Lo sviluppo di terapie mediche innovative: i casi di Vivabiocell e Transactiva

Relatori: Marchetti Stefano / Sfiligoj Antonio

19/06/2017 ore 11.00

FP1640985001
#Sharing3FVG

Webinar realizzato da IALFVG e parte degli 80 di #Sharing3FVG, progetto cofinanziato dal Fondo Sociale Europeo nell'ambito del Programma Operativo Regionale 2014/2020

Impiego di piante trasformate nella produzione industriale di farmaci

Relatore: Stefano Marchetti

Lunedì 19 giugno 2017, ore 11

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Transactiva srl

Biotech SME

Academic spin-off of the University of Udine

Year of establishment	2001
Capital stock	10^5 €
Registered office	PST Udine
Employees	5
Active members	3

Facilities

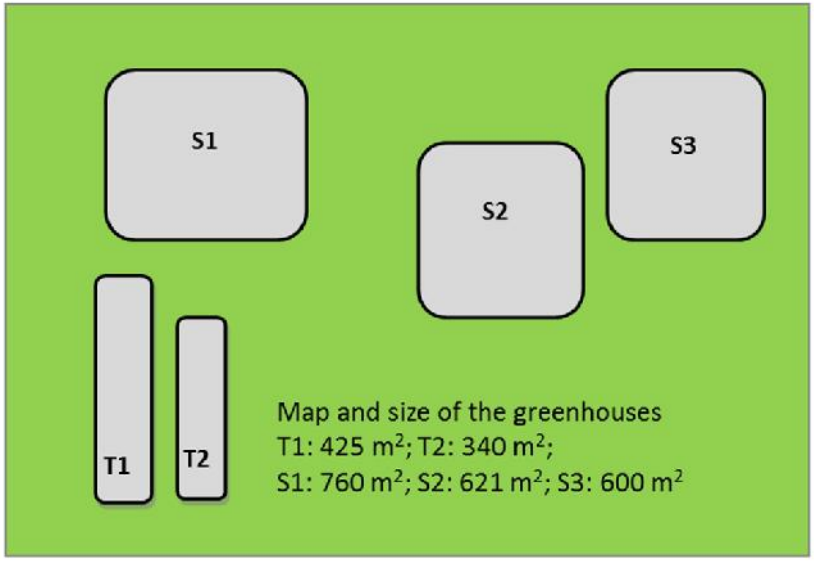
Molecular biology laboratory	150	sq. mt.
Biochemistry laboratory	100	sq. mt.
In vitro laboratory	60	sq. mt.
Growth chambers	50	sq. mt.
Greenhouses	2750	sq. mt.

Growth chamber for *in vitro* culture

Hydroponic culture systems

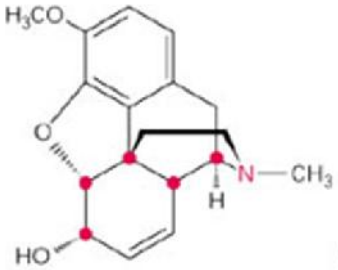
Greenhouses

Yield of paddy rice: 1.4 t/cycle

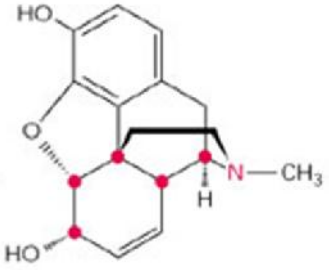


(A)

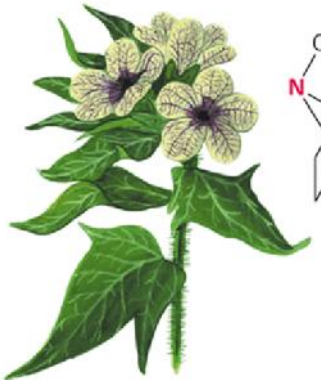
Papaver somniferum



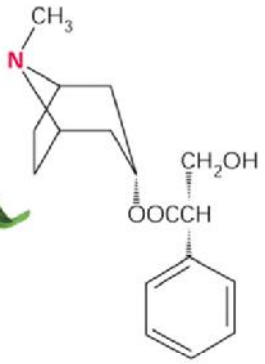
Codeine



Morphine



Hyoscyamus niger

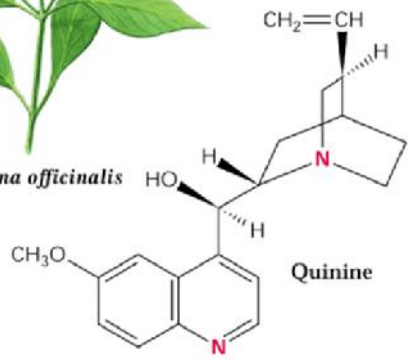


Atropine

Despite tremendous progress
in synthetic chemistry, still more than 28% of prescription drugs has an active ingredient of plant origin.

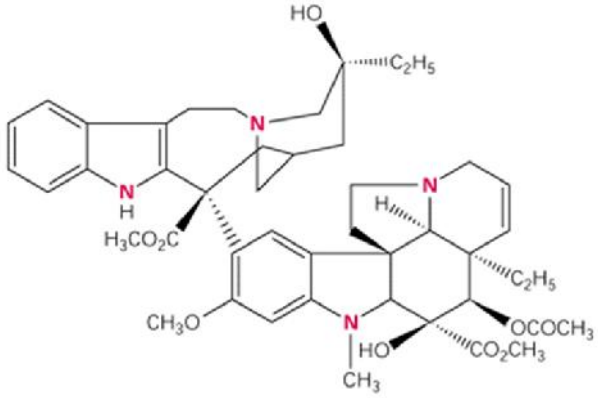
It has been estimated that only
3% of existing therapeutics in plants has been uncovered by man.

Cinchona officinalis



Quinine

Catharanthus roseus



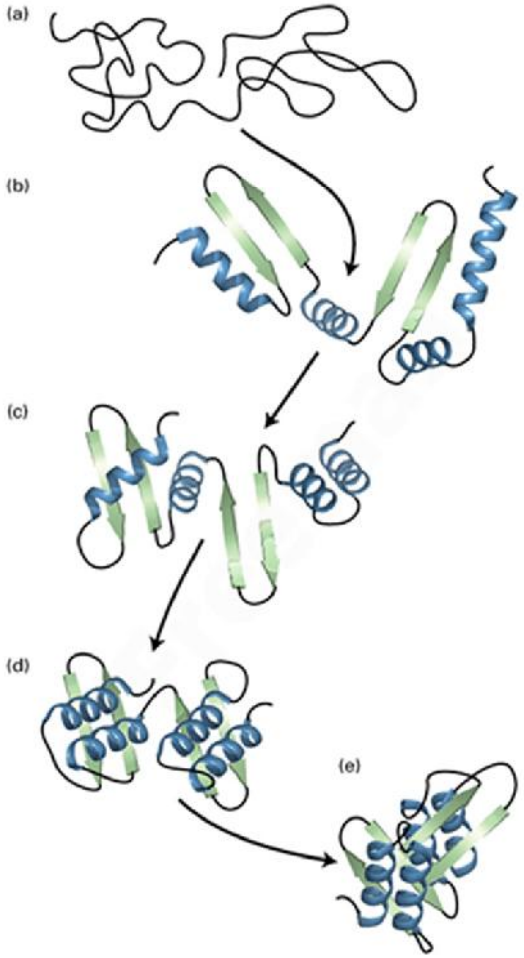
Vinblastine

transactiva

Molecular Farming

is the use of innovative expression systems for the production of *non-food, non-feed, non-fibre* commodities like therapeutic molecules, fuels, biodegradable plastics, industrial and commercial proteins.

R&D activities in Transactiva are primarily aimed at devising novel industrial platforms for the production of pharmaceuticals in plants.



Molecular Pharming

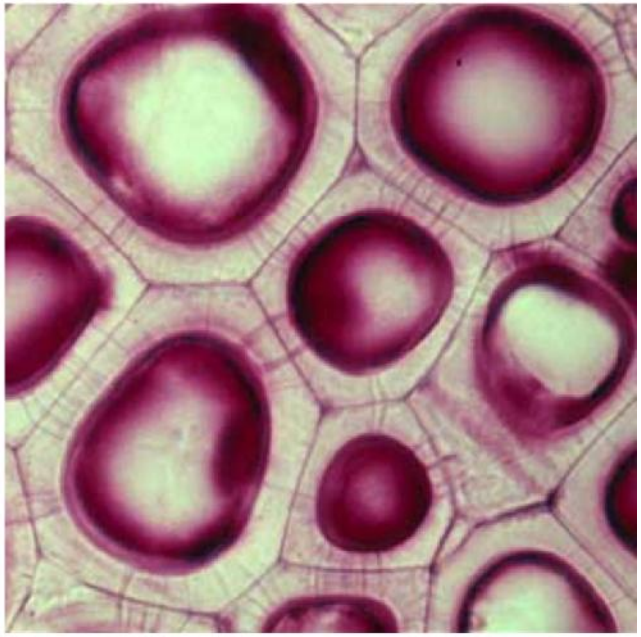
Transgenic plants as an industrial system for pharma production

The use of plants as green bioreactors has many advantages:

- high transformation efficiency as compared to other cell-based production methods;
- low capital investments and running costs compared to other platforms such as animal or plant cell cultures, bacteria, yeast or transgenic animals;
- rapid scale-up of production;
- remarkable safety (cancellation of the contamination risk by human pathogens such as viruses and micoplasma).

Transgenic plants as an industrial system for pharma production

Depending on the amounts requested, target molecules are synthesised in tobacco or rice; these species are engineered in compliance with human safety issues and environmental protection to accumulate the recombinant protein respectively in the leaf or in the seed. Both system are suitable for the production of pharmacologically active proteins with a high level of bioequivalence as compared to their native counterparts.



The tobacco-leaf system

It has the advantage of allowing rapid production of relatively small quantities of high-quality pharmaceutical proteins; this feature is desirable in the case of patient-specific medications, which must be tailored to the patient and produced in the shortest time possible (usually few months).
This method naturally adapts to the needs of traceability and containment of transgenic biomass, and allows easy modular expansion, also making it suitable for different production scales.

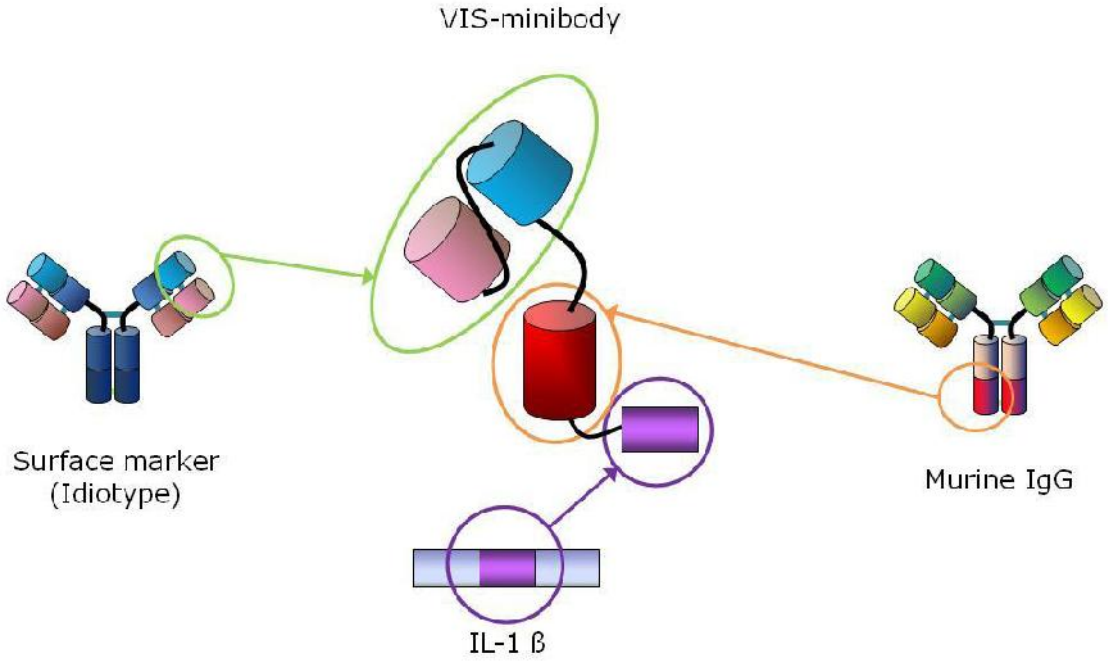
Start cup Udine

PREMIO NAZIONALE INNOVAZIONE

CUBE

Associazione Italiana degli Incubatori Universitari e delle Business Plan Competition

Anti-idiotype vaccines for the patient-specific therapy of NHL

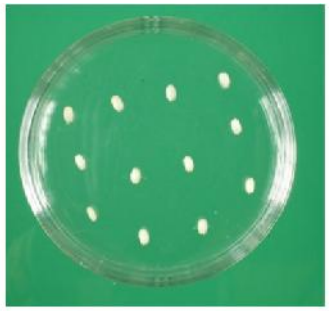


The rice-seed system

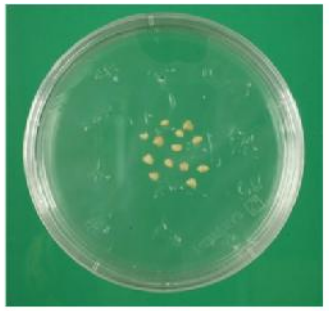
It allows the production and storage of large quantities of high quality recombinant proteins in a natural storage organ where the target protein is preserved by degradation for long periods of time. We gained the exclusive use of the CR W3 variety as the genetic background provider since 2007. Different expression vectors were developed to rule the synthesis and the site-specific accumulation of recombinant proteins in CR W3 endosperm.

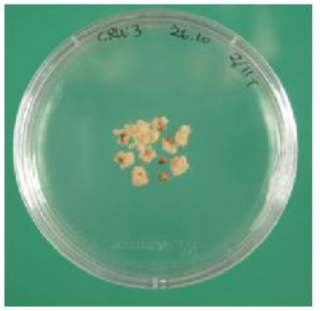
The expression system: rice transformation technique


dehulling

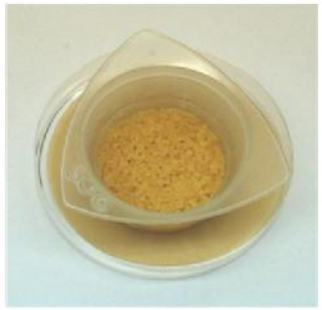

disinfection

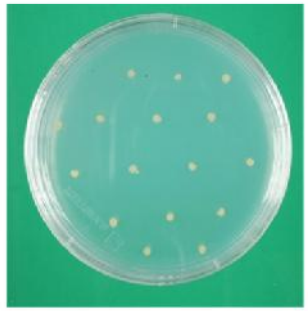

germination

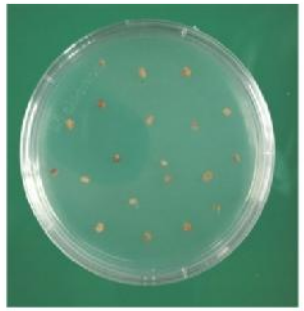

isolation of scutella

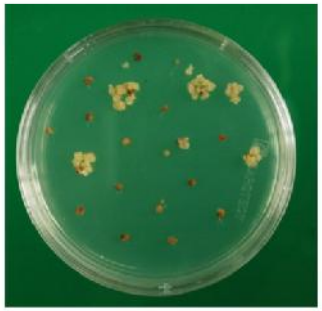

callogenesis

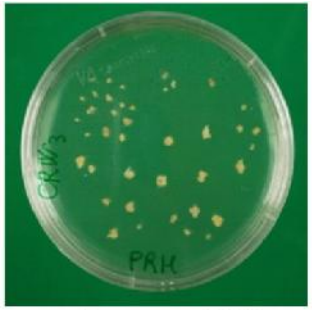

embryoids selection


infection

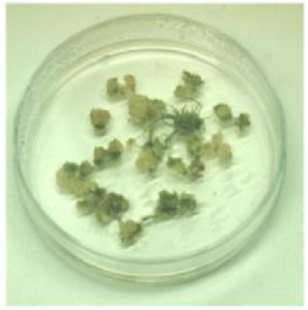

culture on CCM

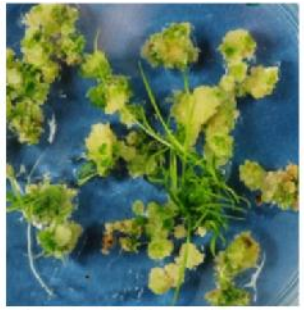

culture on SMI


culture on SMII



culture on PRM


regeneration


shoot differentiation


rooting


hardening and growth

Molecular farming: a touch of green

Gaucher disease

Treatment with ***rhGCase*** at a dose of 60 U/kg/month

60 U x 75 kg = 4.500 U/month x 12 = 54.000 U/year

Cost of 1 U = 5,48 €

Drug cost/patient/year = 296.055,00 €

rhGCase content in 1 kg polished GM rice = 600 mg

Following extraction and purification = 360 mg

1 mg rhGCase = 40 U

360 mg rhGCase = 14.400 U

3,75 kg of GM rice/patient/year

1 hectar of greenhouse = rhGCase amounts sufficient for 1.066 patients

transactiva
MOLECULAR FARMING

www.transactiva.com
info@transactiva.com

Storia Imprenditoriale di VivaBioCell

Relatore: Antonio Sfiligoj

19/06/2017 h. 11:00

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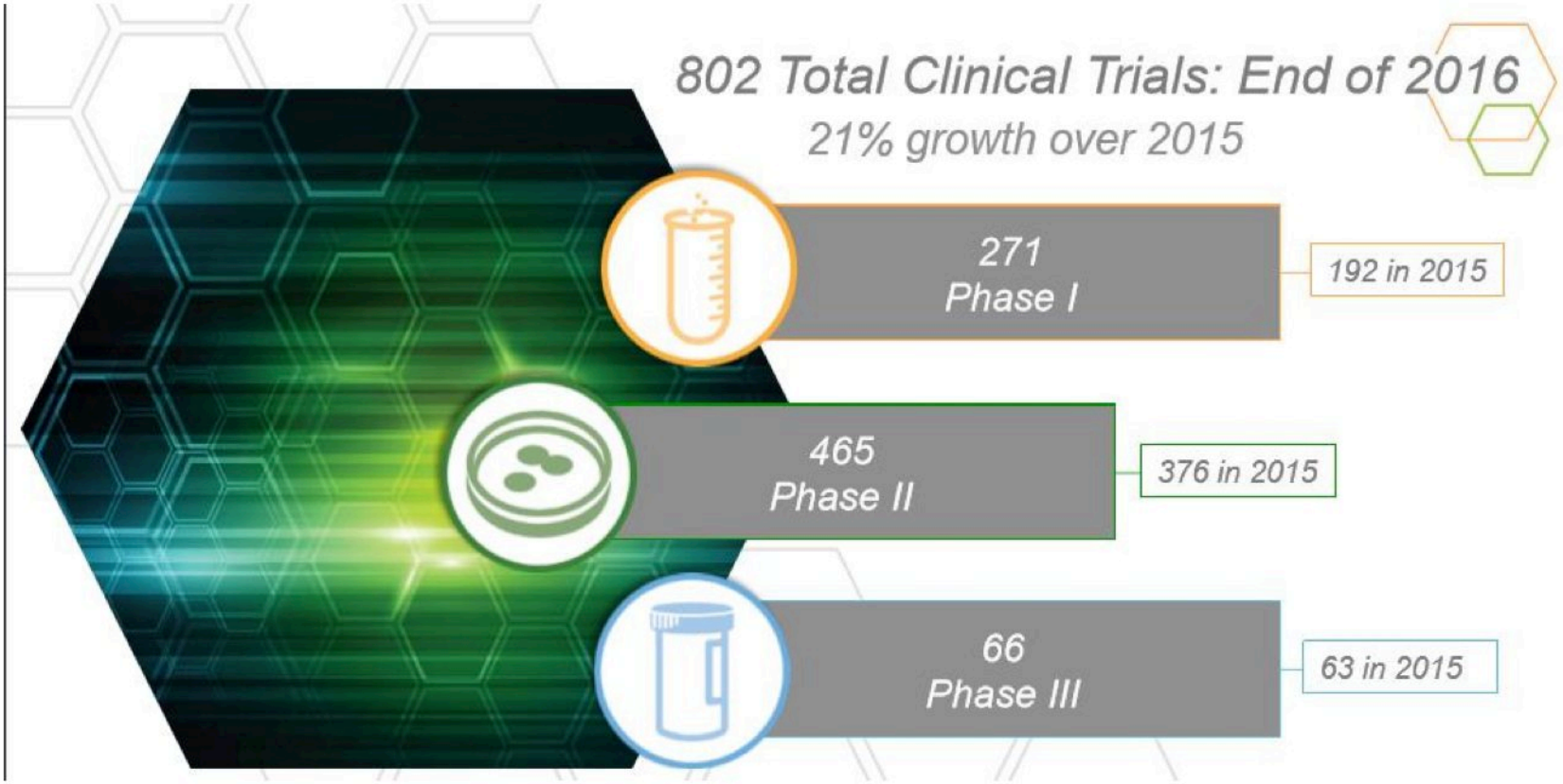
Medicina rigenerativa

- Def. il processo di rimpiazzamento e rigenerazione delle cellule, tessuti e organi umani per ripristinarne le normali funzioni.

- rigenerare tessuti e organi danneggiati nel corpo rimpiazzando il tessuto danneggiato e/o stimolando i meccanismi di riparazione del corpo per guarire i tessuti precedentemente irreparabili o organi.
- Uso di cellule staminali mesenchimali MSC Autologhe derivate da Midollo / Grasso / Cordone Ombelicale

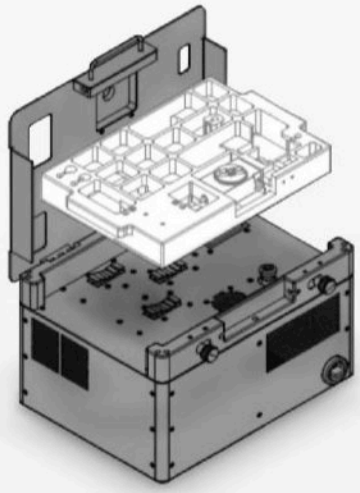
Medicina rigenerativa: Forte aumento di Studi Clinici



Mercato Importante e in crescita : 12 Miliardi USD crescita annua 28 %

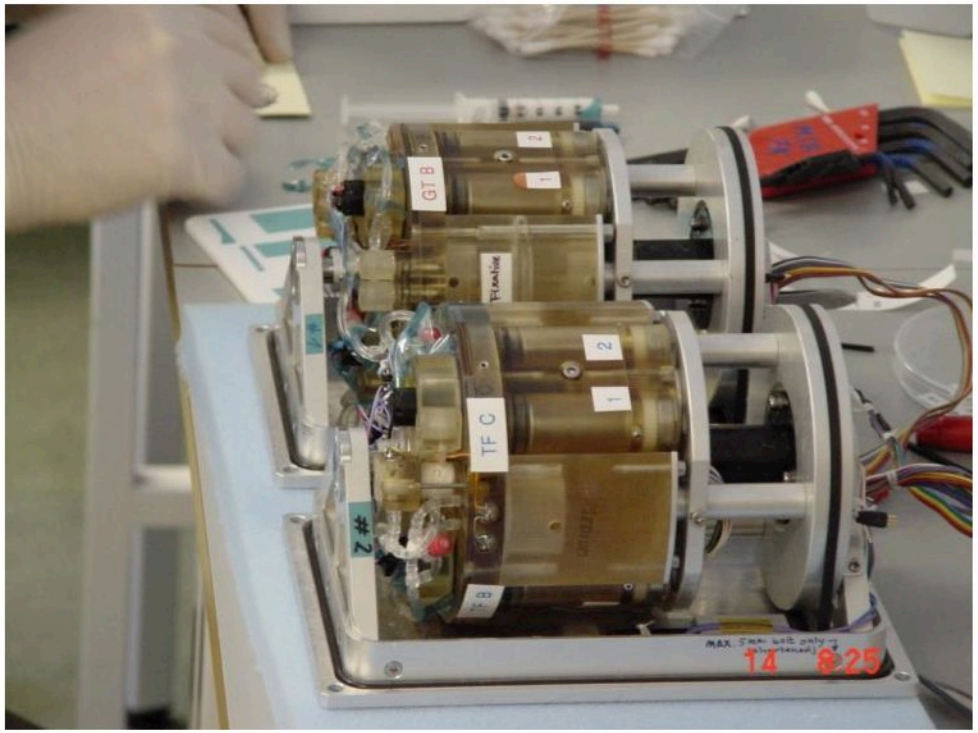
Barriera: Terapie Costose / Alti costi produzione farmaci cellulari

Idea VivaBioCell : Automatizzare il processo produttivo, assicurandone sterilità /qualità per ridurre i costi e migliorare la sicurezza e standardizzazione



GMP in a Box

Origine dell'Idea: Partecipazione a progetti spaziali ASI



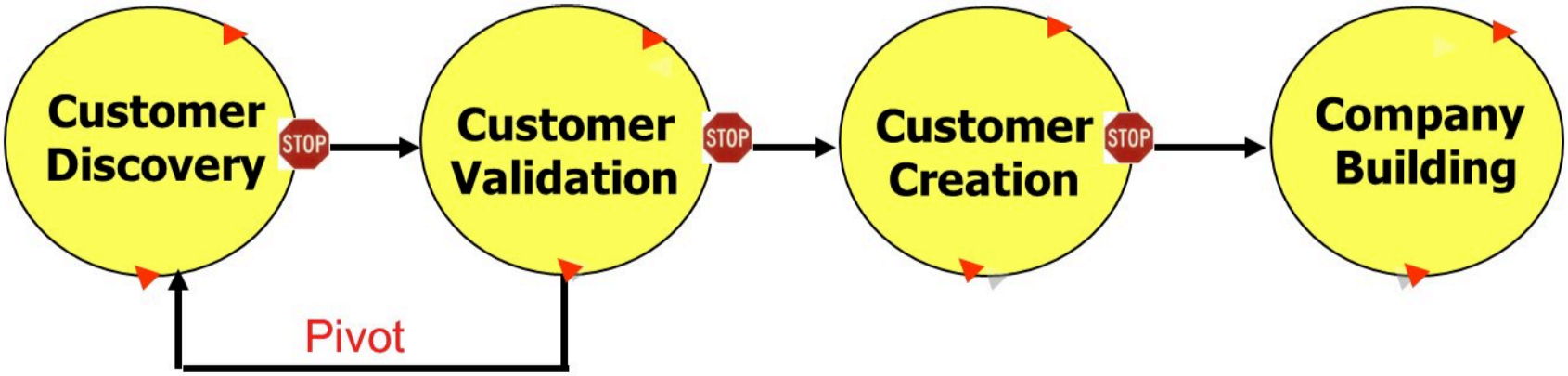
MASER and TEXUS microgravity ESA programs (2002-2008)

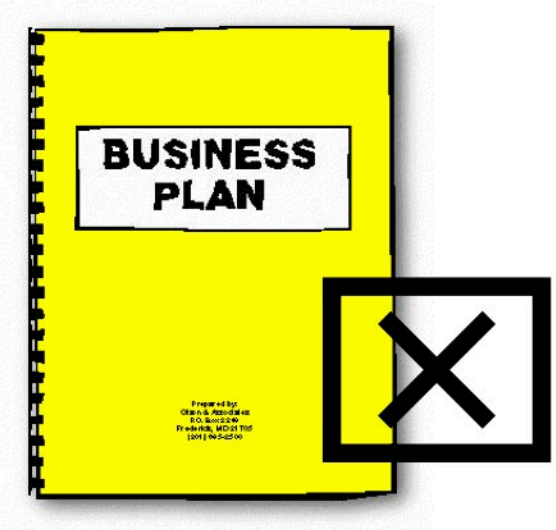
esa

Principali Tappe dello Sviluppo Aziendale

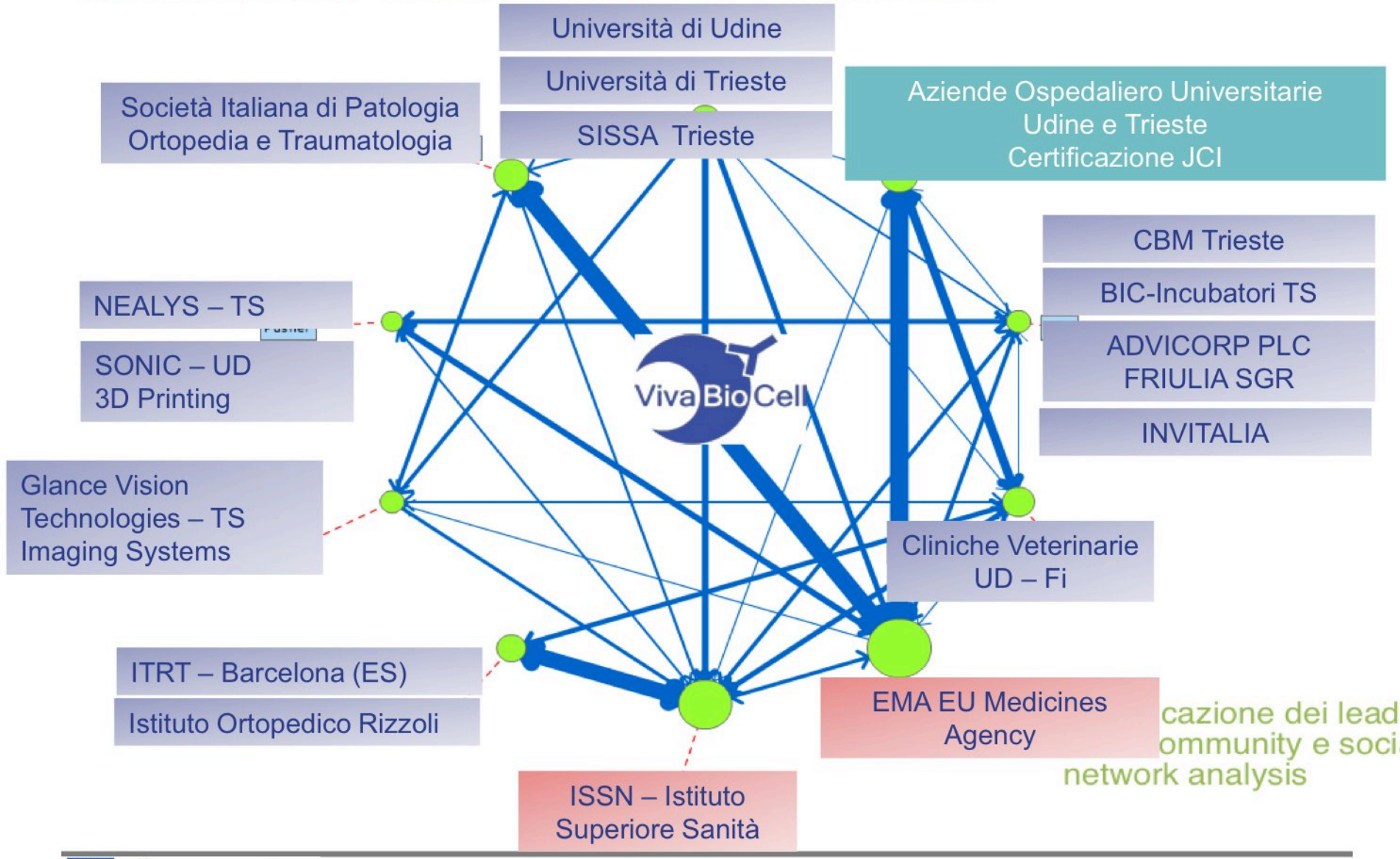
- 2005 - Start-cup nazionale; (Start-Cup Università di Udine)
- 2007 - Costituzione della società a partire da TOR , spin-off UniUd e veicolo dei Fondatori. Soci : Friulia SGR (Fondo Alladin Ventures), Allegro SrL , Banca Popolare di Cividale e ZIP;
- 2012 – inizia la ricerca di investitori internazionali. Investment Forum: Primo premio all'ESA di Tolouse (spin-off di progetti di Ricerca Spaziale) e Healthcare venture contest, Aarhus (DK) (imprese innovative del settore biotech);
- 2015 – Acquisizione da parte di VBC Holdings LLC /Nantworks gruppo guidato da Patrick Soon-Shiong, impegnato nello sviluppo di terapie avanzate.

Dal Business Plan al Processo di Sviluppo dei Clienti

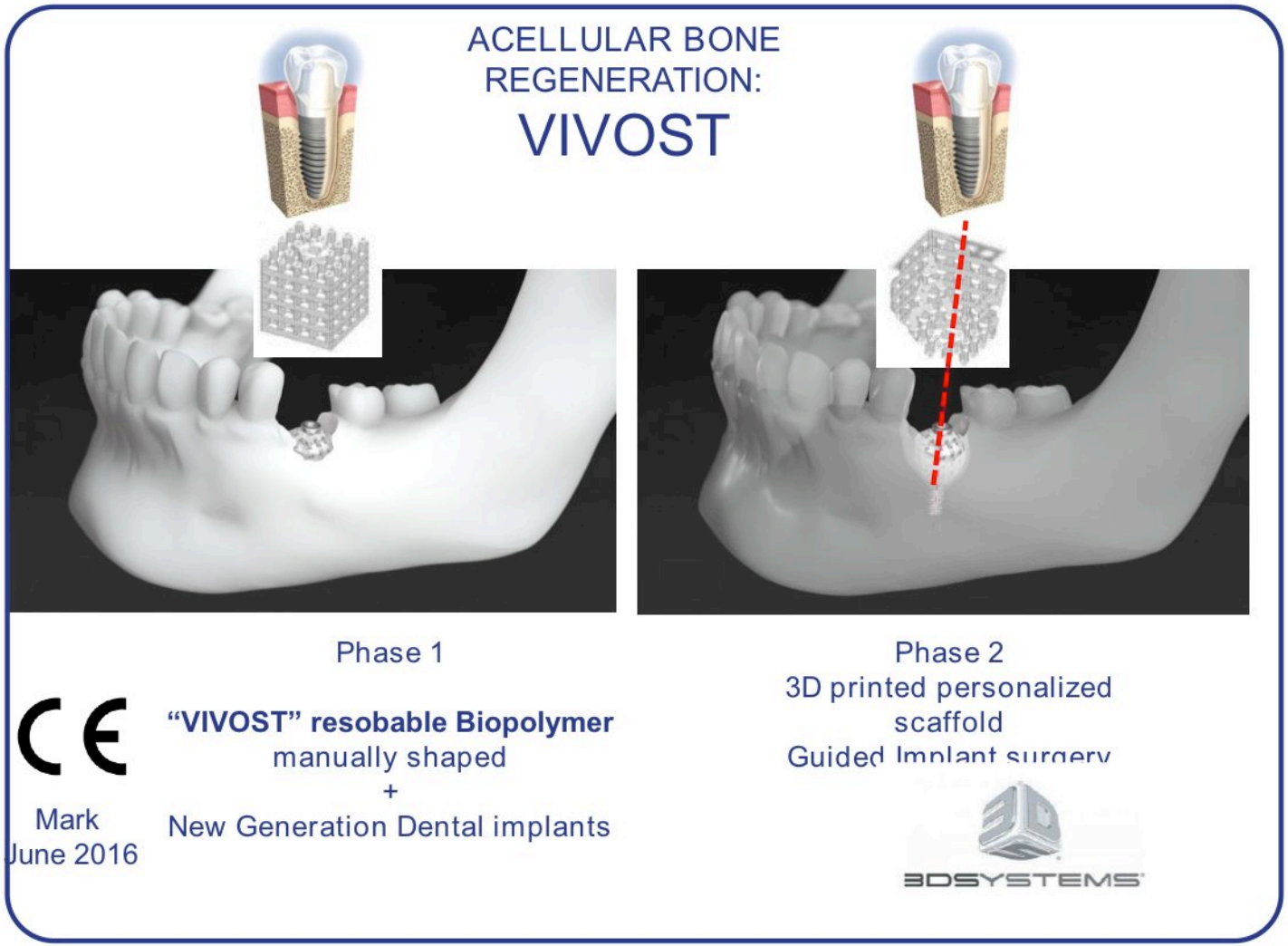




L'Ecosistema di riferimento di VivaBioCell



Primo Prodotto : Scaffold VIVOST



Secondo Prodotto: Terapia Rigenerativa Osteoartrosi Cane

PAWER Mai più artrosi

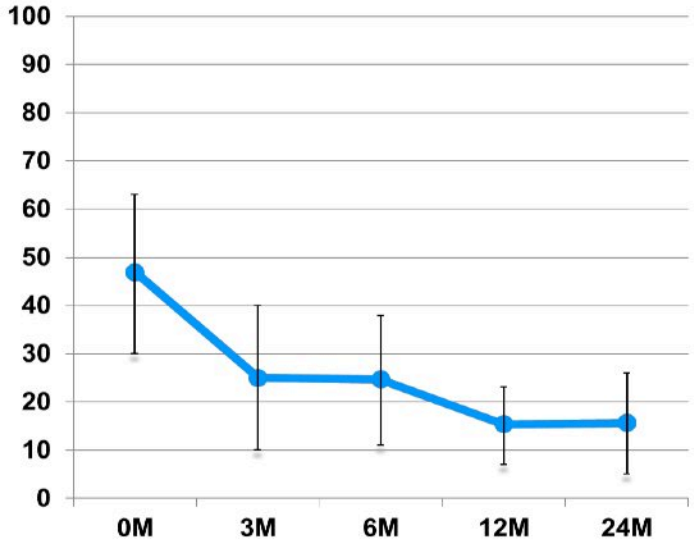
VIDEO

(Scaricabile tra i materiali della scheda)

Risultati nell'Uomo

ITRT
Institut de Teràpia Regenerativa Tissular

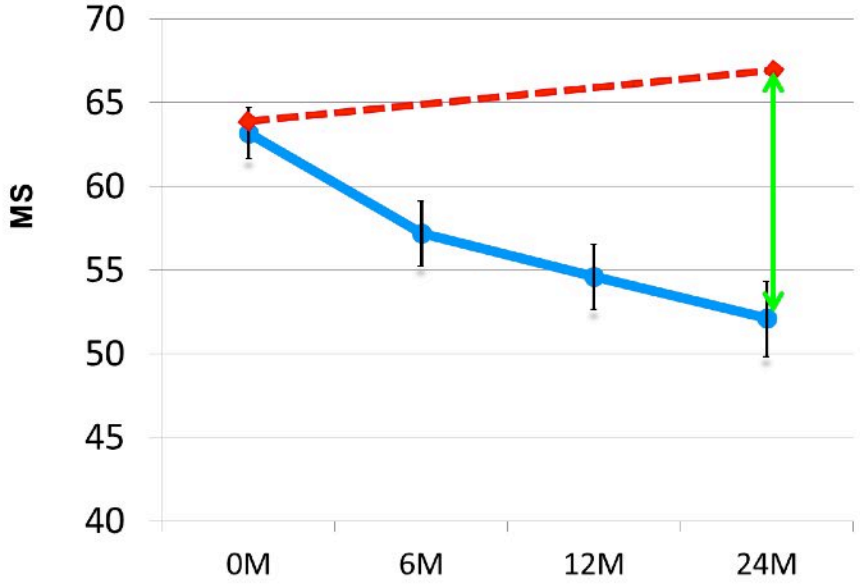
Daily VAS n=12



T2 MAPPING

50-99 ms

CI 95%



Approccio Agile Prototyping and Engineering

- Agile Development
- Continuous Learning
- Self Organizing Teams
- MVP – Minimum Viable Product
- Regulatory Approvals / Pivots

Terzo Prodotto: Bioreattore Automatico

Conclusione: un lungo percorso...un nuovo inizio !