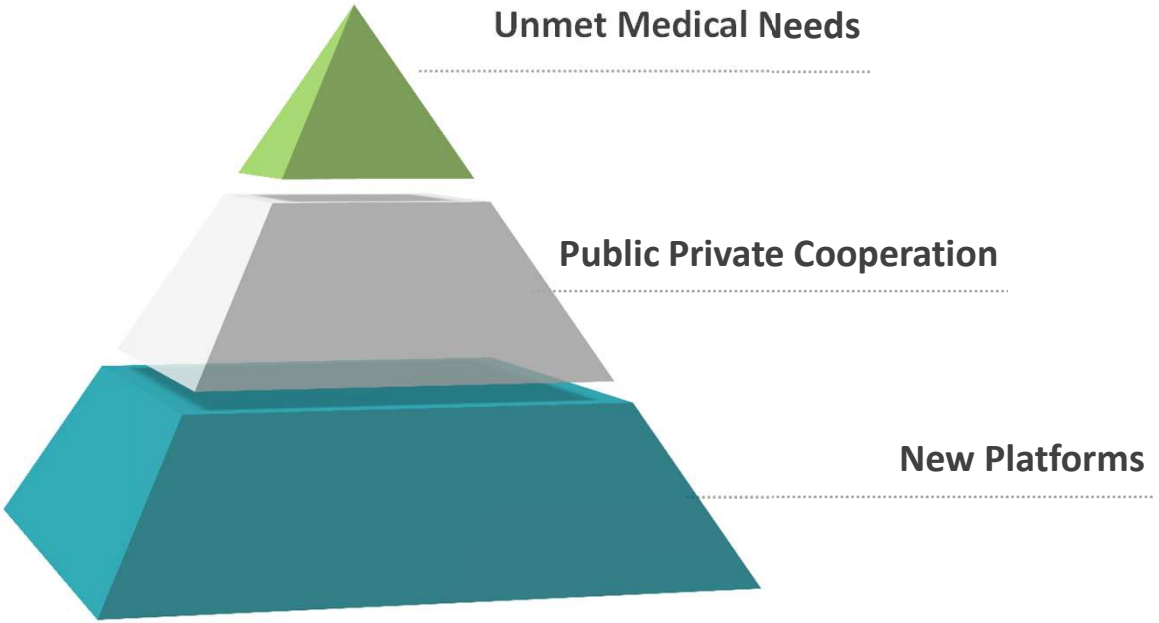


Advanced Biotherapeutics

FERRER: *The Innovation Factory*



ferrer Advanced Biotherapeutics



Gracias!

Making

people

better