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UNIVERSITÀ DEGLI STUDI DI TRIESTE

I punti di forza di Trieste: il sistema della ricerca e dell'innovazione

Trieste

Adriatic sea

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In a Quiet Corner of Italy ... Trieste

Chiara Goia for The New York Times

Late afternoon soccer in the Piazza dell'Unità d'Italia, Trieste's central square. More Photos »

By ADAM BEGLEY
Published: April 29, 2011

A MEDIUM-SIZE seaport teetering on the edge of what we recognize as Italy, Trieste is a mysterious and puzzling place. Its iconic central square, the Piazza dell'Unità d'Italia, bounded on three sides by comically pompous 18th- and 19th-century buildings, most of them decorated like big, boxy wedding cakes, is wide open to the Adriatic, as though the ever-changing seascape were an entertainment staged for the city's benefit. This vast, glorious piazza promises all sorts of civic delights, but in fact it's one of the few immediately gratifying spots in Trieste. The rest takes time — exactly what most of us are unwilling to give up.

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THE SESSIONS COMING SOON

Trieste Travel Guide
Where to Stay
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With a convoluted history of serial conquest, culminating in a century-long tug-of-war between Italy and Austria, a melting-pot population, a street plan that ranges from serenely rational to bewilderingly crooked and steep, and a forbidding

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Late-summer sun - FT.com 7/6/12 9:29 AM

FINANCIAL TIMES

August 27, 2010 6:22 pm

Late-summer sun

By Jan Morris and Joanne O'Connor

If there is one city of the Mediterranean coast that is best visited in the autumn, it is surely Trieste, pre-eminently an autumnal city itself, **writes Jan Morris**. It is perpetually experiencing the autumn of its days, never having recovered the glory of its importance as the principal seaport of the Austro-Hungarian empire. Ever since the collapse of that imperium, and Trieste's absorption into Italy, it has been a place whose allure is the allure of autumn, when the leaves are lying in the side-streets dreaming of sunnier times.

I love Trieste, and I love it largely because of its innate melancholy. It has its bathing beaches and its boulevards but it has never been a holiday resort, and most of its summer visitors spend only a day or two on their way to the coast of Croatia. In its great 19th-century days it was a powerful international seaport – in effect the port of Vienna and central Europe – and when the Habsburgs faded into history, they left behind, in a wistful kind of way, many mementos of their presence. Autumn is the time to enjoy them! Autumn, season of mists and mellow fruitfulness, when you can idle away the hours in the old Viennese cafés of Trieste, drinking some of the best coffee in Europe, chatting with some of the most amiable people, contemplating the great figures of European literature who have idled here before you.

Contemplating history too, for in Trieste the past is always alive. For me one of the great pleasures of travel, especially when summer is over, is to sit over my coffee (fairly well wrapped up) at the Caffe degli Specchi in the great Piazza Unita on the waterfront at Trieste. All around the square, children (also well wrapped up) are running around, playing football, pushing toy prams, laughing among the pompous civic architecture of the place, while their proud mammas keep careful eyes upon them from the café terrace. Then I can imagine, all too clearly, the great old steamships tied up at the quay, and the military band playing waltzes or stirring marches in the bandstand outside the governor's palace, and James Joyce writing a poem at the next table, and some be-feathered imperial general, his sword propped up beside him, treating his aide de camp to a *grappa* while they prepare the order of the day's parade.

Hardly a holiday, you may think? Not a holiday in the tabloid, travel agent, cut-rate airline sense, but a holiday out of the summer, a holiday in the maturing sun. And with any luck a

http://www.ft.com/cms/s/2/6f527ba4-b169-11df-b899-00144feabdc0.html#axzz1zpA1z1c5 Page 1 of 3

sabato 11 settembre 2010

Trieste raccontata su "Life&Arts", supplemento del "Financial Times".

Un'affascinante descrizione dell'atmosfera di Trieste, nell'articolo di Jan Morris e Joanne O'Connor pubblicato sul supplemento mensile del "Financial Times" di agosto.

Lo riporto qui integralmente. Credo sia motivo di soddisfazione per tutti noi il fatto che Trieste trovi spazio, per la prima volta in chiave "turistica", su una testata così autorevole e diffusa.

Late-summer sun
by Jan Morris and Joanne O'Connor
Published: August 27 2010 18:22

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LE VOSTRE FOTO

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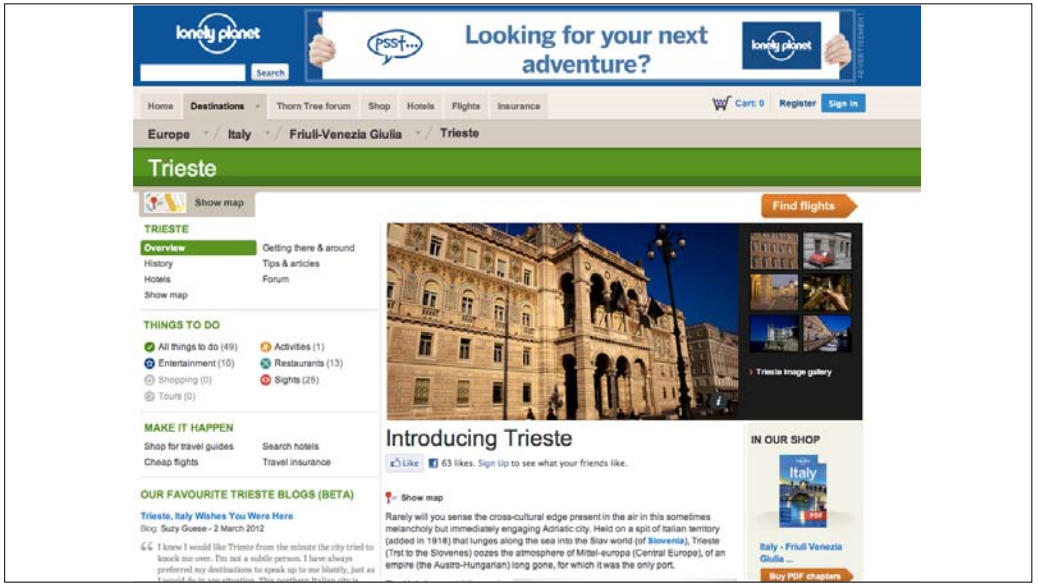
I LETTORI CONSIGLIANO



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OUR FAVOURITE TRIESTE BLOGS (BETA)

Trieste, Italy Wishes You Were Here
Blog: Suzy Guese - 2 March 2012

"I knew I would like Trieste from the minute the city tried to knock me over. I'm not a subtle person. I have always preferred my destinations to speak up to me bluntly, just as I would do in any situation. The northern Italian city is

Trieste image gallery

Introducing Trieste

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Rarely will you sense the cross-cultural edge present in the air in this sometimes melancholy but immediately engaging Adriatic city. Held on a spit of Italian territory (added in 1918) that lunges along the sea into the Slav world (of Slovenia), Trieste (Trst to the Slovenes) oozes the atmosphere of Mittel-europa (Central Europe), of an empire (the Austro-Hungarian) long gone, for which it was the only port.

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The SUNDAY POST

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Trieste is a treasure

DOPO LONELY PLANET

Anche il Sunday Post ammaliato da Trieste

L'inviata è rimasta rapita dai palazzi asburgici e della multiculturalità della città

Il Friuli Venezia Giulia incuriosisce e sorprende Katie Wood, giornalista del Sunday Post, settimanale scozzese edito da De Thomson che nella rubrica Travel & Homes ha pubblicato un articolo interamente dedicato alla città di Trieste, suggerendo ai propri lettori di spingersi fino ad Aquileia e Grado, per scoprire una regione inedita, lontana dagli itinerari turistici più scontati e al riparo dagli abituali prezzi «gonfiati».

La giornalista, affascinata dai maestosi palazzi asburgici e dalla multiculturalità che si riflettono in un'innata eleganza, descrive la città di Trieste (definendola un «tesoro») passando in rassegna anche le illustri visite di personaggi passati alla storia, come James Joyce, Sigmund Freud, Gustav Mahler, Rainer Maria Rilke e Franz Lehar. L'autrice scozzese stimola infine i suoi lettori a spingersi anche ad di fuori del capoluogo regionale, coinvolgendoli in una visita ai preziosi mosaici paleocristiani della Basilica di Aquileia fino alle calli in stile veneziano dell'Isola d'oro di Grado, sottolineando come il Friuli Venezia Giulia, ricco di patrimoni culturali, eccellenze enogastronomiche e spiagge incontaminate, regali un soggiorno inaspettato, all'insegna della scoperta di una «vera Italia» distante dagli itinerari del turismo di massa.

Si può ben dire che il mondo sta scoprendo Trieste e ne rimane affascinato. Dai giornali indiani alla guida australiana Lonely Planet, che ha citato la nostra città come una delle dieci meravigliose fuggonate sui classici itinerari turistici. Tutte attestazioni che fanno un gran piacere.

Ma emerge spontanea una considerazione. Ma se giornali e guide stranieri rimangono così basiti e ammaliati di fronte alle bellezze di Trieste significa che sotto il profilo turistico non è stata fatta un'adeguata promozione al di là delle solite trasferte infruttuose di delegazioni locali altrimenti tutti non rimarrebbero così stupiti. Invece che sopettare pigramente che gli altri vengano qui per renderesi conto delle risorse delle nostre terre, bisognerebbe cominciare a fare una seria promozione. E migliorare la ricettività, come hanno fatto negli ultimi anni gli alberghi.

Palazzi riflessi nel Canal grande (foto Ocean-Corbis)

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Corriere della Sera › Cronache › *Trieste, la bella «dimenticata»*

UNA CITTÀ UN PAESE

Trieste, la bella «dimenticata»

Crocevia di tre mondi, al confine tra Mediterraneo e Mitteleuropa, punta sulla scienza per tornare importante

CORRIERE TV

Siamo tutti in debito con Trieste. Conquistata al prezzo di 650 mila vite, perduta nel disastro della guerra e dell'esodo istriano, ripresa dopo 18 mesi di crimini nazisti, 40 giorni di massacri titini e nove anni di occupazione angloamericana, e poi dimenticata. Trieste che ha dato all'Italia Saba e Svevo, Strehler e Magris, Kezich e Dorfles, Trieste crocevia di tre mondi, latino tedesco slavo, Trieste la città più settentrionale del

Commenti dei lettori 44

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Camorra, arrestato Giuseppe Iovine

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Diaz, la Cassazione conferma le condanne per i vertici della polizia: scatta la sospensione

PIÙletti

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1 Dipendenti pubblici: addio posto e stipendio fisso, potrà andare mobilità obbligatoria

2 Tagli alle spese, via libera al decreto

3 Inchiesta sul volo Rio-Parigi: «Precipitato nell'Atlantico per errori tecnici e umani»

4 «Io rinchiusa in un manicomio criminale per un farmaco e per la

MERIDIANI

Trieste

64 MERIDIANI

ICGEB

Alcune immagini dei laboratori del Icgeb, "International Centre for genetic engineering and biotechnology", cioè Centro internazionale per l'ingegneria genetica e la biotecnologia. Attualmente vi operano circa 200 ricercatori di tredici diversi Paesi, soprattutto nel campo della biologia molecolare e cellulare. In basso, l'ingresso del centro, i cui laboratori occupano più di 10mila metri quadrati.

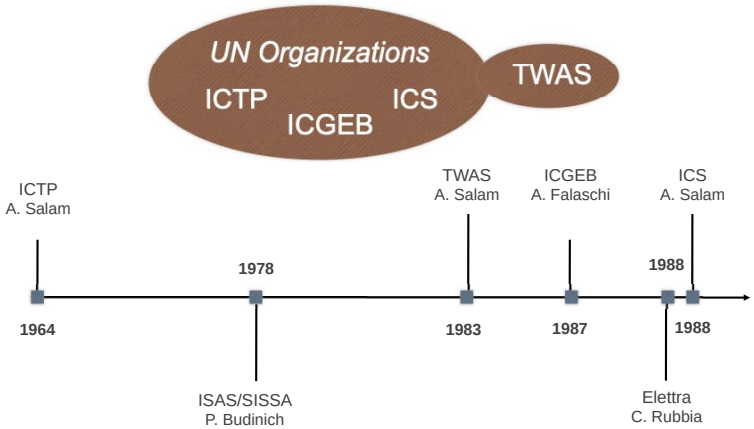
e preparate. La campagna acquisti di Budlisch convinca con l'inglese Dennis Sciama, uno dei padri della cosmologia moderna, e con il matematico russo Boris Dubrovin. Negli anni Novanta arrivano anche grandi nomi delle neuroscienze e neurobiologia, inaugurando un nuovo settore d'eccellenza. Lo dimostrano i finanziamenti europei erogati a sostegno di vari progetti: l'ultimo riguarda lo sviluppo di dispositivi in cui il tessuto nervoso si interfaccia con chip elettronici, per sostituire le funzioni di neuroni danneggiati per esempio da un ictus.

Nel 1983 è la volta della Twas. L'Accademia delle scienze per i Paesi in via di sviluppo, voluta per sollevare il ruolo di Trieste come nodo fondamentale in una rete di rapporti che doveva unire e stimolare i ricercatori di Stati meno avanzati. Grazie ai programmi e ai finanziamenti dell'Istituto, scienziati brasiliani, indiani, kenyoti, palestinesi e così via, possono oggi sostenere le proprie ricerche, per lo più nei settori della salute, dell'agricoltura e della protezione dell'ambiente, e anche incontrarsi e visitare altri centri di ricerca nel mondo. Il 1987, poi, vedrà la nascita del Centro internazionale per l'ingegneria genetica e le biotecnologie (Icgeb), una sede a Trieste, una a Nuova Delhi e, qualche anno più tardi, una anche a Cinà del Capo. Di nuovo formalizzo internazionale e ricerca, che nei laboratori triestini è soprattutto biomedica: qui, per esempio, è stato messo a punto un vaccino contro un particolare tipo di linfoni pronto per cominciare l'iter di sperimentazione clinica.

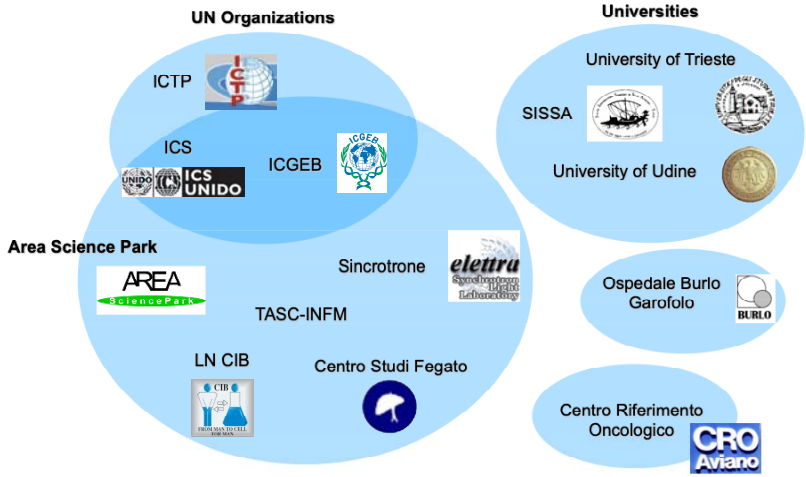
Poco distante dall'Icgeb sorge anche il sincrotrone Elettra, l'anello di luce. È una specie di pista circolare nella quale a correre sono elettroni velocissimi e ricchissimi di energia, che generano fasci di una luce particolare. Questa luce, detta appunto "di sincrotrone", serve a studiare a fondo la natura e le caratteristiche dei materiali. Qualsiasi. Dai superconduttori usati in elettronica ai virus, dalle proteine responsabili di malattie all'impasto del pane (per valutare gli elementi che gli conferiscono la consistenza giusta), fino ad arrivare al legno dei violini: di recente, la luce di Elettra ha scandagliato le profondità strutturali di un Guadagnini del Settecento, rivelando i piccoli interventi di restauro del passato e dando qualche suggerimento su come intervenire in futuro. A Elettra lavorano alcuni gruppi di ricerca fissi ma, di fatto, la struttura è un servizio per la comunità internazionale. «Due volte all'anno, ricercatori di tutto il mondo ci propongono i loro progetti e una commissione seleziona i migliori», spiega Carlo Rizzuto, presidente della società Sincrotrone Trieste.

Infine c'è Area, cioè il parco scientifico tecnologico sorto negli anni Ottanta nell'ex campo profughi di Padriciano.

History of the of the “Trieste System”



Scientific institutions in the FVG region contributing to the field of Molecular Medicine



ICGEB F1 Building, April 1988

26 · Giovedì 17 Maggio 2001 · Corriere della Sera · Italia/Friuli Venezia Giulia

Il capoluogo

17

CAPITALE DELLA SCIENZA

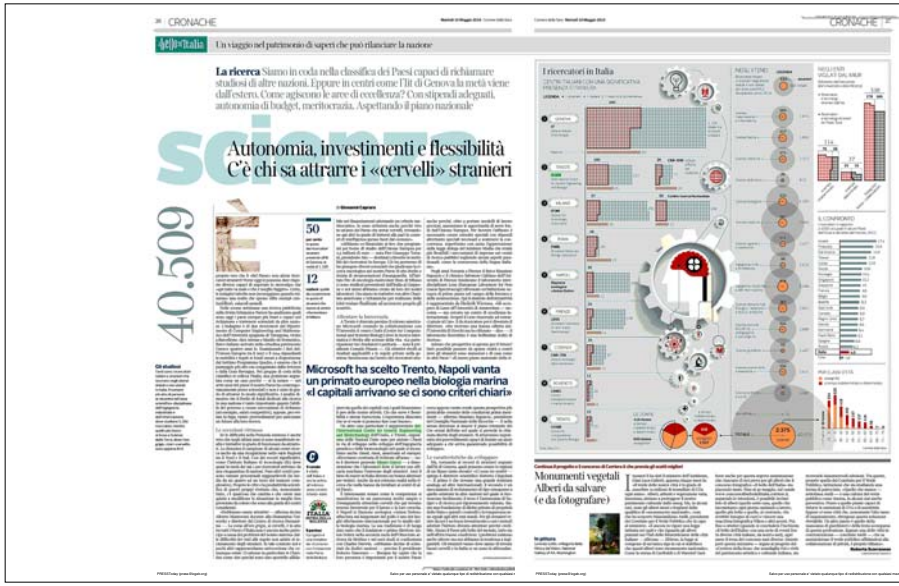
Trieste, mecca dei ricercatori del mondo

Migliaia di studiosi e Nobel nei centri «Tanto sapere deve essere riconosciuto»

di Giovanni Caprara

Settemila scienziati all'anno solo nel centro di Fisica teorica

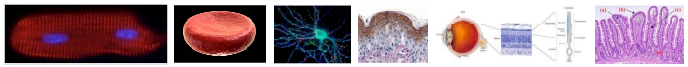
IL DIRETTORE



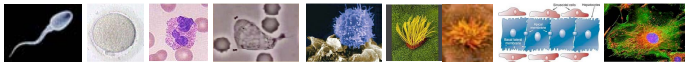
Microsoft ha scelto Trento, Napoli vanta un primato europeo nella biologia marina «I capitali arrivano se ci sono criteri chiari»

Monumenti vegetali Alberi da salvare (e da fotografare)

The body of *H. sapiens* is made up of cells



Cardiomyocyte | Red blood cell | Neuron | Keratinocytes | Eye photoreceptors | Gut epithelial cells

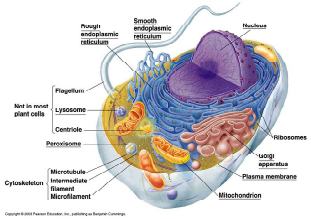


Spermatozoon | Oocyte | Granulocyte | Macrophage | T-lymphocyte | Respiratory tract ciliated cells | Hepatocytes | Endothelial cells

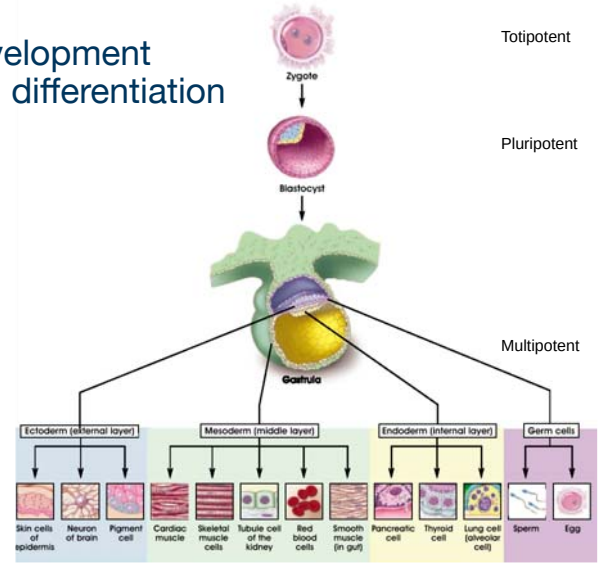
The human body:

~1x10^{14} (100 trillion) cells

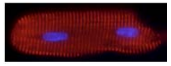
200+ different cell types



Development and differentiation



Cells' functions are exerted by proteins


Cardiomyocyte

Actin and myosin, contraction


Red blood cell

Hemoglobin, oxygen transport


Neuron

Receptors for neurotransmitters, movement and sensitivity


Keratinocytes

Keratin, skin barrier


Eye photoreceptors

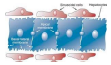
Opsin, light sensor


Granulocyte

Various enzymes, destruction of microorganisms


T-lymphocyte

Perforin, killing of virus-infected cells


Hepatocytes

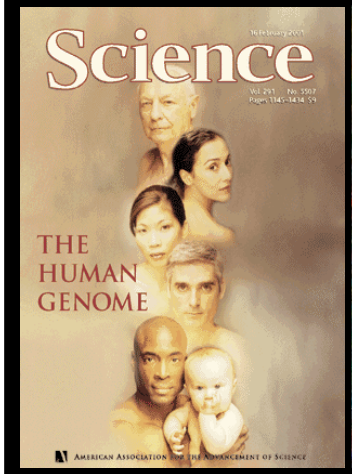
Various enzymes, metabolism

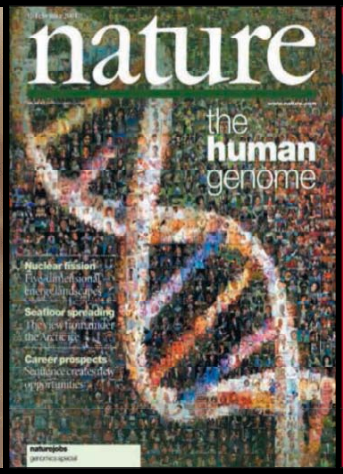

Endothelial cells

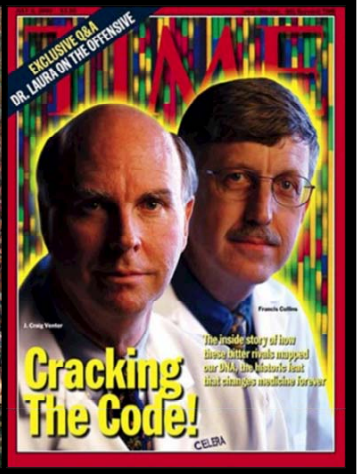
Junctional proteins, vessel impermeability

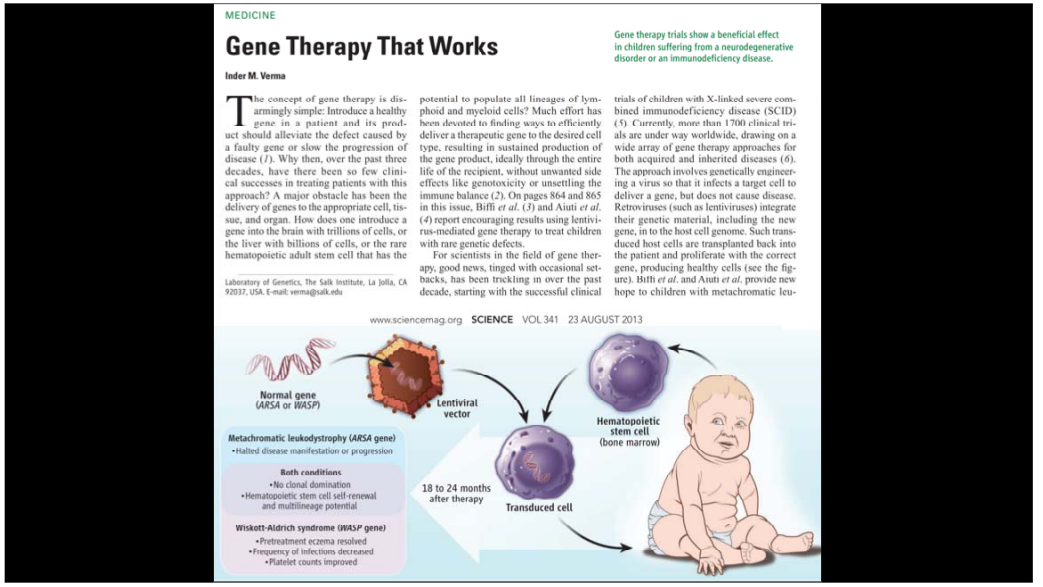
1952

2001

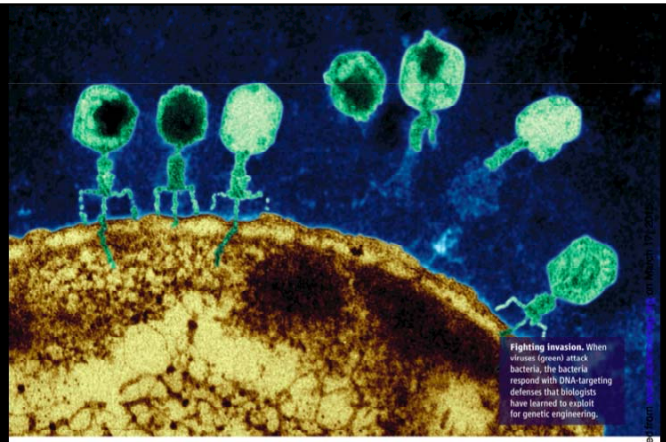








Genome Editing



The CRISPR Craze

A bacterial immune system yields a potentially revolutionary genome-editing technique

Biopharmaceutical Products

Product	Year
Insulin	1982
Human Growth Hormone (hGH)	1985
α-Interferon	1986
Hepatitis B Vaccine	1986
Tissue Plasminogen Activator (TPA)	1987
Erythropoietin-α	1989
γ-Interferon	1990
Granulocyte Colony Stimulating Factor (G-CSF)	1991
Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF)	1991
Interleukin 2	1992
Factor VIII	1992
β-Interferon	1993
DNase (Pulmozyme®)	1993
Glucocerebrosidase (Cerezyme®)	1994
ReoPro®	1994
… several others …	…

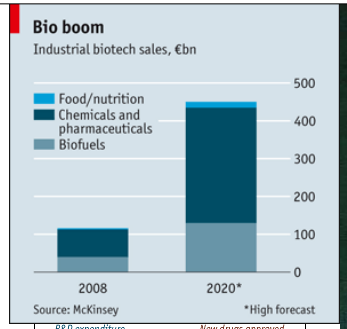
Source: Consulting Resources Corp.

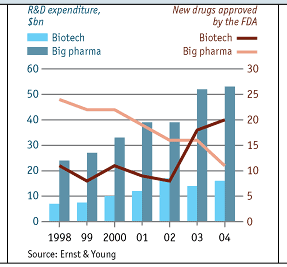
Cancer therapy using monoclonal antibodies

Table 1 US and EU therapeutic mAb approvals to date

Generic	Company/location	Trade	Description	Therapeutic category	Approval date
Muromonab-CD3	Johnson & Johnson New Brunswick, New Jersey	Orthoclone OKT3	Murine, IgG2a, anti-CD3	Immunological	06/19/86 (US)
Abciximab	Centocor	ReoPro	Chimeric, IgG1, anti-GPIIb/IIIa; Fab	Hemostasis	12/22/94 (US)
Rituximab	Genentech	Rituxan	Chimeric, IgG1κ, anti-CD20	Oncological	11/26/97 (US) 06/02/98 (EU)
Daclizumab	Hoffmann-La Roche Basel	Zenapax	Humanized, IgG1κ, anti-CD25	Immunological	12/10/97 (US) 02/26/99 (EU)
Basiliximab	Novartis Basel	Simulect	Chimeric, IgG1κ, anti-CD25	Immunological	05/12/98 (US) 10/09/98 (EU)
Palivizumab	MedImmune Gaithersburg, Maryland	Synagis	Humanized, IgG1κ, anti-respiratory syncytial virus	Anti-infective	06/19/98 (US) 08/13/99 (EU)
Infliximab	Centocor	Remicade	Chimeric, IgG1κ, anti-tumor necrosis factor (TNFα)	Immunological	08/24/98 (US) 08/13/99 (EU)
Trastuzumab	Genentech	Herceptin	Humanized, IgG1κ, anti-HER2	Oncological	09/25/98 (US) 08/28/00 (EU)
Gemtuzumab ozogamicin	Wyeth Madison, New Jersey	Mylotarg	Humanized, IgG4κ, anti-CD33, immunotoxin	Oncological	05/17/00 (US)
Alemtuzumab	Genzyme Cambridge, Massachusetts	Campath-1H	Humanized, IgG1κ, anti-CD52	Oncological	05/07/01 (US) 07/06/01 (EU)
Ibritumomab tiuxetan	Biogen Idec	Zevalin	Murine, IgG1κ, anti-CD20; radiolabeled (Yttrium 90)	Oncological	02/19/02 (US) 01/16/04 (EU)
Adalimumab	Abbott Deerfield Park, Illinois	Humira	Human, IgG1κ, anti-TNFα	Immunological	12/31/02 (US) 09/1/03 (EU)
Omalizumab	Genentech	Xolair	Humanized, IgG1κ, anti-IgE	Immunological	06/20/03 (US)
Tositumomab-I131	Corixa Seattle	Bexxar	Murine, IgG2aλ, anti-CD20; radiolabeled (Iodine 131)	Oncological	06/27/03 (US)
Efalizumab	Genentech	Raptiva	Humanized, IgG1κ, anti-CD11a	Immunological	10/27/03 (US) 09/20/04 (EU)
Cetuximab	Imclone Systems New York	Erbitux	Chimeric, IgG1κ, anti-Epidermal growth factor receptor	Oncological	02/12/04 (US) 06/29/04 (EU)
Bevacizumab	Genentech	Avastin	Humanized, IgG1, anti-vascular endothelial growth factor	Oncological	02/26/04 (US) 01/12/05 (EU)
Natalizumab[a]	Biogen Idec	Tysabri	Humanized, IgG4κ, anti-α4-integrin	Immunological	11/23/04 (US)

See **Box 1** for methodology.
[a]Voluntary suspension of natalizumab marketing announced February 28, 2005.





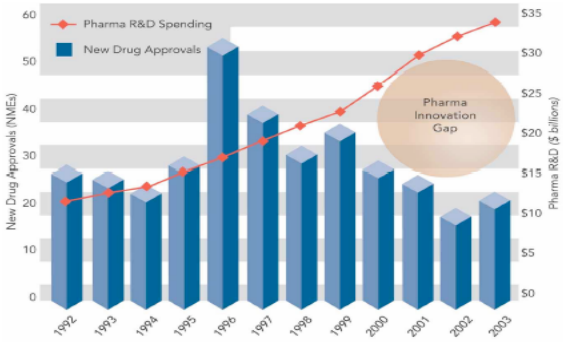
History of Biotechnology

- ✓ 1953 - double helical structure of DNA published in *Nature* by Watson and Crick*
- ✓ 1980 - the U.S. patent for cloning genes is awarded to Cohen and Boyer**
- ✓ First biotech companies formed:
  - 1976 - Genentech
  - 1978 - Biogen
  - 1980 - Amgen
  - 1981 - Immunex
  - 1981 - Chiron
  - 1981 - Genzyme

The Health Bioeconomy

- Over **350 biotechnological drugs** have now gained clinical approval and over 400 have entered clinical trials
- Biotechnology products are expected to account for **48% of the top 100 drugs** of 2016 (S&P)
- Monoclonal antibody market estimated to be worth no less than **75 billion** USD
- Global In Vitro Diagnostic (IVD) market forecast to reach **75 billion** by 2020
- **Biogenerics market** expanding rapidly for recombinant gene products and with enormous prospects for monoclonal antibodies

Innovation gap for traditional drugs



Burrill & Co

World's 10 bestselling prescription drugs made $75bn last year

Majority of bestsellers are created by biological processes rather than chemically synthesised and several are used as cancer medicines

Rank in 2013 (in 2012)	Product	Company	Therapeutic category	2013 sales ($US m)	2012 sales ($US m)
1 (1)	Humira	AbbVie	Other anti-rheumatics	10,659	9,616
2 (2)	Enbrel	Pfizer/Amgen	Other anti-rheumatics	8,776	8,496
3 (4)	Remicade	Johnson & Johnson/ Merck & Co	Other anti-rheumatics	8,386	7,990
4 (3)	Seretide/Advair	GlaxoSmithKline	Other bronchodilators	8,251	7,634
5 (6)	Lantus	Sanofi	Anti-diabetics	7,592	7,155
6 (5)	Rituxan	Roche	Anti-neoplastic MAbs	7,503	6,377
7 (9)	Avastin	Roche	Anti-neoplastic MAbs	6,751	6,282
8 (7)	Herceptin	Roche	Anti-neoplastic MAbs	6,562	6,253
9 (8)	Crestor	AstraZeneca	Anti-hyperlipidaemics	5,622	6,149
10 (10)	Abilify	Otsuka Holdings	Anti-psychotics	5,500	5,304

Humira (adalimumab) – Monoclonal antibody against TNFalpha

Enbrel (etanercept) – Fusion between the p75 TNFalpha receptor and an Ig

Remicade (infliximab) – Monoclonal antibody against TNFalpha

Seretide/Advair – Salmeterol and fluticasone

Lantus – insulin glargine

Rituxan (rituximab) – monoclonal antibody against B cell CD20

Avastin - monoclonal antibody against VEGF-A

Herceptin (trastuzumab) – monoclonal antibody against HER2/neu

Crestor (rosuvastatina) - statin

Abilify (aripiprazolo) – schizophrenia and bipolar disorders

EP vantage
Powered by Evaluate

Drug Development-Critical Path

- Observation
- Proof of concept
- Characterization
- Production
- Preclinical pharm/tox
- Formulation
- Manufacturing
- Clinical evaluation
- Regulatory approval
- Commercial development

Myocardial infarction

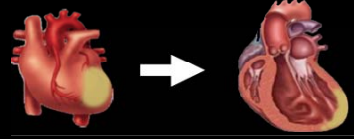
by Frank H. Netter

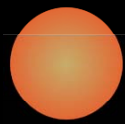
Loss of contractile tissue after acute myocardial infarction

Myocardial infarction

Repair by scarring

Dilatation and failure

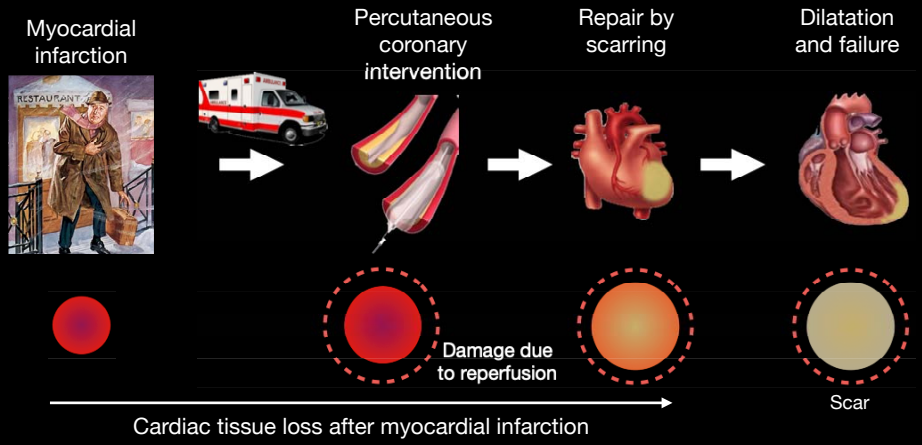




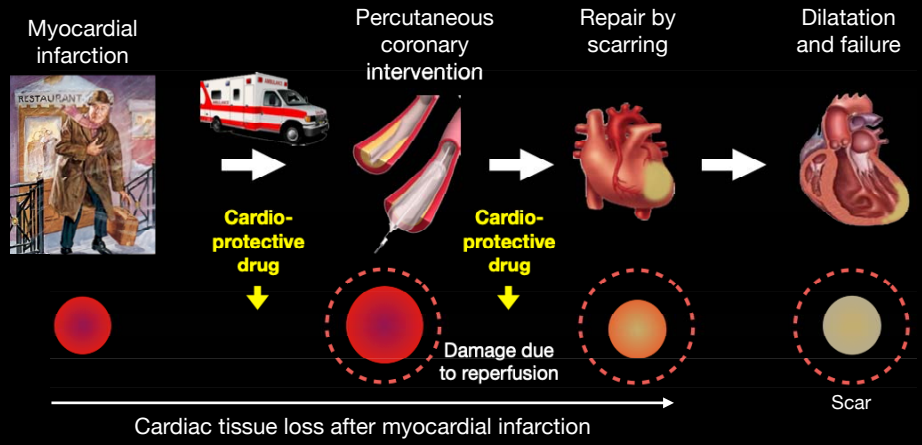
Scar

Cardiac tissue loss after myocardial infarction

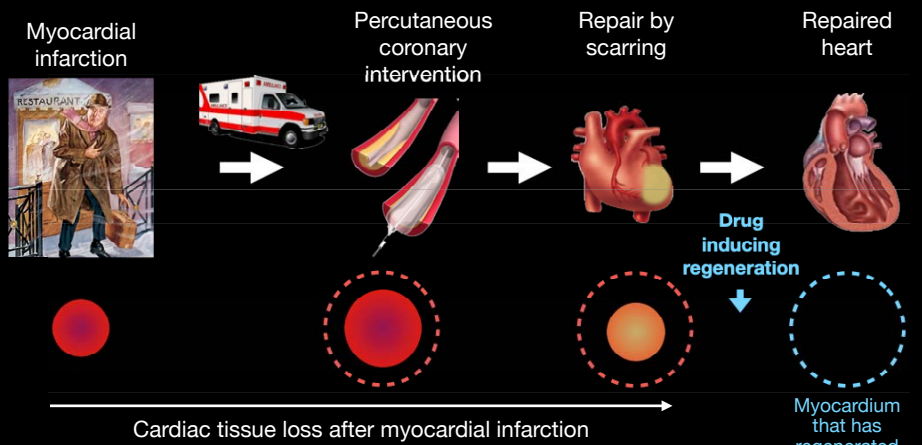
Loss of contractile tissue after acute myocardial infarction



Loss of contractile tissue after acute myocardial infarction



Loss of contractile tissue after acute myocardial infarction



Cardiology for dummies: prevention and therapy of heart failure

Protection from cardiomyocyte loss

No drug or treatment

Improvement of residual cardiac function

Digitalis
Diuretics
Aldosterone antagonists
ACE inhibitors
Beta blockers
Neprilysin inhibitors
ARBs
Ivabradine
Omecamtiv mecarbil
Dapagliflozin

Generation of new cardiomyocytes

No drug or treatment

Sorting the wheat from the chaff

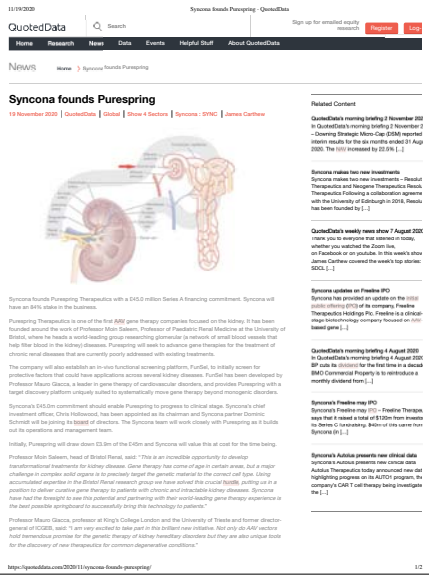
Only 1 drug of every 5,000 is commercialized (most drugs fail!)

It costs over $1M

And takes over 12 years

2/3 of all drugs that make it to the market do not recover R&D expenses

Who is going to pay?



11/19/2020 Syncona founds Purespring - QuotedData

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Syncona founds Purespring

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Syncona founds Purespring Therapeutics with a £45.0 million Series A financing commitment. Syncona will have an 84% stake in the business.

Purespring Therapeutics is one of the first *AAV* gene therapy companies focused on the kidney. It has been founded around the work of Professor Moin Saleem, Professor of Paediatric Renal Medicine at the University of Bristol, where he heads a world-leading group researching glomerular (a network of small blood vessels that help filter blood in the kidney) diseases. Purespring will seek to advance gene therapies for the treatment of chronic renal diseases that are currently poorly addressed with existing treatments.

The company will also establish an in-vivo functional screening platform, FunSel, to initially screen for protective factors that could have applications across several kidney diseases. FunSel has been developed by Professor Mauro Giacca, a leader in gene therapy of cardiovascular disorders, and provides Purespring with a target discovery platform uniquely suited to systematically move gene therapy beyond monogenic disorders.

Syncona's £45.0m commitment should enable Purespring to progress to clinical stage. Syncona's chief investment officer, Chris Hollowood, has been appointed as its chairman and Syncona partner Dominic Schmidt will be joining its board of directors. The Syncona team will work closely with Purespring as it builds out its operations and management team.

Initially, Purespring will draw down £3.9m of the £45m and Syncona will value this at cost for the time being.

Professor Moin Saleem, head of Bristol Renal, said: "*This is an incredible opportunity to develop transformational treatments for kidney disease. Gene therapy has come of age in certain areas, but a major challenge in complex solid organs is to precisely target the genetic material to the correct cell type. Using accumulated expertise in the Bristol Renal research group we have solved this crucial hurdle, putting us in a position to deliver curative gene therapy to patients with chronic and intractable kidney diseases. Syncona have had the foresight to see this potential and partnering with their world-leading gene therapy experience is the best possible springboard to successfully bring this technology to patients.*"

Professor Mauro Giacca, professor at King's College London and the University of Trieste and former director-general of ICGEB, said: "*I am very excited to take part in this brilliant new initiative. Not only do AAV vectors hold tremendous promise for the genetic therapy of kidney hereditary disorders but they are also unique tools for the discovery of new therapeutics for common degenerative conditions.*"

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Biotech Companies are Entrepreneurial

- ✓ Founded by an individual or perhaps a small group, usually scientists
- ✓ Technology obtained from tech transfer
- ✓ Angel or Venture capital backed
- ✓ High risk

Venture capital investment in CAR-T companies 2011–2016.

Company	Venture capital ($ in millions)	Date	CAR-T approach
Kite Pharma	15	Mar 2011	Autologous
Kite Pharma	20	May 2013	Autologous
Kite Pharma	50	Apr 2014	Autologous
Juno	176	Apr 2014	Autologous
Juno	134	Aug 2014	Autologous
Bellicum	55	Aug 2014	Autologous
Autolus	45	Jan 2015	Autologous
Poseida	23	Dec 2015	Allogeneic
CARsgen	30	Jan 2016	Autologous
Autolus	57	Mar 2016	Autologous
Total	605		

Includes only venture capital funding for companies involved in CAR-T program(s) at the time of investment. For example, venture capital funding of Bluebird Bio occurred prior to their CAR-T programs, while the company had only a gene therapy focus. These investments are not included.
Source: Company press releases.

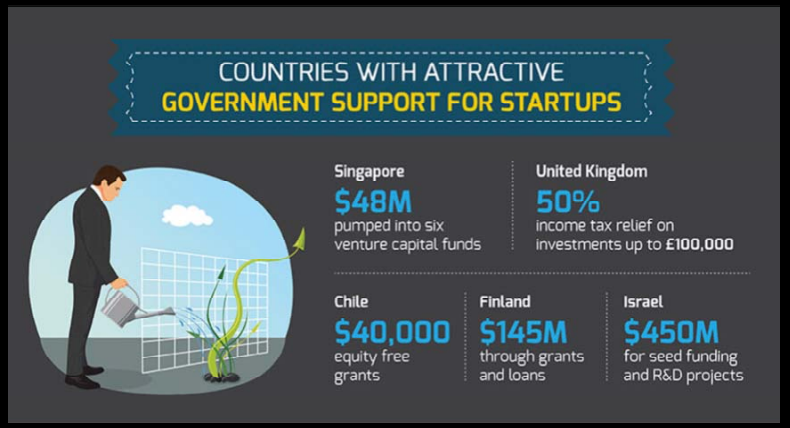
Cell & Gene Therapy Insights
Published: Oct 3 2016

Financing: a Critical Path

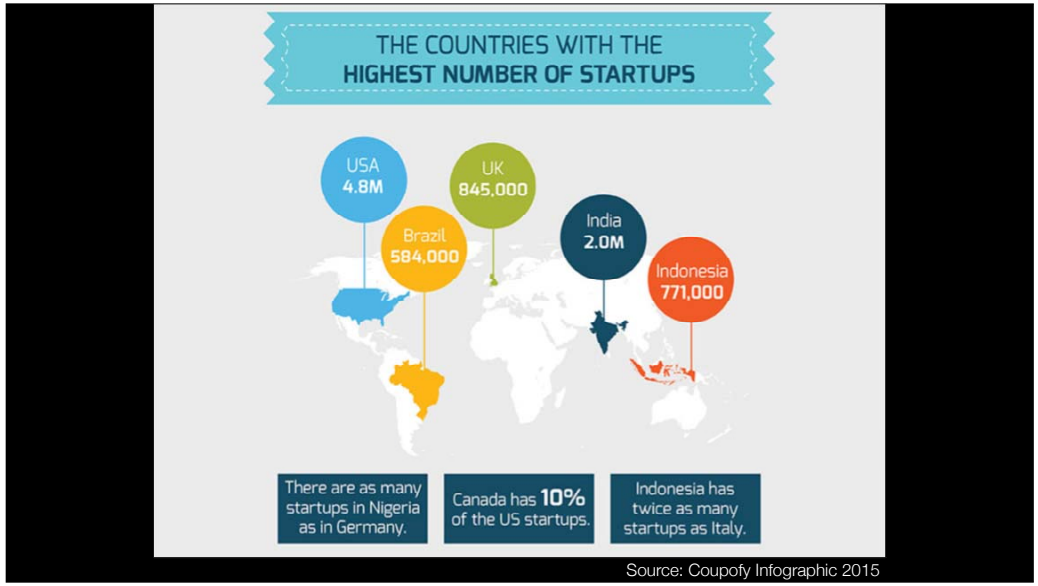
3 F's (friends, family, fools)
Public Grants
Angel investors
Venture capital
Partnering
Public offering (institutions)
Merger/acquisitions

Capital Financing Needs

Company Stage	Private investment
Proof of Concept	$25,000 – $100,000
Pre-seed	$50,000 – $500,000
Seed	$150,000 – $2 million
Early-stage	$1 million – $5 million
Expansion-stage	Up to $10 million



Source: Coupofy Infographic 2015



Basic science vs. intentional discovery

If I had asked my clients what they wanted, they would have asked for faster horses.

Henry Ford

Products and technologies discovered by enlightened serendipity

Penicillin
X-Rays
Genetic engineering
Magnetic resonance imaging
Lasers in surgery
Fax machine

An enabling environment for start-up development

- IP consultancy (freedom to operate)
- Patent office
- Business development
- Initial economic support to startups (*public support essential*)
- Bridge to Angels and VCs
- Bridge to large pharmacompanies