

MEDICAMENTOS INNOVADORES
Plataforma Tecnológica Española



Plataforma de Mercados
Biotecnológicos
(Spanish Biotech Platform)

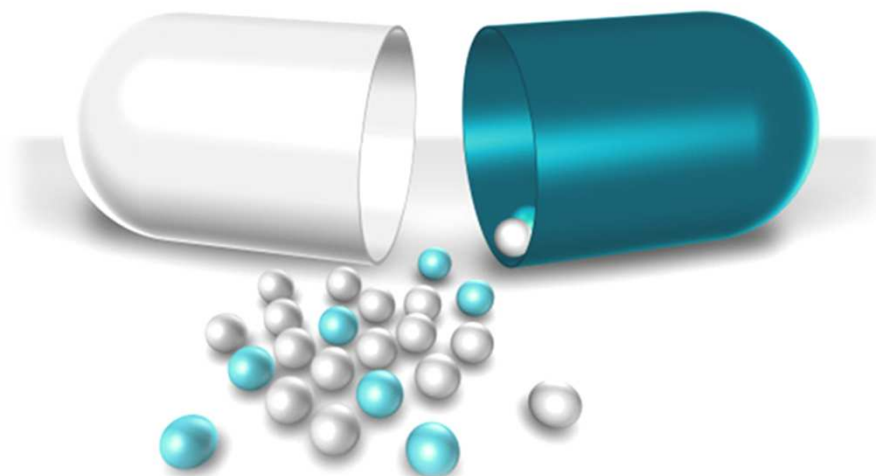
NANOMED
S P A I N

PLATAFORMA
ESPAÑOLA INNOVACIÓN
TECNOLOGÍA SANITARIA

Pharma - Biotech - Academia Cooperation

Collaborative Innovation @ FERRER

Joining our Efforts, Sharing our Future



Andrés G. Fernández
Innovation in Biotechnology
Grupo Ferrer

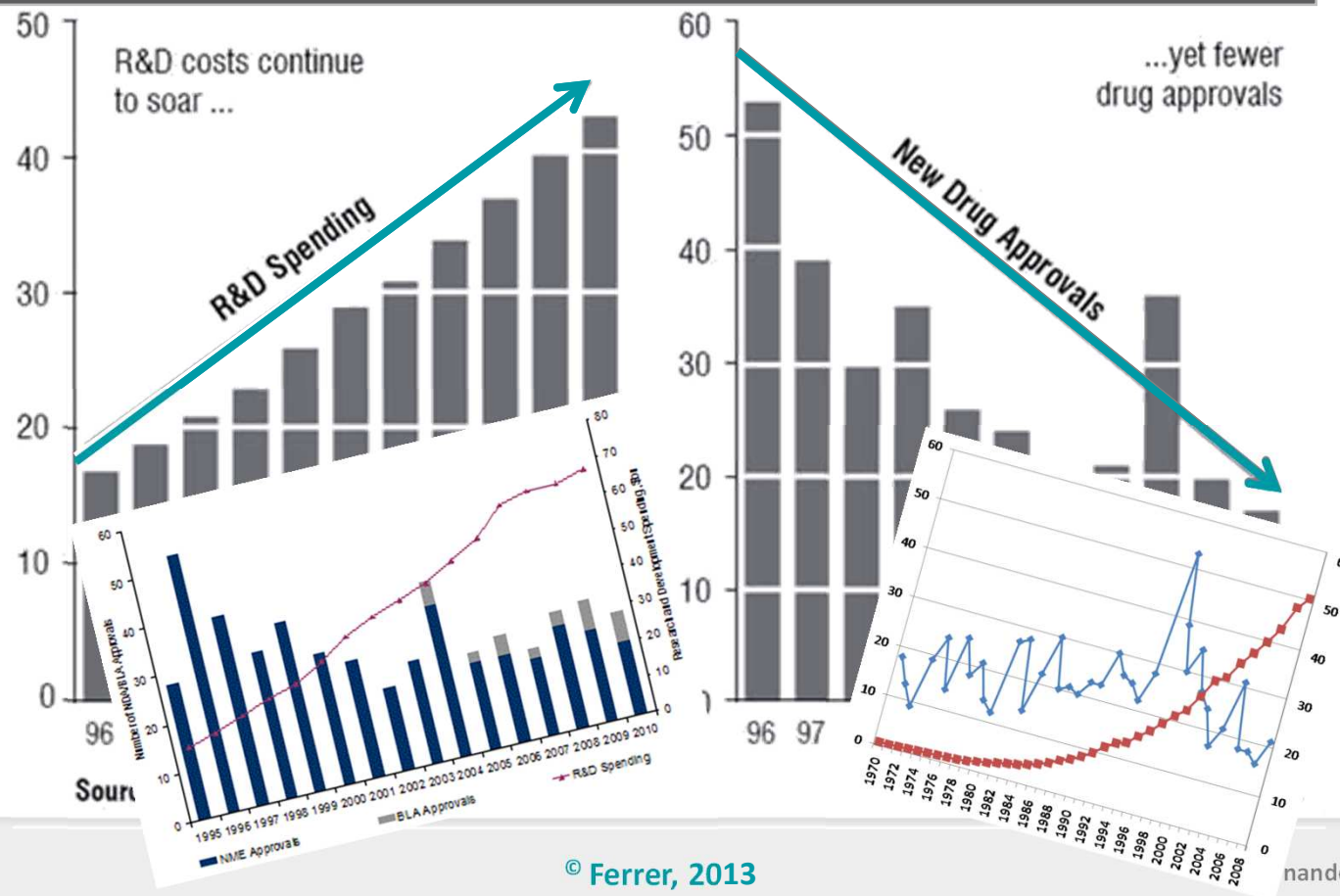


To be *sustainable*
or not to be

Sustainable Innovation

Decrease on productivity...

R&D spending vs. FDA approvals



Sustainable Innovation

1982



Price: 595 USD
GDP per capita (SP): 6.877 USD
Equivalence: 32 days

2010



Price: 631 USD
GDP per capita (SP): 31.700 USD
Equivalence: 7 days

Sustainable Innovation

Is drug innovation sustainable ?

1992



Price/dose: 10 USD
GDP per capita (SP): 14.680 USD
Equivalence: 0.15 days

2010



Price/dose: 2.853 USD
GDP per capita (SP): 31.700 USD
Equivalence: 33 days

Sustainable Innovation

Is drug innovation sustainable ?

Glybera (UniQure)



July 20, 2012 8:40 pm

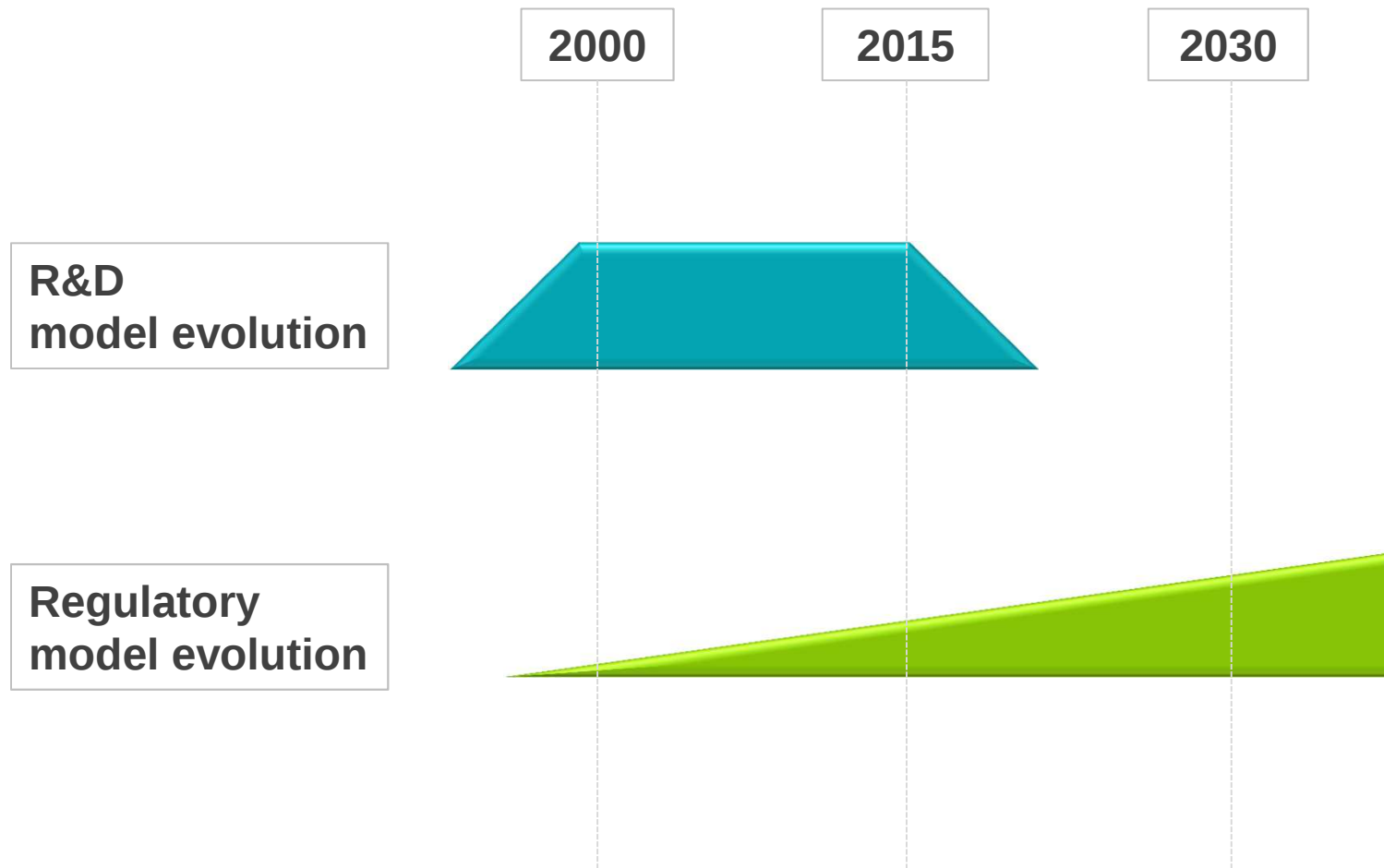
Gene therapy wins Europe approval

By Andrew Jack in London

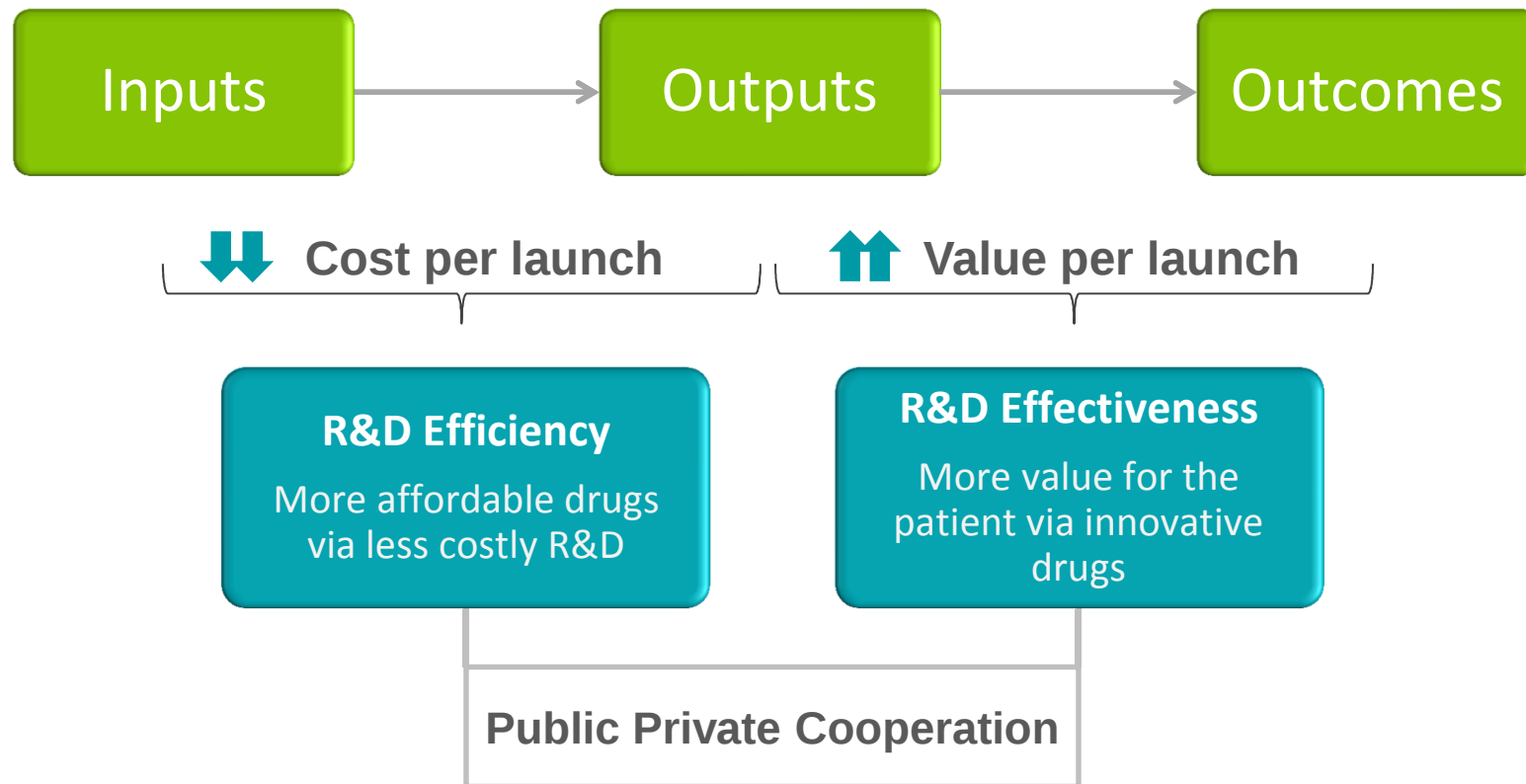


Europe's regulators have authorised the western world's first gene therapy, paving the way for a breakthrough treatment for an extremely rare genetic disease that, **at more than €1m per patient**, will be the most expensive medicine on the planet.

Biopharma Sustainability Challenges



R&D model evolution: Challenges



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

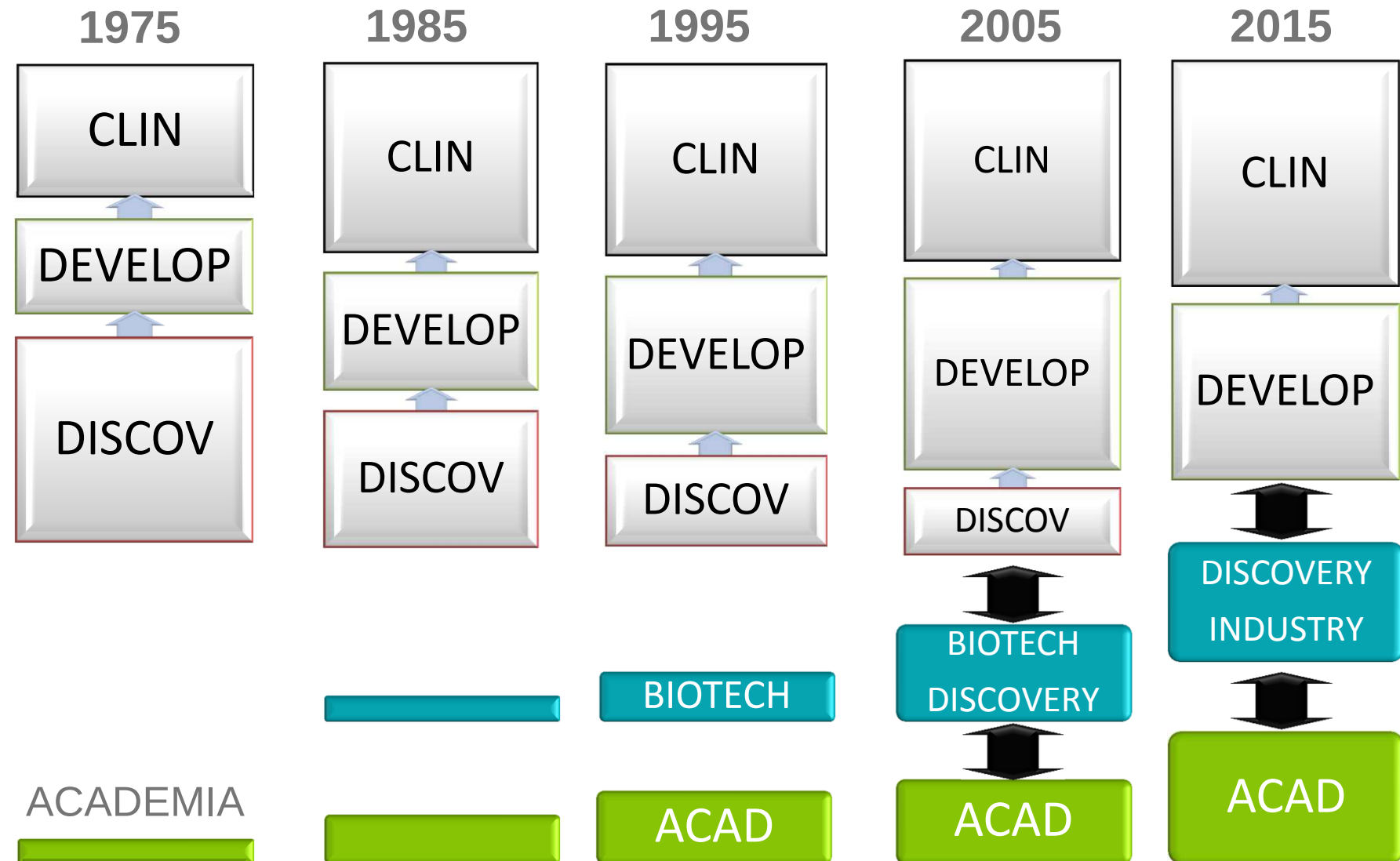
R&D Efficiency



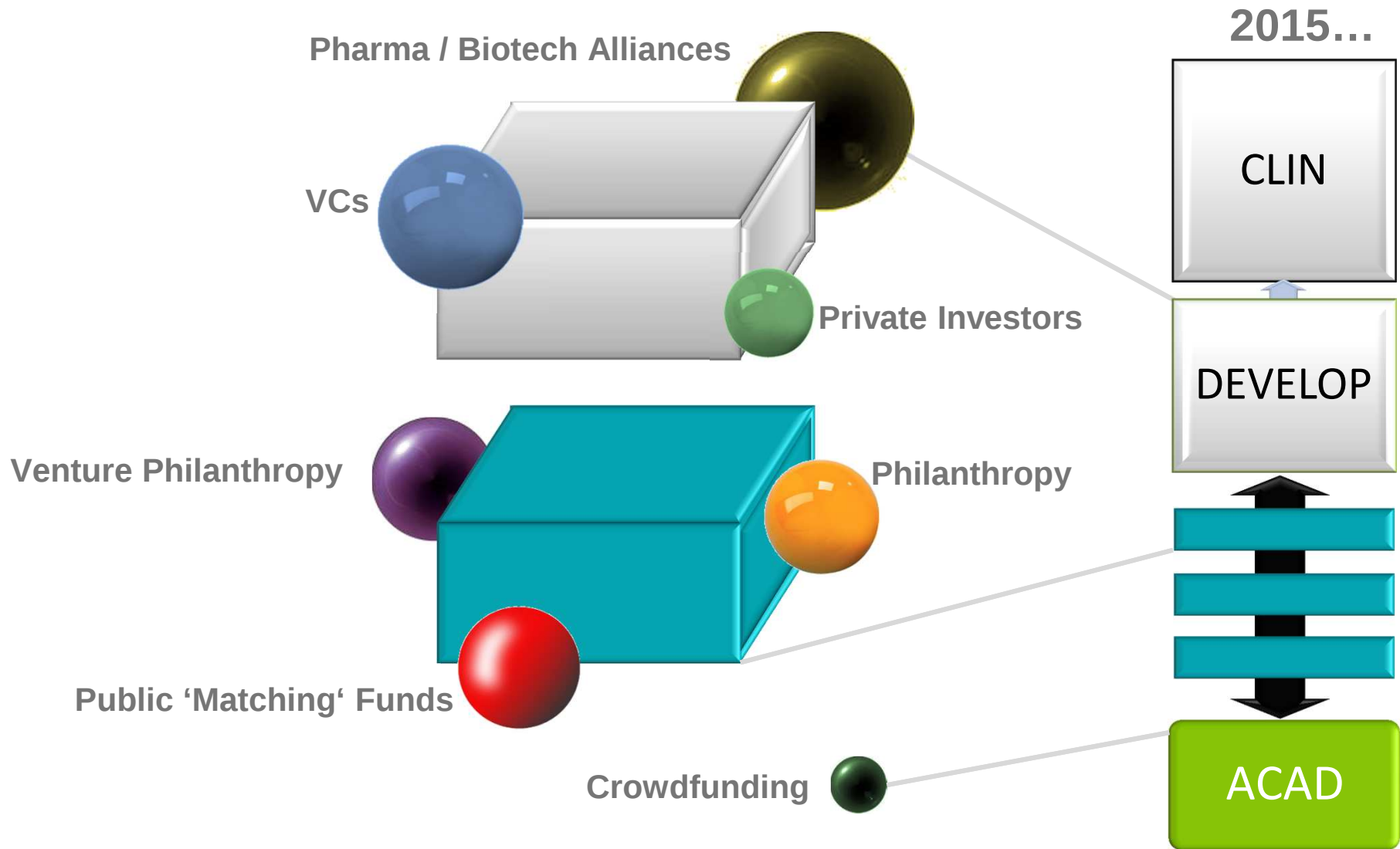
**'True Innovation
doesn't occur inside
Pharma Companies'**

Paul Isherwood
GlaxoSmithKline
Director of Innovation

R&D Efficiency



R&D Efficiency



R&D Efficiency

COUNTRY	GDP (M USD)	R&D (% GDP)	R&D (M USD)	# patents (triadic, 2010)
SWEDEN	520.256	3,37	17.532	882
SWITZERLAND	622.855	3,0	18.685	847
SPAIN	1.340.266	1,33	17.825	242

Source: OECD Factbook 2011-2012: Economic, Environmental and Social Statistics

R&D Effectiveness

The **innovation** factory: a classical view



R&D Effectiveness

The innovation factory: time to change

Unmet
Medical
Need
driven

Cost
driven

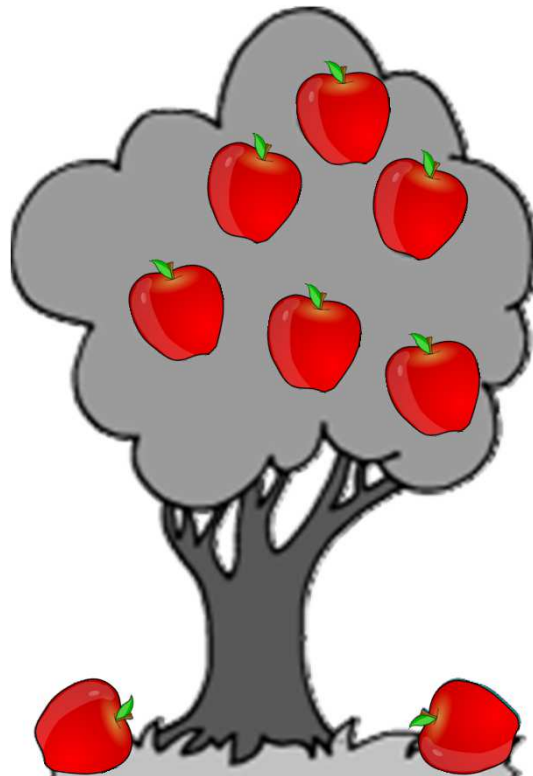
High return
High risk

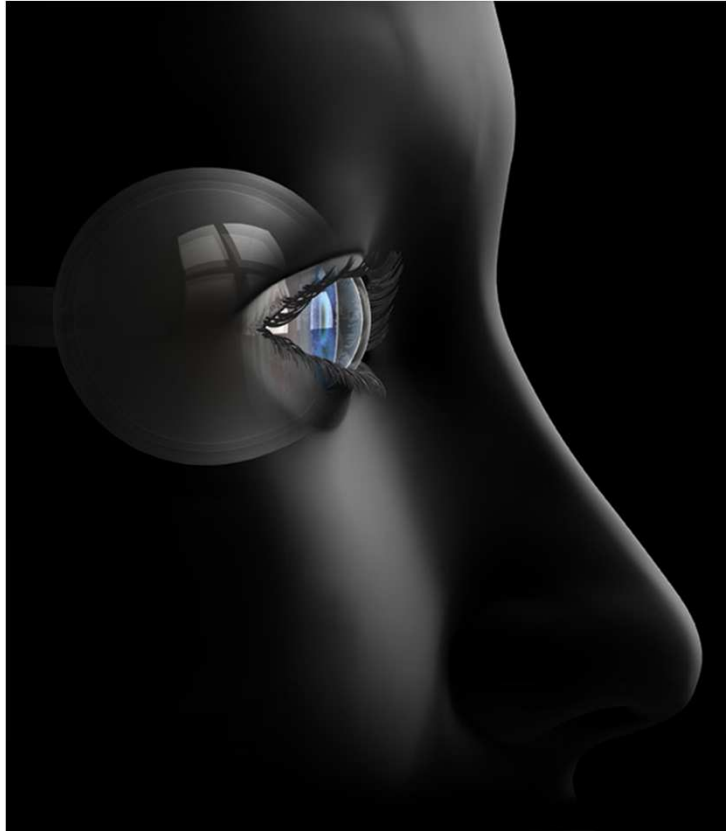
Low return
Low risk



Non-Added Value = high risk

Technological platforms: their pivotal importance





Our model

Collaborative Innovation @ Ferrer



Area of Innovation in Biotechnology



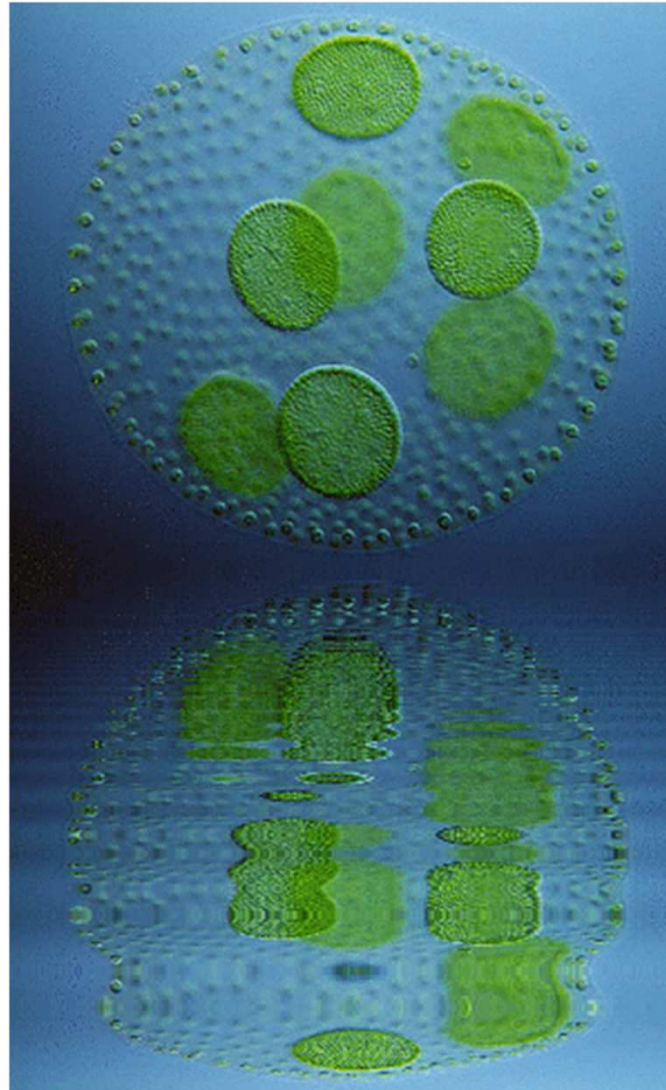
- Focused on expanding Collaborative Research.
- Identifying, incorporating and (co-)managing projects originated outside the company.
- Fully independent of internal R&D funds.

Collaborative Innovation @ Ferrer

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We like to consider
the world as our lab...



What do we look for



Ferrer



➔ Projects yielding **high added value** products

Next slides will define our...



- Development Segment Target (**Entry Point**)
- Main **Strategic Areas**
- Preferred **Technological Platforms**

but, we do also like **OPPORTUNITIES**

What do we look for



Ferrer

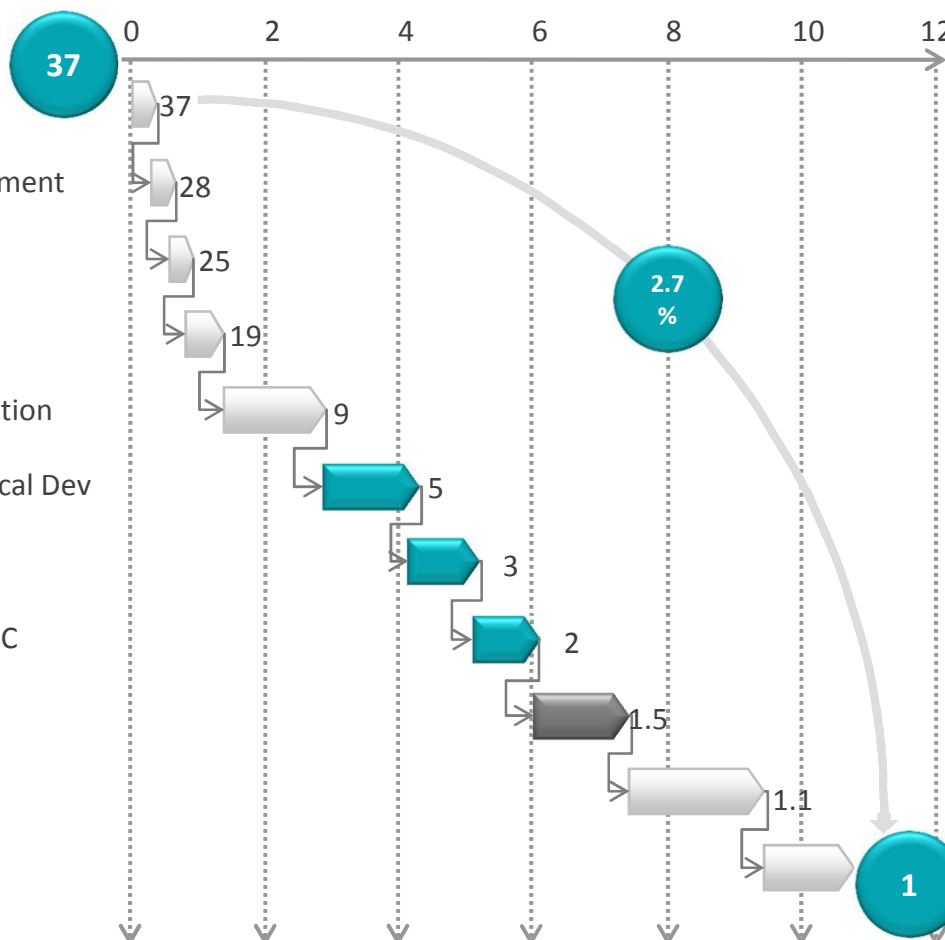


ENTRY POINT

D
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Target ID
Assay Development
HTS Screening
Hit To Lead
Lead Optimization
pPoC / Preclinical Dev
Phase I
Phase IIA / cPoC
Phase IIB
Phase III
Registration



Industry averages

Cost *	Time**	PoS***
~0	<0.25	100%
0.4	0.25	75%
0.5	0.25	90%
1.2	0.5	75%
9.0	1.5	45%
5.3	1.25	60%
9.3	1	67%
12.5	1	60%
32.1	1.5	75%
98.0	2	75%
11.8	1.5	88%

* MUSD

** Years

*** Probability of Success

Source: Gaviraqui, 2011

What do we look for



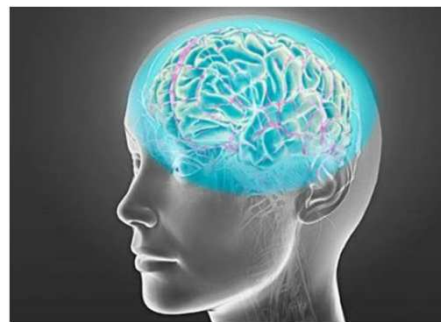
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Main Strategic Areas

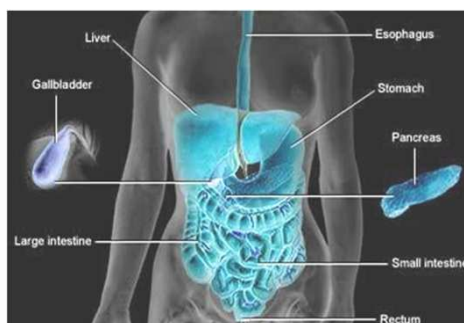


Cardiovascular



Neurology

Psychiatry



Gastrointestinal

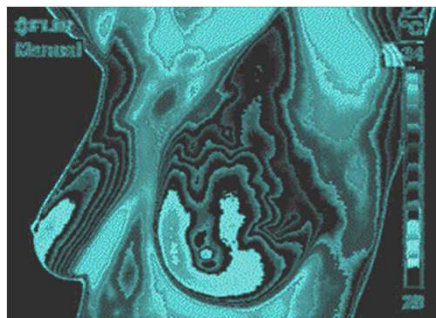
What do we look for ?



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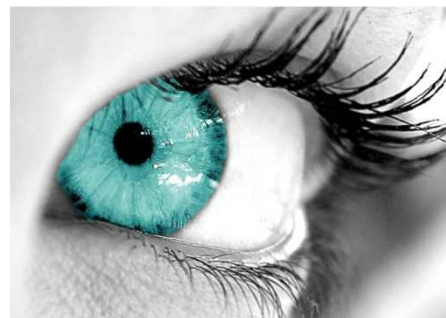


Main Strategic Areas

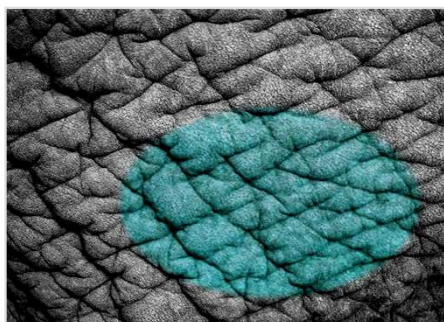


Oncology

Oncology support



Ophthalmology



Dermatology

What do we look for

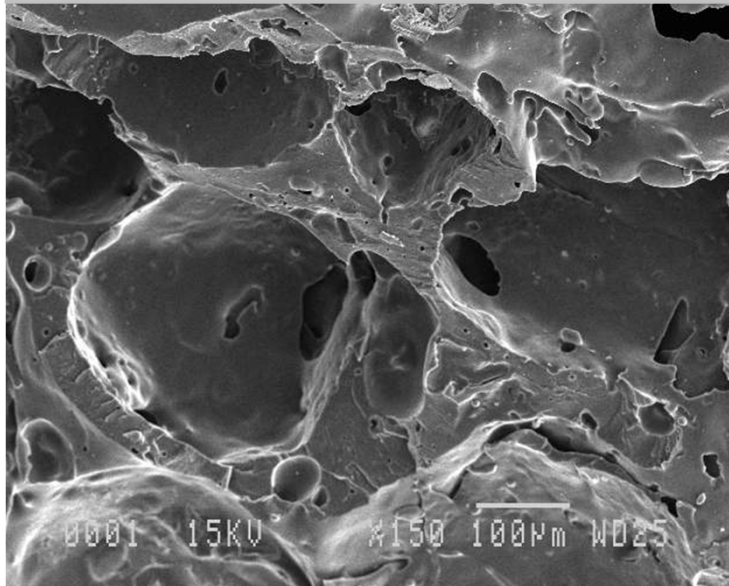


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Preferred technological platforms

Regenerative Medicine



- Ophthalmology
- CNS
- Specific opportunities
- Technology scouting:
 - Reprogramming evolution
 - New biomaterials

What do we look for

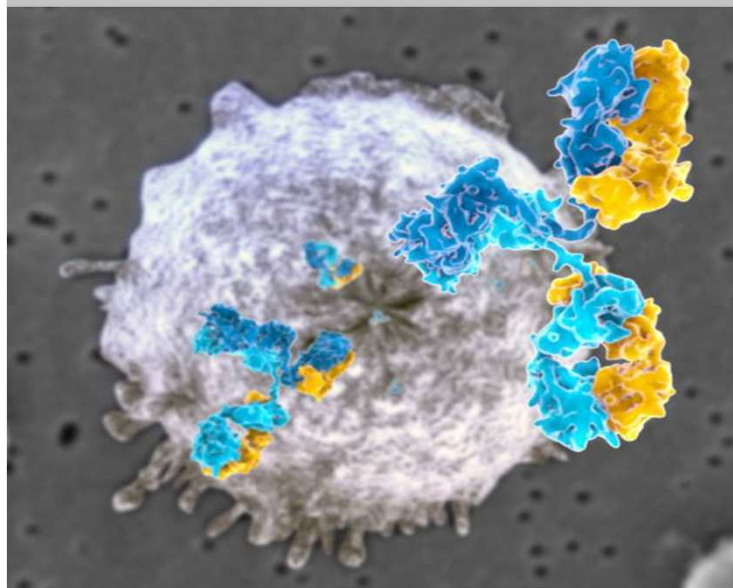


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Preferred technological platforms

New Drug Therapies: antibodies



- New Generation Antibodies
 - LMW: Nanobodies
 - Bispecific – Trispecific antibodies

What do we look for

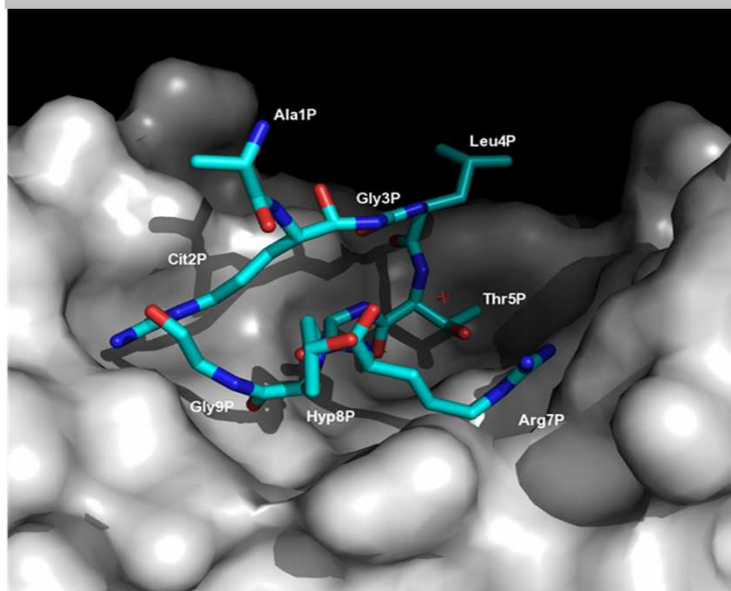


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Preferred technological platforms

New Drug Therapies: New GPCRs modulators



- Deorphanised GPCRs: focus in CNS
- “Valued” Oligomeric GPCRs: focus in CNS
- PAM, NAM & bitopics discovery technologies or opportunities

What do we look for ?

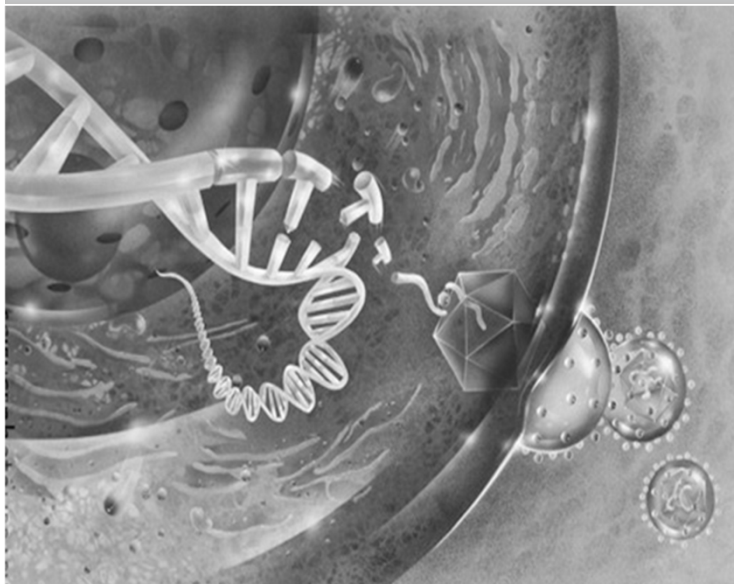


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Preferred technological platforms

Gene Therapies



- Plasmids
- Very specific/singular opportunities in:
 - siRNA
 - Antisense
 - Oncolytic viruses

What do we look for



Ferrer



Preferred technological platforms

Repositioning



- Strong IP status
- Fast development

What do we look for

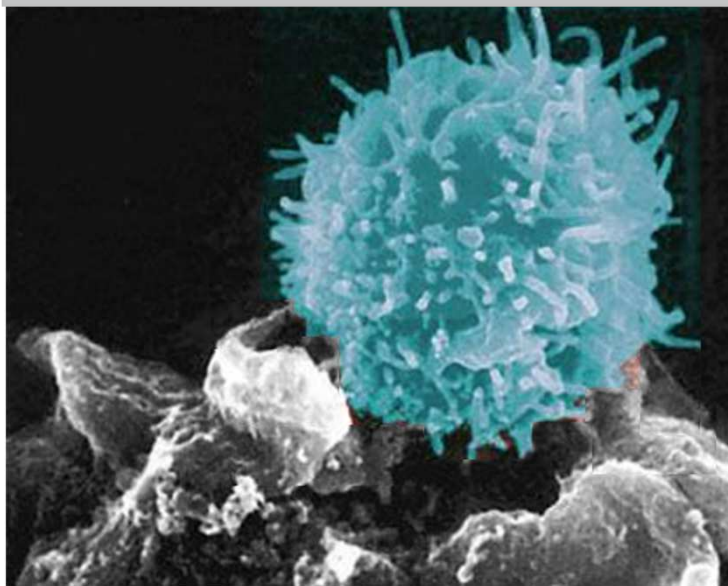


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Preferred technological platforms

New Protein Production or Delivery Technologies applied to



- Rare diseases
 - Protein deficiency
- Very specific opportunities in therapeutic vaccines
 - Advanced stage of development

What do we look for

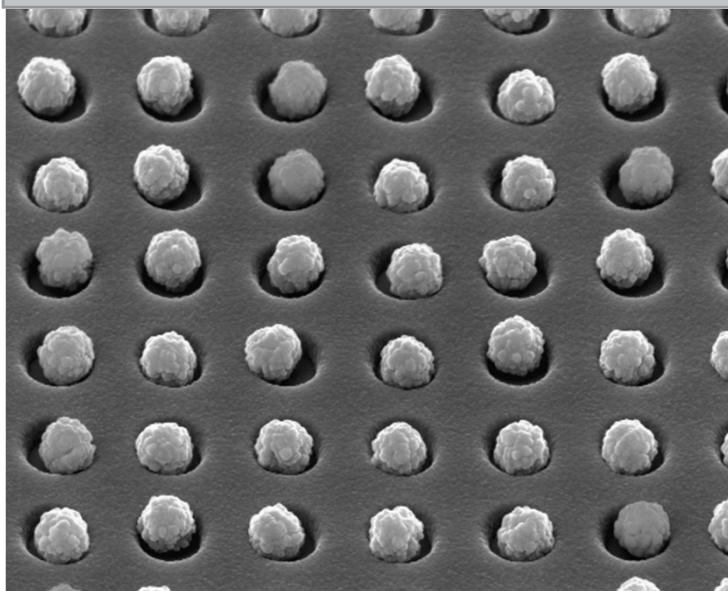


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Preferred technological platforms

New Frontiers in Personalized Medicine



- Theranostics
- Epigenetics
- Metabolomics
- Transcriptomics
- Non-image based CNS diagnostic tools

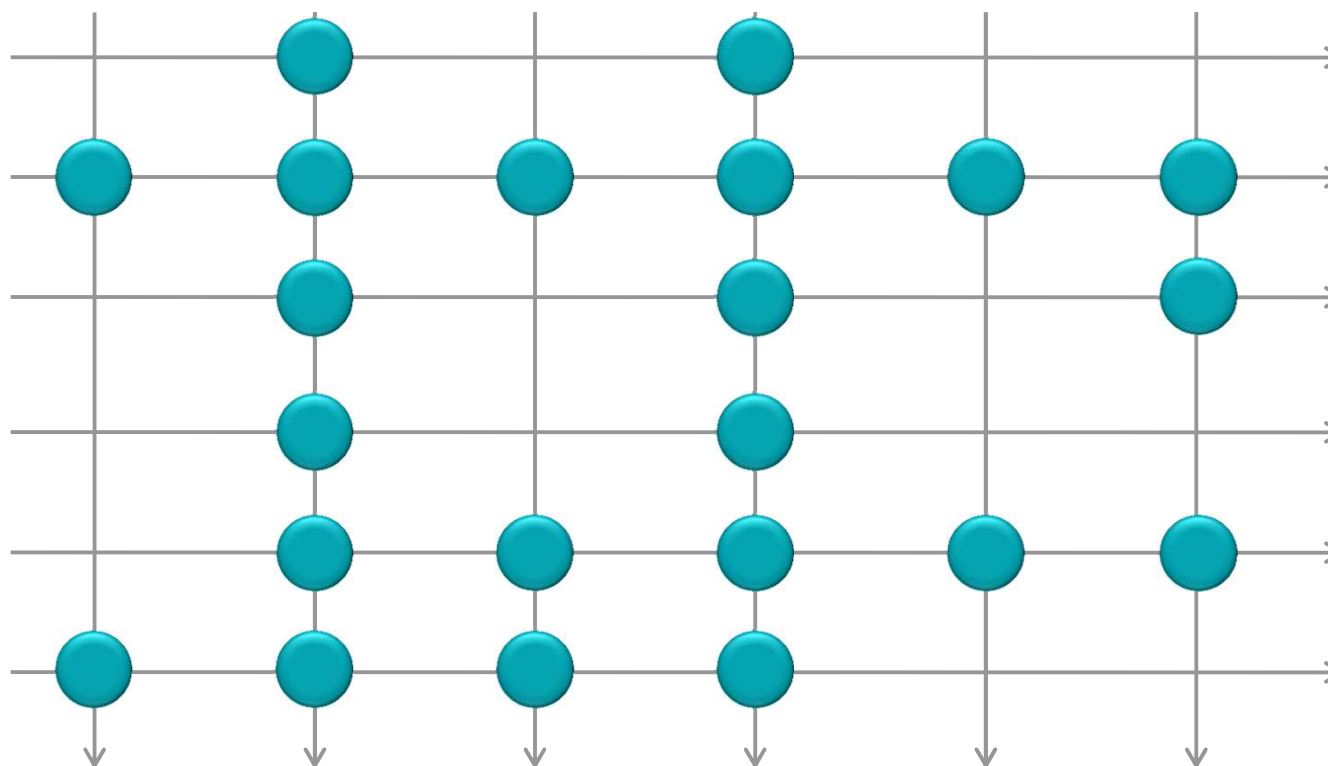
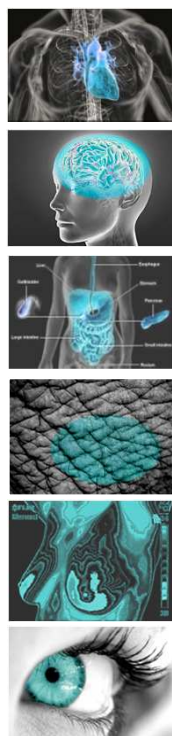
What do we look for



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Reg Med New Drug Gene Th. Reprof. Biolog. Theranos/
Diagnos.



...and opportunities

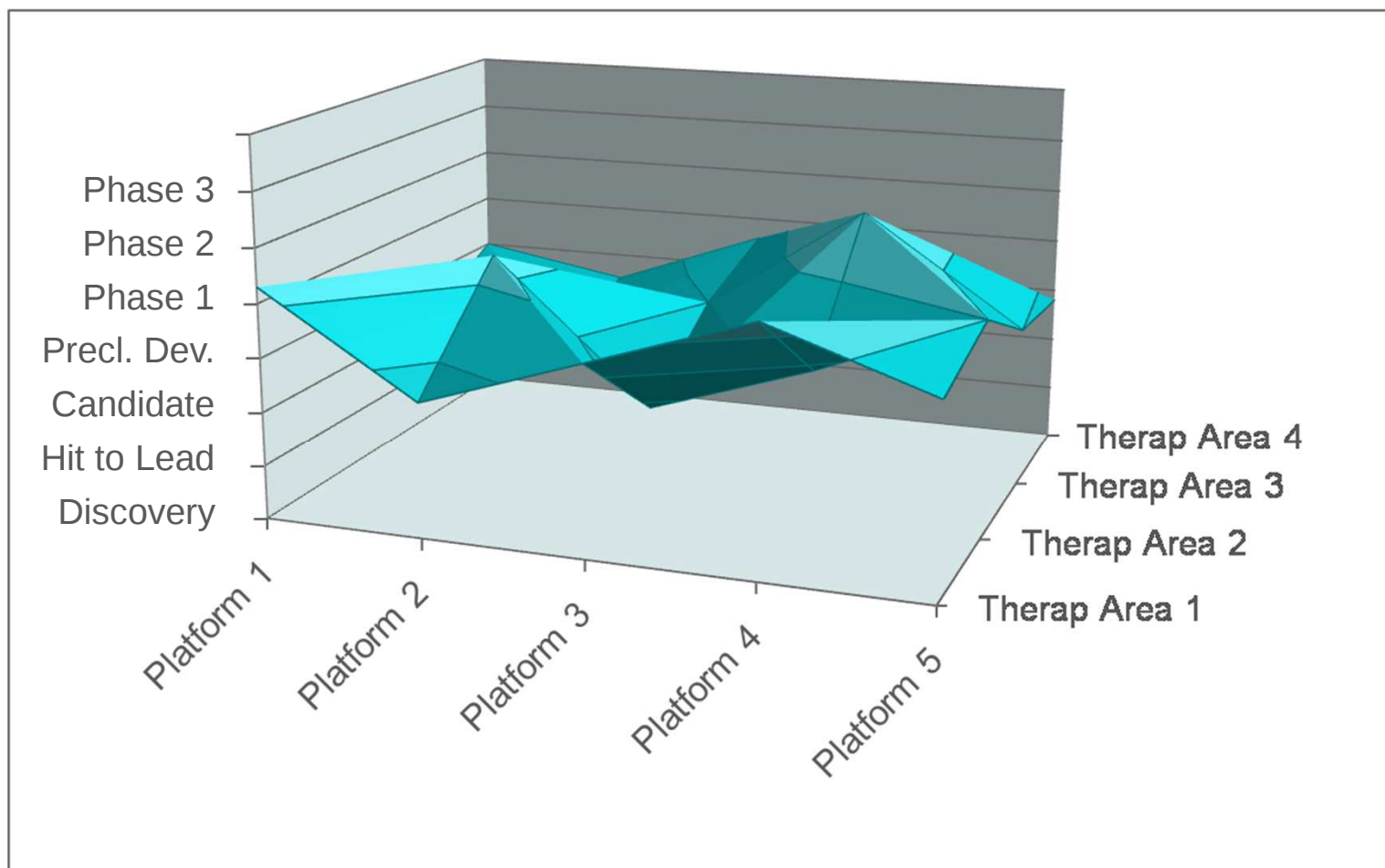
What do we look for

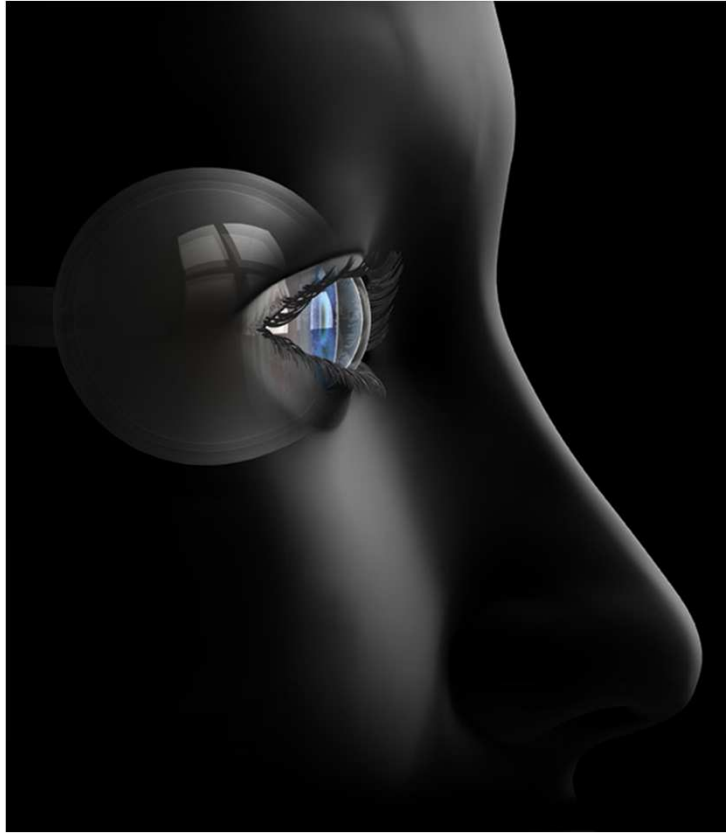


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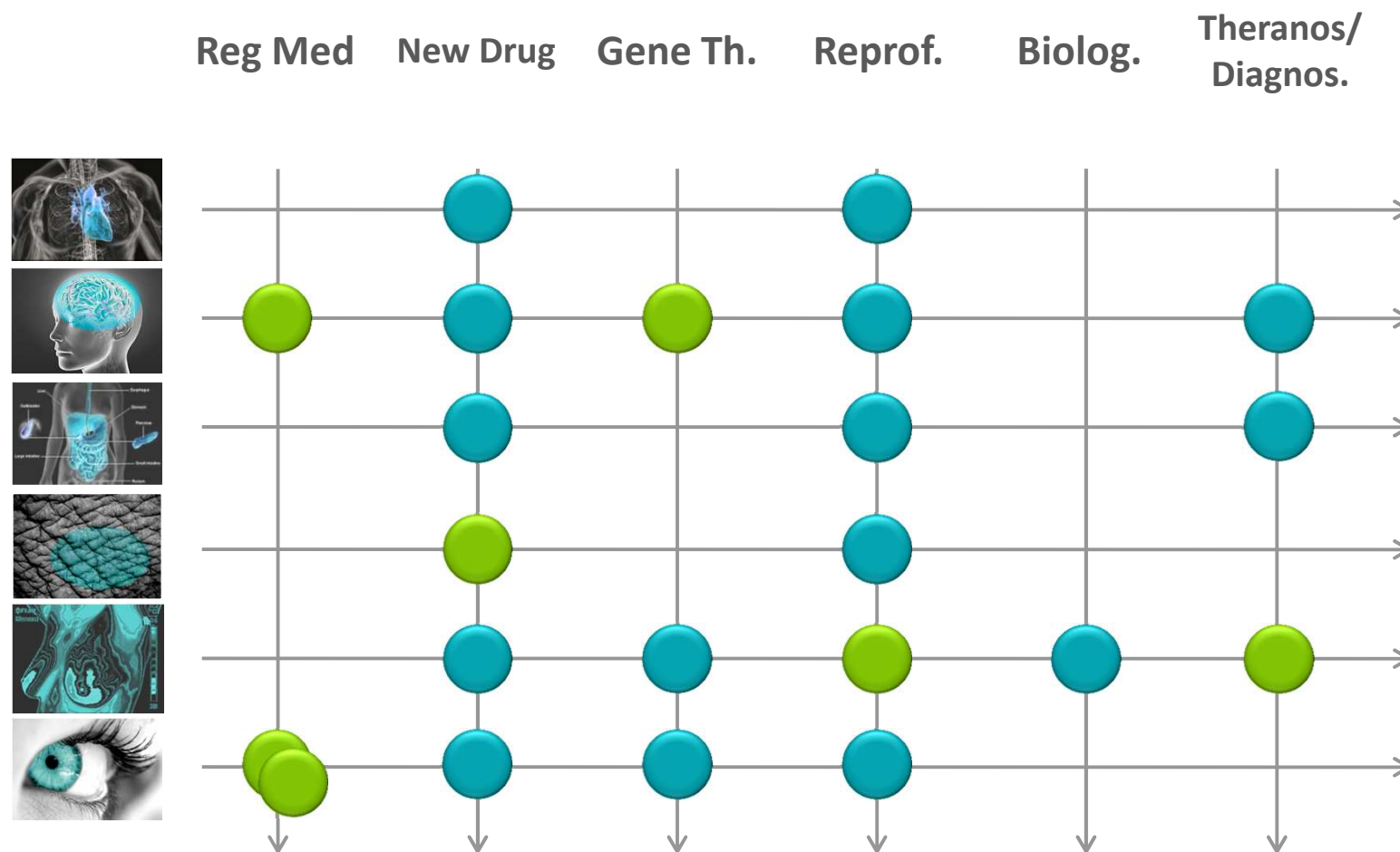
Pipeline: an idealized 3D representation





Our Projects

Our matrix of interests



...and opportunities

Ongoing FERRER collaborative projects

Project Code & Partners		1 st Indication	2 nd Indication	Advanced therapy	Small molec.	Dx
FIB-111		Retinitis Pigmentosa	AMD			
FIB-112	 	Corneal damage				
FIB-114		NHL	CLL			
FIB-115		CUP diagnostic				
FIB-116	 	Psoriasis	Atopic D.			
FIB-117		Spinal Cord Injury	TBI			
TTC-ELA	  	ALS				



Making
people
better