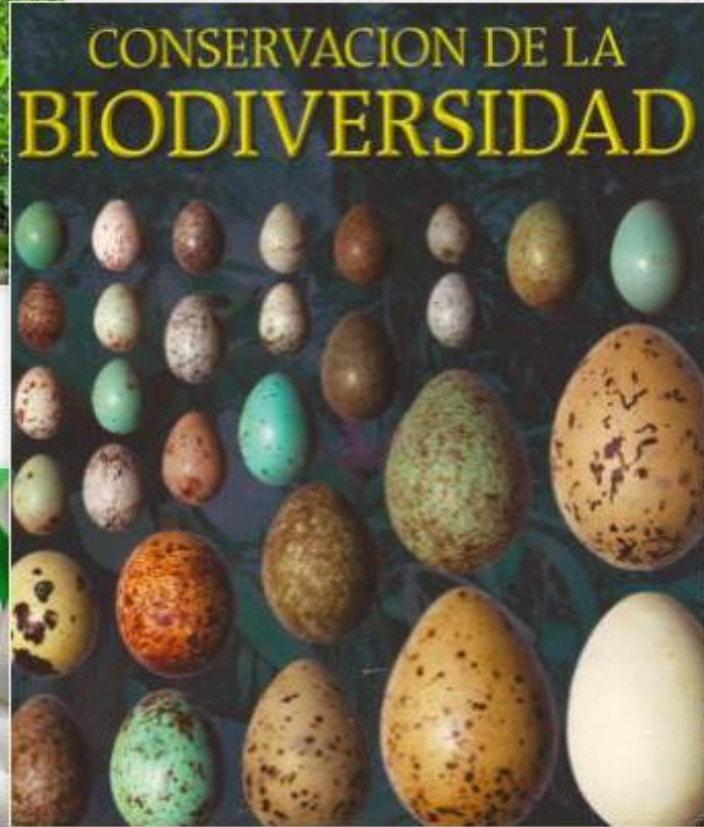


# EXPLORING BIODIVERSITY FOR HEALTH







## Exploración de la Biodiversidad Obtención de medicamentos

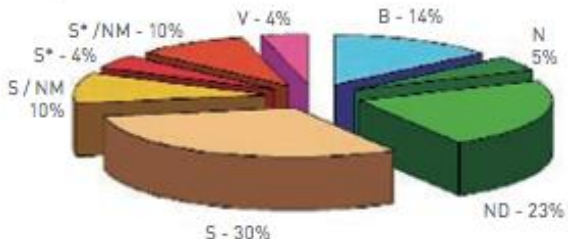


Los productos naturales han sido consistentemente la fuente más productiva de medicamentos

### Entre 1983 y 1997

- el 43% de los antibióticos y antitumorales
- el 39% de todos los medicamentos aprobados fueron derivados de productos naturales.

All new chemical entities, 01/1981-06/2006, by source (N) 1184).



- B ..... Biological
- N ..... Natural product
- ND ..... Derived from a natural product and is usually a semisynthetic modification
- S ..... Totally synthetic drug, often found by random screening, modification of an existing agent
- S\* ..... Made by total synthesis, but the pharmacophore is/was from a natural product
- V ..... Vaccine
- NM ..... Natural product mimic

# EXPLORING BIODIVERSITY FOR HEALTH



## NORTE

Países Ricos en Tecnología

2500 medicamentos derivados de la biodiversidad (100 años)

Medicamentos de la biodiversidad  
**PARADIGMA NORTE/SUR**

## SUR

Países Ricos en Biodiversidad

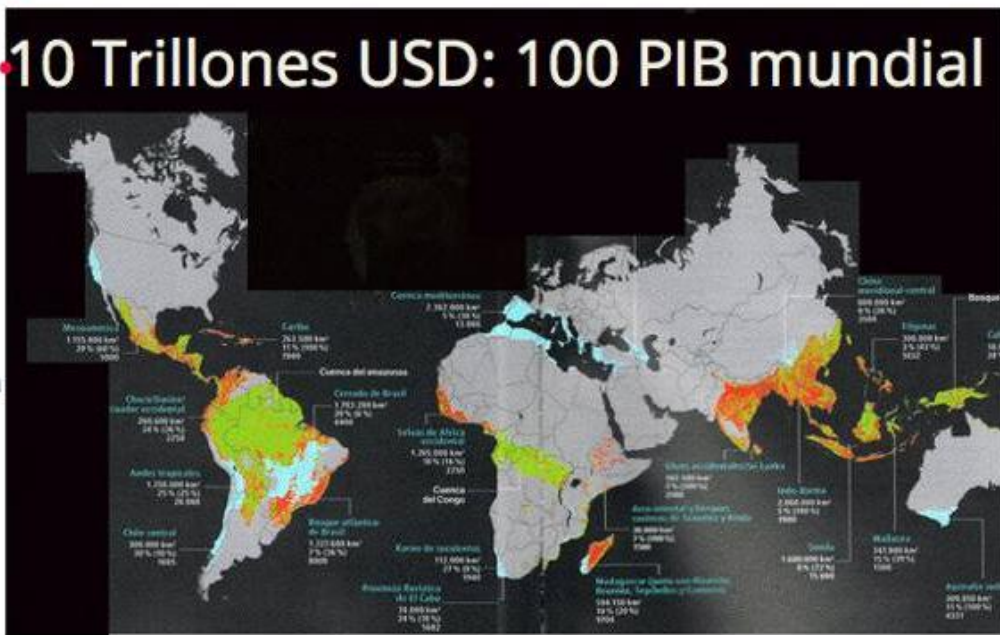
Ningún medicamento



**10 Trillones USD: 100 PIB mundial**

## PRIMER NEGOCIO MUNDIAL

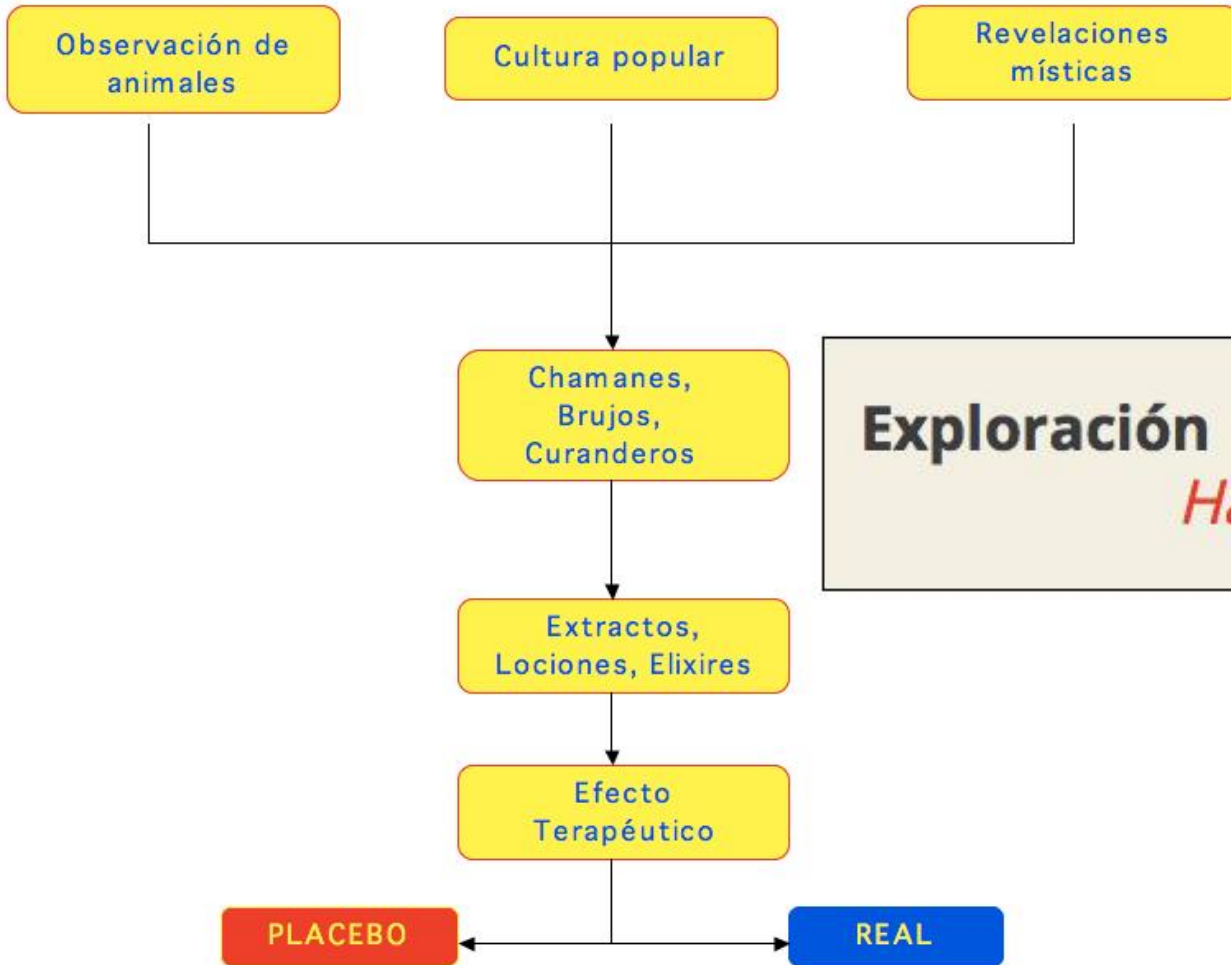
- 26 empresas top 100
- 15 USy 11 EU
- NINGUNA DEL SUR
- 3,5 Trillones de USD capitalización
- 15 millones de puestos de trabajo





**Exploración de la Biodiversidad**  
*Tablillas de Nippur 4.000 a.C.*

# EXPLORING BIODIVERSITY FOR HEALTH



**Exploración de la Biodiversidad**  
*Hasta 1800*

# EXPLORING BIODIVERSITY FOR HEALTH



Cultura popular

Casualidad

Exploración de la Biodiversidad  
*Hasta 1980*

Laboratorios  
Farmacéuticos

Extractos Plantas

Principios Activos

Síntesis química

Me-too

Efecto Terapéutico

Principios Activos  
Sintéticos

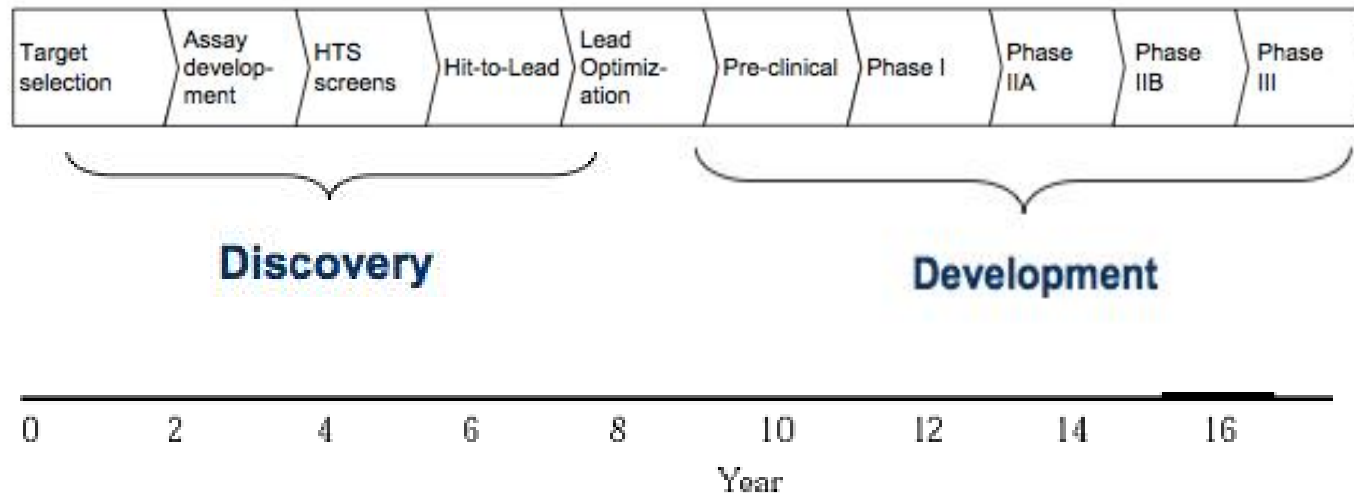
Experimentos  
animales in vivo

REAL



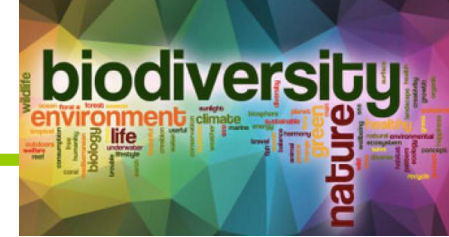
## Drug Discovery Desarrollo de nuevos medicamentos

- Muy lento (15 años)
- Muy complicado (Fases)
- Muy costoso (\$ 100 M)
- Muchos pacientes (1000)





# EXPLORING BIODIVERSITY FOR HEALTH



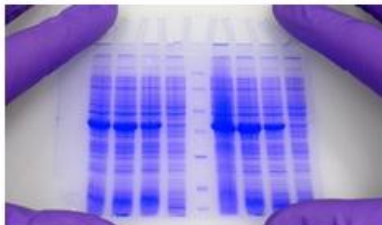
## REVOLUCION GENOMICA

OGM  
Proteínas recombinantes  
Recursos Genéticos

Exploración de la Biodiversidad  
*Revolución Biotecnológica*

## REVOLUCION HTS

Screening masivo farmacológico





## 2010



### Exploring Biodiversity in the Philippines

By RICH MOOI MAY 25, 2011 5:52 PM



**NIH Small Molecule Repository**  
Welcome to the NIH SMR Online Store

Powered by **evotec**

HOME SETS

**Welcome!**  
NIH SMR is offering compound subsets for screening by qualified applicants. [Learn More](#)

[Browse Sets Now](#)

New to NIH SMR? [Register Here](#) | Already Registered? [Sign In](#)


**NIH SMR Externalization Program**

The Molecular Libraries Program (MLPCN), funded by the NIH National Center for Advancing Translational Sciences (NCATS), offers public sector biomedical researchers access to the large-scale screening capacity necessary to identify small molecules that can be optimized as chemical

[Site Overview](#)  
[MLPCN](#)  
[NIH SMR](#)  
[Book Shelf](#)


# EXPLORING BIODIVERSITY FOR HEALTH





Fogarty International Center  
Advancing Science for Global Health

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- ▶ eCapacity
- ▶ Ecology and Evolution of Infectious Diseases (EEID)
- ▶ Emerging Global Leader
- ▶ Emerging Epidemic Virus Research Training (Guinea, Liberia, Sierra Leone)
- ▶ Environmental and Occup Health (ITREOH)
- ▶ Framework Innovations
- ▶ Fulbright-Fogarty Fellows and Scholars
- ▶ GEOHealth
- ▶ Global Health Fellows and Scholars

## International Cooperative Biodiversity Groups (ICBG)

**Status:** Closed

### Announcement(s)


- Expired announcement: Limited Competition: International Cooperative Biodiversity Groups (U19) (RFA-TW-13-001)

### Eligibility

- Individuals with expertise in any area relevant to the RFA from U.S. institutions of higher education or other U.S. nonprofit institutions are invited to apply.
- Interdisciplinary teams should be assembled and must include collaborators from institutions in biodiverse LMICs. See the RFA for more information on these partnerships.

### Program Overview

NIH and the National Science Foundation (NSF) support to **International Cooperative Biodiversity Groups (ICBG)** program to address the interdependence of biodiversity exploration for potential applications in health, with investments in research capacity that support sustainable use of these resources, the knowledge to conserve them, and equitable partnership frameworks among research organizations in the U.S. and low- and middle-income-countries (LMICs). LMICs are defined as low or middle income countries in the World Bank list of economies.



**Biodiversity project receives Nagoya funding**

An NIH-supported biodiversity project in Panama will receive \$1 million as the first award under the Nagoya Protocol Implementation Fund

[Learn More](#)

### Related News

- Grantee news: Foes can become friends on the coral reef
- Georgia Tech news,

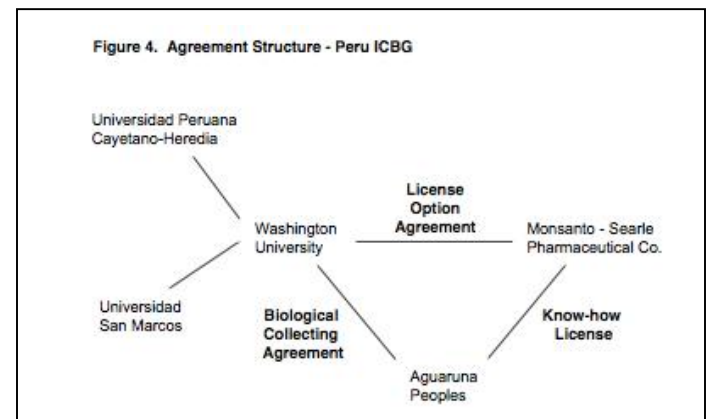
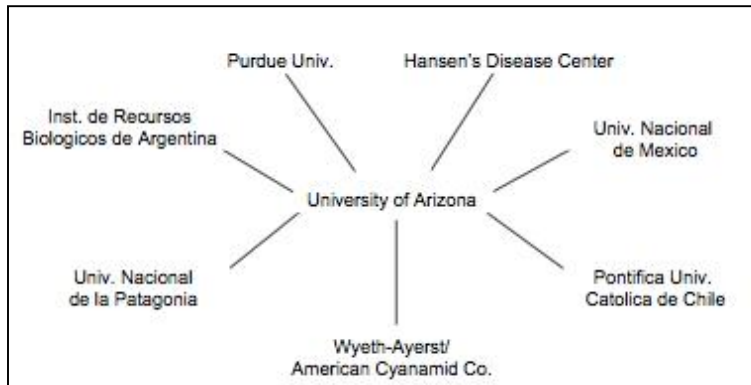
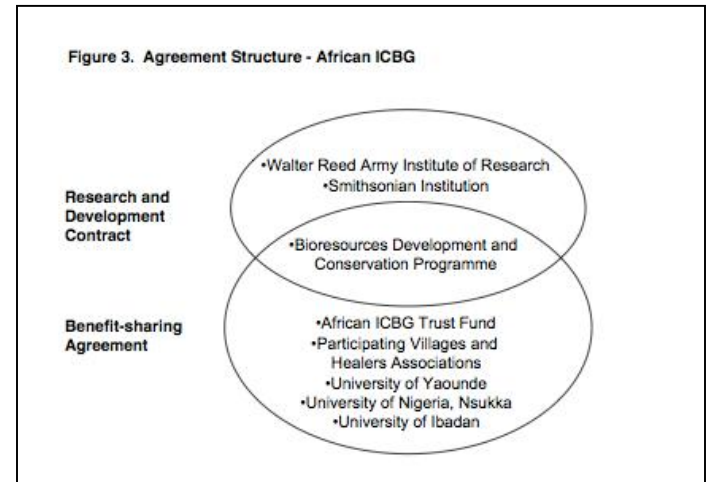
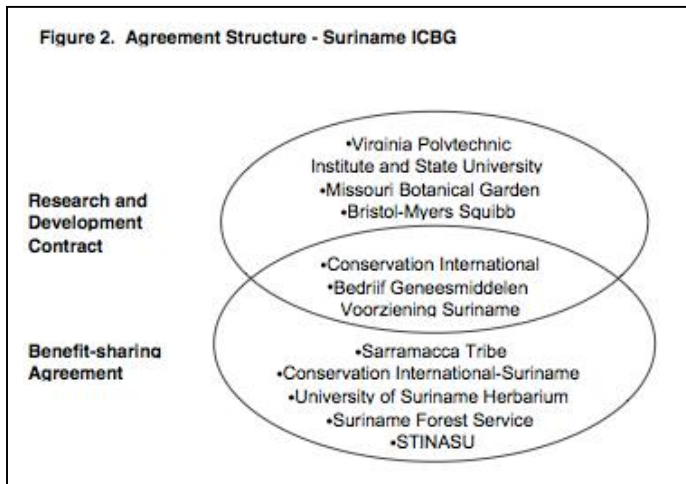
- FAS (NSF y el NIH-Fogarty International Center)
- 7 subproyectos en 11 países (regiones tropicales)
- Proyectos de 400.000 USD-700.000 USD, 5 años
- Control total del conocimiento generado, en custodia en Universidades de los USA
- Las muestras son entregadas a empresas privadas biofarmaceuticas para que realicen los estudios

# EXPLORING BIODIVERSITY FOR HEALTH



## The International Cooperative Biodiversity Groups (ICBG) Program

A U.S. Government funded effort to promote equitable sharing of biodiversity benefits in the context of integrated research and development toward drug discovery, biodiversity conservation and economic development.





## Box 1. Types of Benefits to Source Country Partners from Bioprospecting Agreements

**Royalties** - A percentage of earnings from commercial sales by the licensing partner may be agreed upon in the initial agreement, or the agreement can specify a range and require the parties to negotiate the final rate on a case by case basis. Some issues to consider in royalty structures include: a) relative contribution of partners to invention and development; b) information provided with samples; c) novelty or rarity of sample organisms.

**Advance payments** - Access fees may take the form of lump-sum or milestone payments, per sample fees, payment for re-supply of samples, or in-kind contributions of equipment, training, medicines, etc.. Advance payments are valuable for establishing trust funds that can provide immediate benefits to stakeholders.

**Equipment, training and infrastructure** - Commercial partners or non-profit funding organisations may provide resources to help build the capacity of source country partners to execute current or future needs for bioprospecting research, medical care, biodiversity management, etc..

**Priority research areas** - Agreements can require that locally important, but understudied, diseases and indigenous therapies will be investigated by commercial and other scientific partners. Additionally, they can focus specimen collections and identification on geographical areas or biological groups that are high priorities for conservation needs.



5 años de duración

11 regiones con 15 países (organismos)

10.000 muestras cada país

30 subproductos por muestra

50 resintesis por subproducto

TOTAL LIBRERIA : 225.000.000

compuestos/5 años

Total pagos : 500.000 USD por organismo/5 años

Total por compuesto : 30 USD

Screening por día: 125.000 compuestos

# EXPLORING BIODIVERSITY FOR HEALTH



HOME PROJECT + PARTNERS MEDIA + PARTNERSHIPS

**Venoms**

**173,000** | **40,000,000**  
Venomous animals | Venom proteins

**VENOM PEPTIDE**

## 2011

- 2 Francesas
- 1 Alemana
- 2 Inglesas
- 2 Españolas
- 1 Holandesa



VENOMICS, A European project supported through the Seventh Framework Program (FP7 HEALTH), 2011-2015

22 EL PAÍS Domingo 12 de julio de 2015

### CIENCIA Y TECNOLOGÍA

## Se busca veneno que cure

Un consorcio extrae 5.700 proteínas de ponzoñas de 203 animales para fabricar medicamentos contra el dolor, la diabetes o el cáncer

**JANE PRATS, Valencia**  
Desde cualquier pradera su veneno mata allá de una ametralladora serpiente, una tarántula peluda o un gran lagarto agresivo, los investigadores del consorcio europeo Venomics contemplan, por el contrario, una oportunidad de desarrollar medicamentos contra el dolor, la diabetes, el cáncer o enfermedades autoinmunes. El proyecto Venomics, integrado por laboratorios de universidades y empresas de Bélgica, Hungría, Francia, Portugal y España, ha recopilado venenos de 203 especies animales de los que ha extraído 5.700 pequeñas proteínas (proteínas) que cree potenciales candidatas para convertir en fármacos. La iniciativa ha entrado en su fase final, que consiste en medir la actividad farmacológica de esas y que, tras un meticuloso y sofisticado proceso de cribado, pretende hablar entre uno y cinco

pepétidos con propiedades terapéuticas. Puede parecer un escaso hallazgo para tanto trabajo, pero así funcionan las cosas en la industria del medicamento. No es nada fácil dar con una nueva molécula, y si se encuentra, el resultado puede traducirse en otro o varios medicamentos que ayuden considerablemente a la salud.

Los primeros pasos de esta iniciativa se dieron a finales del año 2011 en las selvas de la Guayana y la Polinesia francesas, así como en la isla de Mayotte, al norte de Mozambique. En total se recogieron venenos de 203 especies, de las que se extrajeron 293 muestras biológicas (21 de cada glándula y 172 de suero). El hecho de trabajar en territorio francés no es casual, el proyecto es una iniciativa de Csic Sacyr (French Alternative Energies and Atomic Energy Commission), una ent-

dad integrada en la principal red de centros de investigación de Francia que cuenta con un grupo líder en biología. El presupuesto asciende a nueve millones de euros, seis de ellos subvencionados por la Comisión Europea.

**Un sofisticado crible**  
Los venenos animales no son una sustancia homogénea, sino un sofisticado cóctel compuesto por distintas proteínas que han ido perfeccionando su mecanismo de acción combinado a lo largo de miles de años de evolución para provocar los mayores da-

ños neurotóxicos, hemotóxicos o cardiotoxicos en la víctima. Cada una de estas moléculas tiene una función diferente: una puede alterar el proceso de coagulación, otra reducir la presión arterial, otra más destruir las células con las que entra en contacto. Por ello, el siguiente paso fue describir cada una de estas moléculas entre las muestras seleccionadas. Por un lado, se descodificó la secuencia de ADN de los venenos, y, por otro, se seleccionaron las proteínas potencialmente relevantes. El objetivo: obtener de cada proteína seleccionada su ARN secuenciado.

No es fácil dar con nuevas moléculas para convertir en fármacos

Las muestras proceden de las selvas de la Guayana y la Polinesia francesas

do. El resultado es una lista de 5.700 proteínas con su correspondiente transcripción. Siguiendo este proceso, los investigadores ya han conseguido fabricar los venenos. Ahora de empezar la recta final del proyecto. La compañía danesa Zealand Pharma ha iniciado ensayos clínicos para determinar el efecto de los péptidos en las células humanas. El objetivo es medir cuáles tienen capacidad inmunomoduladora (con propiedades terapéuticas para patologías autoinmunes como la psoriasis o la artritis) o intervienen en la respuesta de la insulina (podrían servir para desarrollar medicamentos para la diabetes). Además, se pretende observar otras reacciones, como las uniones intracelulares, en las que se regula el intercambio de pequeñas moléculas y que tienen un papel muy relevante en distintos procesos, algunos de ellos presenciales, por ejemplo, en las enfermedades cardiovasculares.

Una de las tarántulas de las que se ha extraído veneno.

# EXPLORING BIODIVERSITY FOR HEALTH



## GOOD BUSINESS & PRACTICE & CASE STUDIES ON BIODIVERSITY



# EXPLORING BIODIVERSITY FOR HEALTH



## 4.3 | CASE STUDY 1

### ASTRAZENECA AND GRIFFITH UNIVERSITY, BRISBANE, QUEENSLAND, AUSTRALIA

AstraZeneca is one of the world's leading pharmaceutical companies with over 12,000 people working on the Research and Development of new medicines for treating human health. AstraZeneca scientists investigate new treatments for cancer, infection, pain and cardiovascular, respiratory, inflammation, gastro-intestinal and central nervous system diseases as well as others.

Griffith University, Brisbane and the Queensland State Government entered into an agreement with AstraZeneca in 1993. This set up a Natural Product Discovery laboratory in Brisbane; specifically located to take advantage of the intellectual strength in Brisbane and the proximity to the unique natural environment of Queensland - the rainforest and reef. Australia is one of the twelve mega-diverse countries and is a party to the Convention on Biodiversity.

The agreement was set up in compliance with the Biodiversity laws of the State of Queensland and the Australian Federal Government<sup>19</sup>. These laws encourage the Conservation of Biodiversity and the sustainable use of natural products, and they further encourage Access and Benefit Sharing. Some general principles include:

- Give effect to CBD & other international obligations
- Facilitate ecologically sustainable access and use
- Enable fair and equitable sharing of benefits
- Ensure use of traditional knowledge undertaken with cooperation and approval of holders of such knowledge
- Enhance biodiversity conservation and value
- Facilitate continued non-commercial research
- Integrated into biotechnological development policies and strategies

Under the agreement, Griffith University retains intellectual property rights with AstraZeneca having

the active ingredient(s) and identify the chemical structure(s).

The active ingredient is usually not suitable to develop as a medicine but is a lead for creating different chemical structures for extensive pharmacological investigation.

Since the collaboration commenced, the Natural Product Discovery laboratory has tested over 35,000 specimens from plant and marine environments. These specimens have been collected via contracts with the Queensland Herbarium and with the Queensland Museum as well as from other sources.

#### Benefits for Griffith University, Queensland and Australia

The agreement and associated funding has established a world-leading research facility in the area of natural product discovery. This facility has lured several leading Australian researchers back to their homeland.



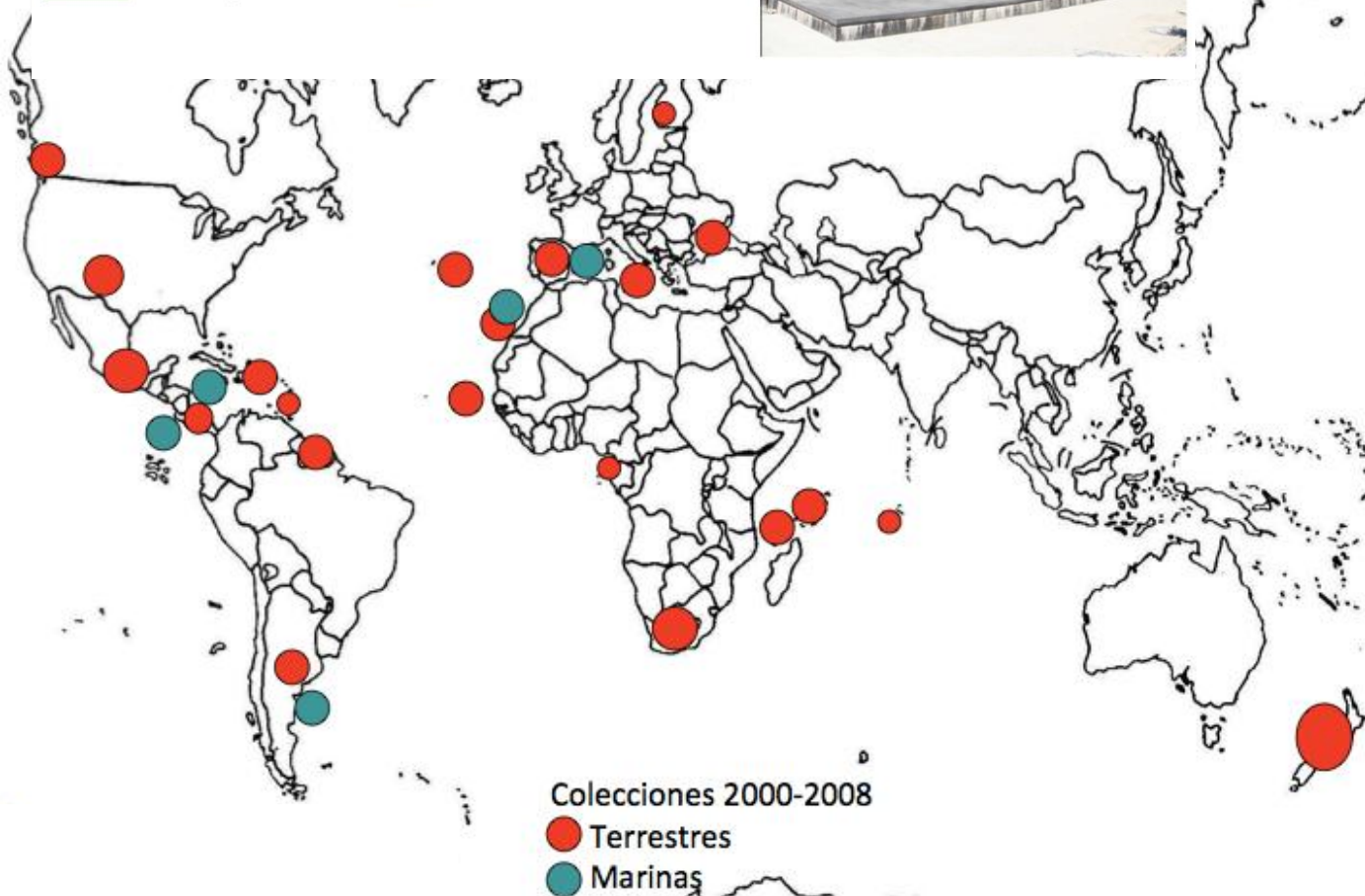
Natural Product Research Institute, Brisbane.



# EXPLORING BIODIVERSITY FOR HEALTH



Centro de Excelencia en Investigación de Medicamentos Innovadores en Andalucía



Medicamentos Innovadores  
a partir de Productos Naturales de  
origen microbiano

# EXPLORING BIODIVERSITY FOR HEALTH



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**World leader in the development  
and commercialization of anticancer drugs of marine origin**



# EXPLORING BIODIVERSITY FOR HEALTH



## MMV Discovery Portfolio 2008 – Fourth Quarter

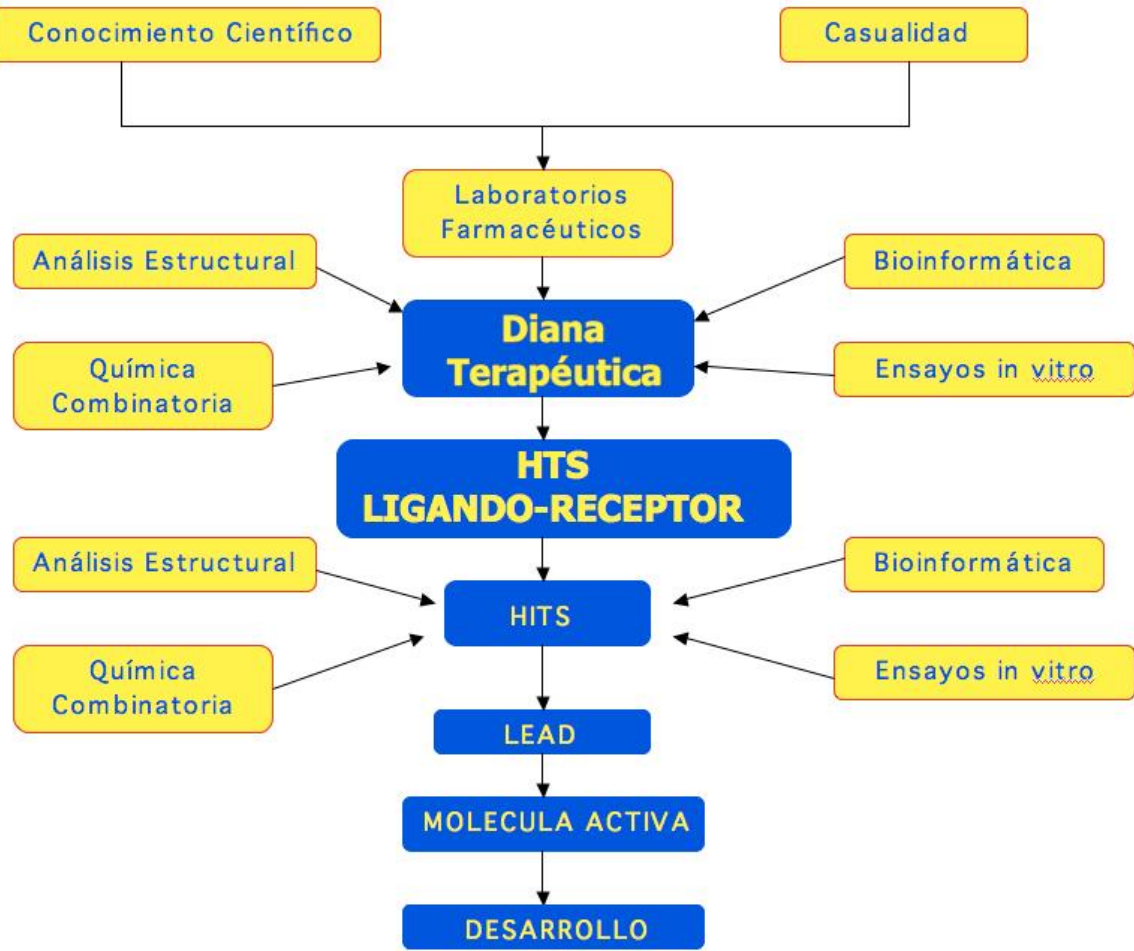


### LEAD GENERATION

- HTS Workup, GSK
- HTS Workup, Broad/Genzyme
- Natural Products, Broad/Genzyme
- GNF Screening, Novartis
- Liver Assays, Novartis
- Natural Products, Eskits
- Gametocyte Screen, Eskits
- Natural Products Screen, Eskits/DPi
- SF Library Screen, Eskits
- Liver Assays, SBFI
- Kinase Screen, Dundee
- Natural Products, USF
- DHODH, GSK
- HSP90, Broad/Genzyme
- DHODH, Broad/Genzyme
- HDAC, Broad/Genzyme
- Kinase Hits, Novartis
- Heterocyclic Hit-to-Lead, TDR/Pharmacopoeia
- Immucillins, Albert Einstein
- Quinolones, USF

### LEAD OPTIMIZATION

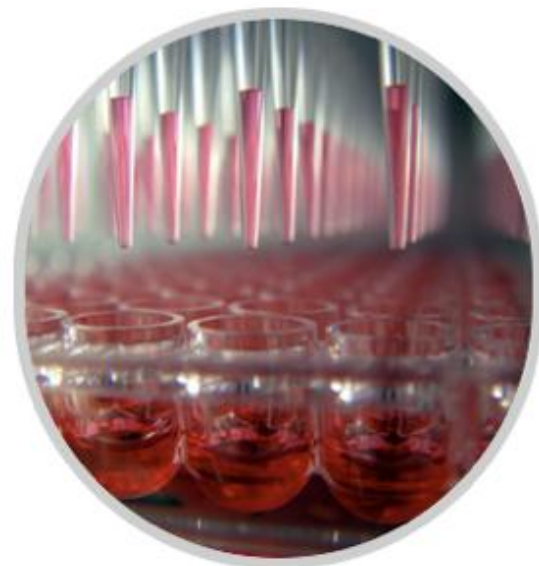
- THIQ, GSK
- Aminoindoles, Broad/Genzyme
- Whole Cell Leads, Novartis
- DHODH, USTW/LW/Monash
- Falcipains, GSK
- IPT Pyridones, GSK
- Macrolides, GSK
- DHFR, BIOTEC/Monash/LSHM
- Ozonides, Monash/UNMC/STI
- KAC776, Novartis



**Exploración de la Biodiversidad**  
*Revolución*  
*High-Throughput Screening*



## High Throughput Screening Drug Discovery Technology



In the 20 years since High Throughput Screening Drug Discovery Technology (HTS-DD) has been implemented it has become universal within the pharmaceutical and biotech industries as the major approach to identify novel prototype molecules for new targets.

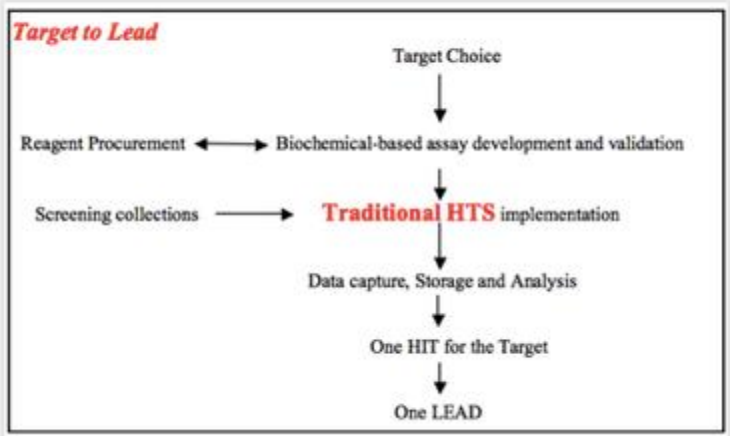
It has also spawned a billion dollar industry that supports the increasing demands for speed, capacity, efficiency and cost effective screening of vast libraries of compounds.



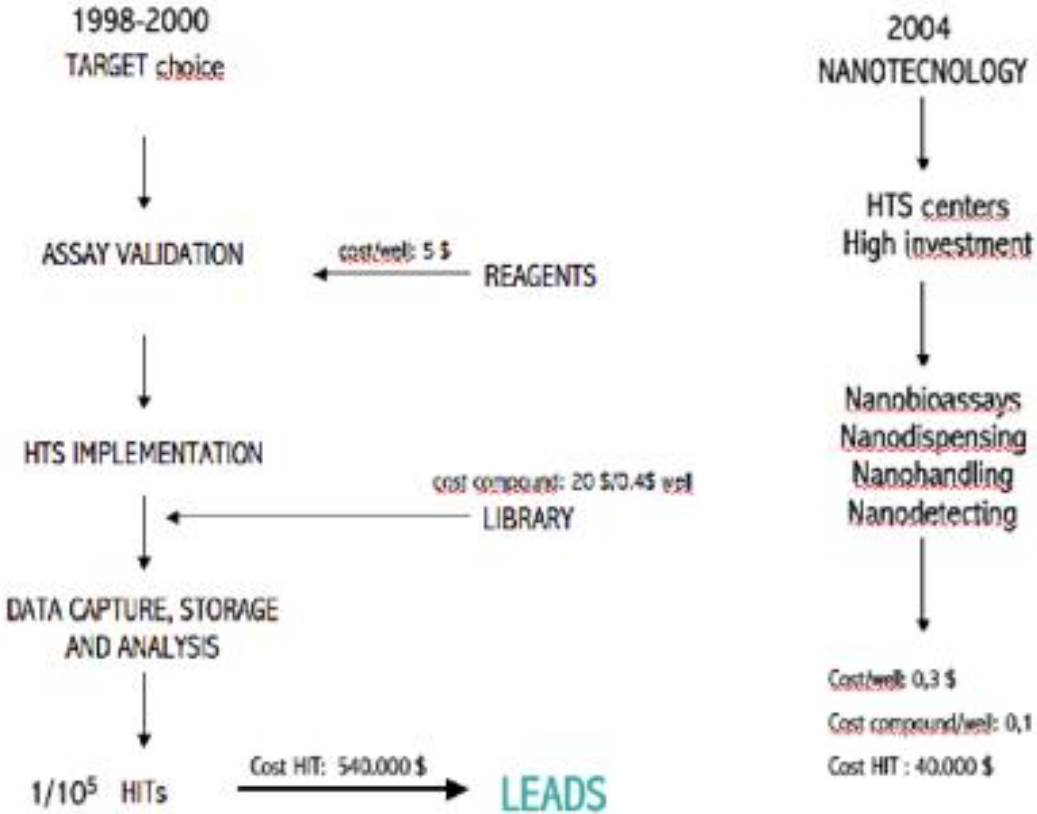
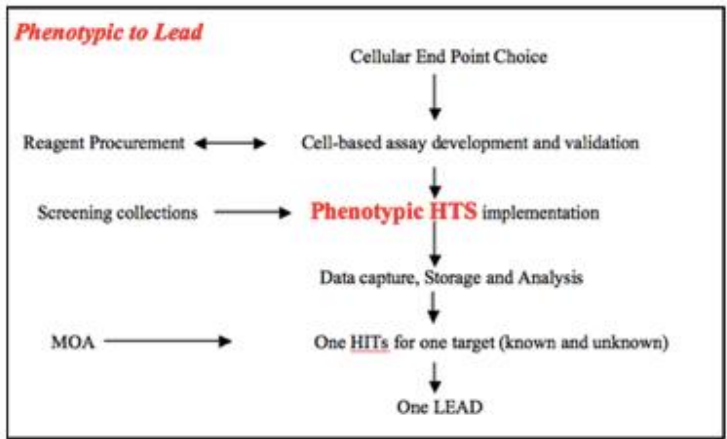
EXPLORING BIODIVERSITY FOR HEALTH



Traditional HTS



Phenotypic HTS



# EXPLORING BIODIVERSITY FOR HEALTH



Facility Name	Institution		
Biomolecular Screening Facility	EPFL, Ecole Polytechnique Fédérale de Lausanne		
Broad Institute Chemical Biology Platform	Broad Institute Chemical Biology Platform		
HT-Technology Development Studio	Max Planck Institute of Molecular Cell Biology and Genetics (MPI-CBG)		
Vanderbilt Screening Center for GPCRs, Ion Channels, and Transporters	VANDERBILT UNIVERSITY		
University of Cincinnati HTS	University of Cincinnati		
ECCRB/NCSU	North Carolina State University		
High-Throughput and High Content Screening Core Facility	University of Colorado Anschutz Medical Campus		
Berkeley Screening Center	University of California, Berkeley		
Michigan High Throughput Screening Center	Michigan State University Community College		
Boston University Center for Molecular Discovery	Boston University		
Broad Institute High Throughput	Broad Institute of Harvard and MIT		
Center for Predictive Medicine HT Biology Core Facility	University of Louisville		
High-Throughput Screening Resource Center	The Rockefeller University		
Yale Small Molecule Discovery Center	Yale University		
Institute for Tuberculosis Research	University of Illinois at Chicago		
High-Content Screening Core Facility	University of Miami Miller School of Medicine		
Experimental Therapeutics Programme, HTS	Spanish National Cancer Research Centre (CNIO)		
Center for Bio-Active Molecules Screening (Centre de Ciblage pour Molécules Bio-Actives (CMBIA))	Commissariat à l'Énergie atomique, département des Sciences du Vivant (CEA/DSV) CNRS		
Genomics Resource - High-Throughput Screening Facility	Fred Hutchinson Cancer Research Center		
Drug Screening Resource	University of Utah		
Chemical Genetics Screening Core	Washington University		

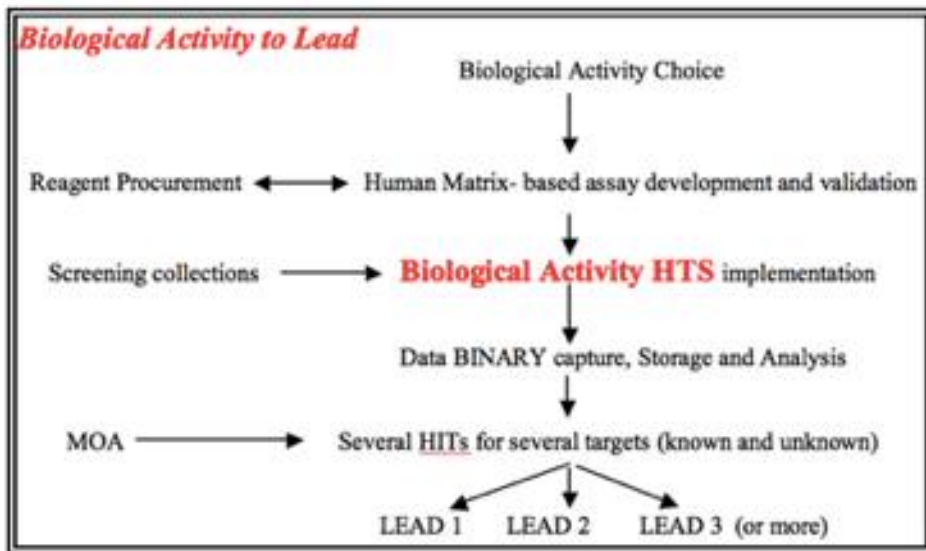
**> 150 Centros de Screening en el mundo**  
**125 en los USA**  
**Coste medio : \$ USD 300 M**





# Biological Activity High Throughput Screening Drug Discovery Technology

2006

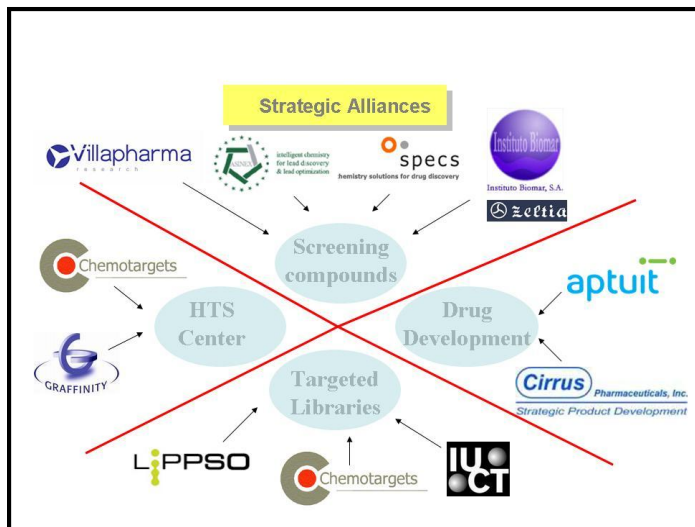


Biological Activity HTS-DD enables the direct assessment of compound action simultaneously on a several and specific pathways in a specific human matrix, either than a defined target or a defined end point.





A partir de 48.500 productos (small molecules, péptidos, extractos de plantas) procedentes de librerías de organismos públicos y privados :



ASINEX (Rusia)  
SPECS (Holanda)  
MAGELLAN (USA)  
BIOMAR-ZELTIA (España)  
LIPPSO (España)  
UIC (España)  
Universidad de Sofia (Bulgaria)  
Universidad de Plovdiv (Bulgaria)  
BAS (Academia Búlgara de Ciencias)

Se descubrieron 218 nuevos HITS de areas de la Trombosis, hemostasia, oxidación y cáncer. Rindiendo 13 leads



## Competitive advantages comparison

HTS METHOD	BASED IN	TYPE OF INTERACTION	CAPACITY TO DISCOVER NEW TARGETS	CORRELATION <small>In vivo conditions</small>	PROVIDES DATA TO DRIVE CLINICAL PHASES	MOA	TYPE OF RESULT	IT PERSONNEL REQUIRED	HIGH INVESTMENT IN HTS FACILITIES	COST (reagent/well)	EFFICIENCY (HITS per 100.000 compounds)	COST PER NEW HIT
Traditional or Biochemical (ligand binding)	Known Target	Artificial environment (buffers)	NO	NO	NO	Known	Numeric	YES (large datasets)	YES HTS with nanotech	\$1.00 (\$0.30 nanotech)	1 HIT	\$540.000 (\$40.000 nanotech)
Phenotypic	Known cellular pathway	Artificial environment (cell culture)	NO/YES	NO/YES	NO	Un known	Numeric and/or Binary	NO/YES	YES HTS with nanotech	\$2.00 (\$0.30 nanotech)	1 HIT	\$540.000 (\$40.000 nanotech)
<b>Biological Activity</b>	<b>Known activity</b>	<b>Natural environment (blood, plasma, LCR)</b>	<b>YES</b>	<b>YES</b>	<b>YES</b>	<b>Un known</b>	<b>Binary</b>	<b>NO</b>	<b>NO HTS w/o nanotech</b>	<b>\$0.05</b>	<b>450 HITS</b>	<b>\$100.00</b>

# EXPLORING BIODIVERSITY FOR HEALTH



Entrevista con el Dr. Javier Pedreño, Presidente de Thrombotargets Group

## Revolucionaria tecnología para la industria farmacéutica

La facturación prevista para el primer fármaco supera los 1.500 millones de euros

Thrombotargets Group usó a varios científicos españoles con experiencia en investigación, docencia, y trabajo en laboratorios farmacéuticos para desarrollar una biotecnología única en el mundo. *By: J. Rodríguez*

Esta tecnología permite la identificación de nuevos candidatos a fármacos en áreas terapéuticas médicas muy rentables (las de mayor medicación), como la hemostasia, trombosis, anemias, etc... Mediante el uso de esta tecnología Thrombotargets ha obtenido, en tan sólo dos años de existencia, una colección de candidatos a nuevos fármacos en desarrollo (pipelines) innovadores y competitivos. Thrombotargets ha financiado su crecimiento con capital de inversores particulares (*financiado en parte*), con el objetivo de priorizar la creación de valor en la etapa inicial de la compañía. El Dr. Javier Pedreño, Presidente de Thrombotargets Group, explica las novedades que aporta la tecnología patentada por la empresa y los principios sobre los que creó este proyecto innovador. **Fig 7**

El Dr. Javier Pedreño, de pie en el centro de la imagen, Presidente de Thrombotargets Group, junto a José Joaquín Rodríguez, a la derecha, director de I+D

DEPARTMENT OF HEALTH & HUMAN SERVICES Public Health Service

Office of Orphan Products Development (OP-11)  
Food and Drug Administration  
3000 Parkway Lane  
Rockville, MD 20857

January 23, 2007

Aptus Consulting, Inc.  
10245 Hickman Mills Drive  
Kansas City, Missouri 64137

Attention: Kathy Olivia-Whalen  
Principal Consultant

Re: Designation request # 06-2347

Dear Ms. Olivia-Whalen:

Reference is made to your request for orphan-drug designation submitted on Thrombotargets Corporation on December 4, 2006, of microencapsulated non-glycosylated recombinant tissue factor (company name: TT-1030M) for "the non-life threatening, mild-to-severe bleeding episodes due to catenousa inia procedures in hemophiliac patients." Please also refer to our letter of December 14, 2006.

Pursuant to section 526 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 527 (21 U.S.C. 360cc)). Therefore, prior to final marketing approval, we request that you compare the drug's designated orphan indication with the proposed marketing indication and submit additional information to amend the orphan-drug designation if necessary.

Please note that if the above drug receives marketing approval for an indication other than what is designated, it may not be entitled to exclusive marketing rights under section 527 (21 U.S.C. 360cc). Therefore, prior to final marketing approval, we request that you compare the drug's designated orphan indication with the proposed marketing indication and submit additional information to amend the orphan-drug designation if necessary.

DEPARTMENT OF HEALTH & HUMAN SERVICES Public Health Service

Office of Orphan Products Development (OP-11)  
Food and Drug Administration  
3000 Parkway Lane  
Rockville, MD 20857

OCT 11 2007

Aptus Consulting, Inc.  
10245 Hickman Mills Drive  
Kansas City, Missouri 64137

Attention: Kathy Olivia-Whalen  
Authorized agent

Re: Designation request #07-2464

Dear Ms. Olivia-Whalen:

Reference is made to your request for orphan-drug designation request submitted on behalf of Thrombotargets Corp., dated July 16, 2007, of microencapsulated modified glycosylated tissue factor (company name: TT-030M) for "the treatment of spontaneous and trauma-induced bleeding episodes in severe von Willebrand disease (vWD) and moderate von Willebrand disease when the use of desmopressin is either ineffective or contraindicated." Please also refer to our letter of July 16, 2007, and to the August 24, 2007, release with Mr. Peter L. Vaccari of this office in which the orphan indication was amended to "catenousa bleeding episodes in patients with vWD."

Pursuant to section 526 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 527 (21 U.S.C. 360cc)). Therefore, prior to final marketing approval, we request that you compare the drug's designated orphan indication with the proposed marketing indication and submit additional information to amend the orphan-drug designation if necessary.

Please note that if the above drug receives marketing approval for an indication broader than what is designated, it may not be entitled to exclusive marketing rights under section 527 (21 U.S.C. 360cc). Therefore, prior to final marketing approval, we request that you compare the drug's designated orphan indication with the proposed marketing indication and submit additional information to amend the orphan-drug designation if necessary.

## Investigació tecnològica



PER FRANCESC XAVIER GIL ASALS, JOSEP CASANOVAS, JOSEP MIQUEL PIQUÉ, MITAT ATORGAR EL PREMI TECNOLÒGIC A

**Thrombotargets Europe,** perquè ha creat una plataforma tecnològica que permet el descobriment de nous fàrmacs en l'àmbit cardiovascular, que aporten solucions a l'hemofília i altres malalties hemorràgiques. El Jurat vol destacar que, des d'una recerca científica d'excel·lència i un conjunt de patents internacionals, s'ha creat una empresa a Barcelona que atén una necessitat sanitària mundial.

El Jurat atorga una menció especial al Grup de Recerca en Òptica Aplicada i Processament d'imatge de la UPC, dirigit per la Dra. Maria S. Millán, pel desenvolupament d'un sistema de seguretat basat en la identificació òptica de senyals biomètrics i multifactorials.



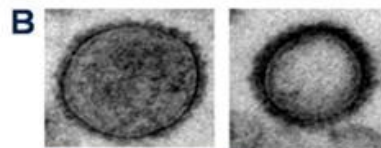


# High-Throughput Screening Drug Discovery

## Obtención de medicamentos

### TT-173

Primera proteína humana recombinante de Factor Tisular Activo Genéticamente Modificado



0.2 μm

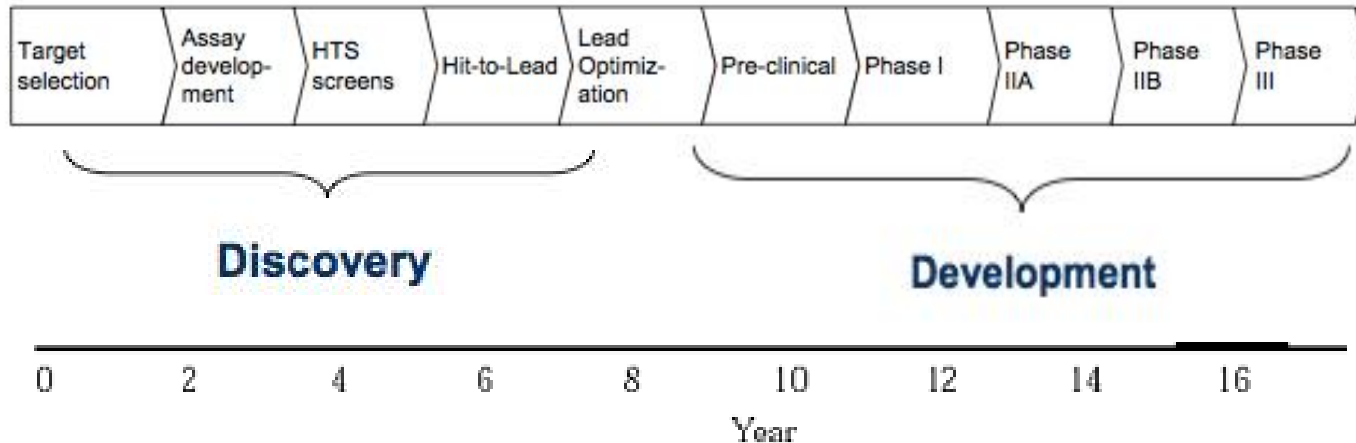




25 M USD investment (public and private)  
10 years from inception and 5 after product patent  
Preclinical, PhI and PhIIa finished  
Protocol of PhIIb/III approved



Out-licensing of TT-173 (2015-2016)

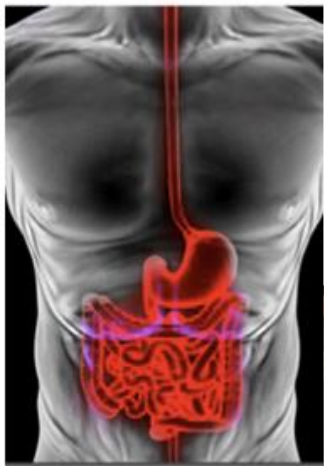


40 x return in 10 years for private investors



# TT-173

Antihemorrágico tópico universal

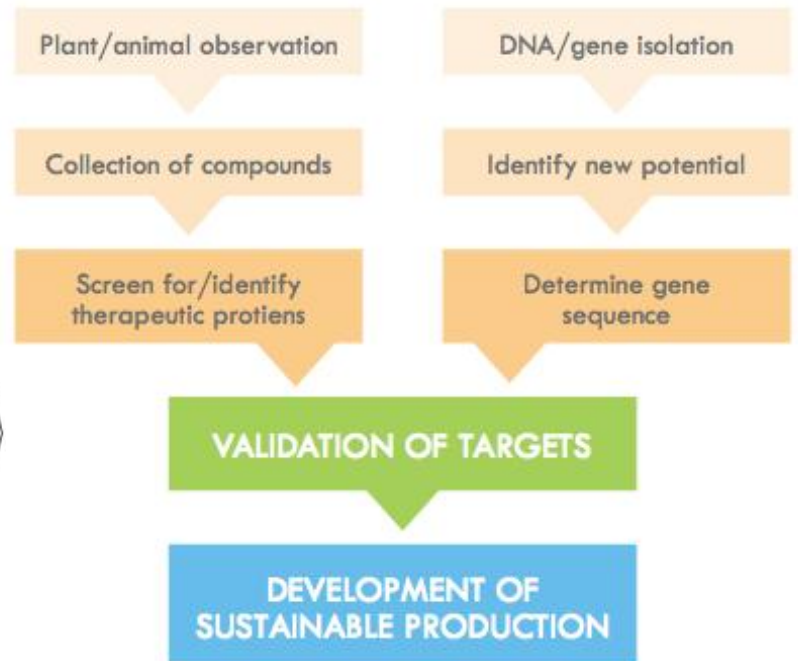
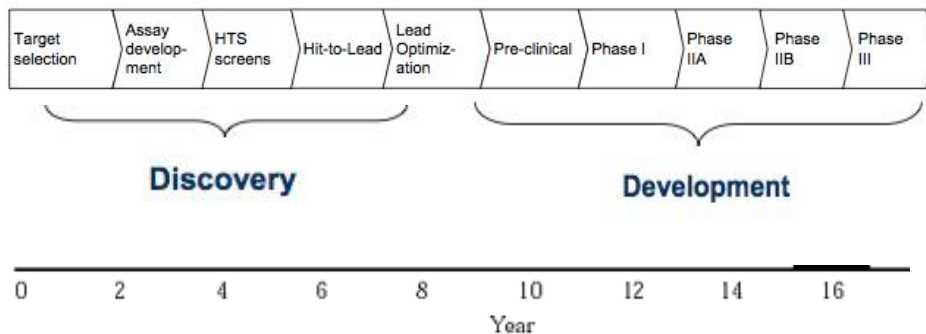




# Reto Tecnológico del Siglo XXI

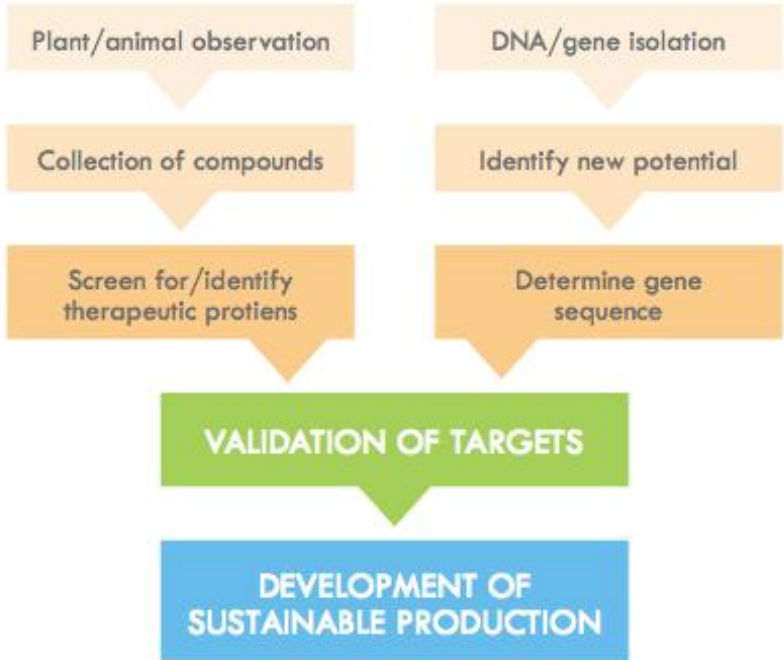
## Drug Discovery Desarrollo de nuevos medicamentos

- Muy lento (15 años)
- Muy complicado (Fases)
- Muy costoso (\$ 100 M)
- Muchos pacientes (1000)





# Reto Tecnológico del Siglo XXI





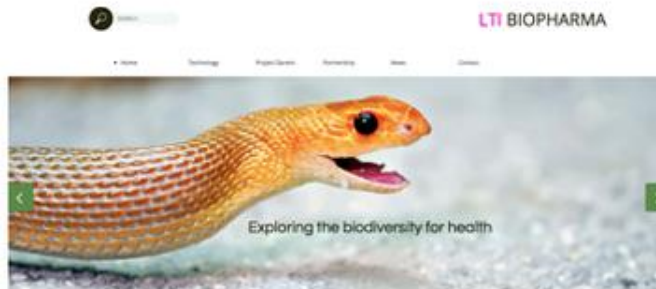
# Plant/Animal Observation





## Facultad de Agronomía de la UBA

Departamento de Bioeconomía, Políticas Públicas y Prospectiva del Programa de Agronegocios y Alimentos.



## LTI Biopharma S.A.

Empresa Biotecnológica Argentina especializada en las tecnologías del High-Throughput Screening Drug Discovery (HTS-DD)

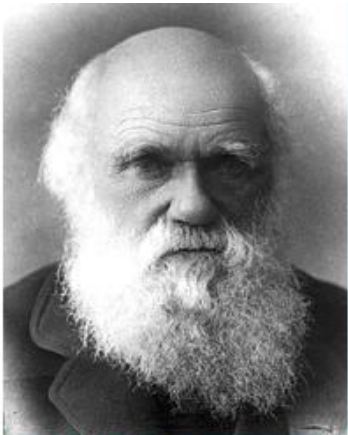
### Actividades Académicas y Empresariales

- Cursos de postgrado en Bionegocios
- Programa de Becarios y Tesis Doctorales
- Proyectos de I+D
- Proyecto Darwin



## PROJECT DARWIN

EXPLORING BIODIVERSITY WITH  
HIGH-THROUGHPUT SCREENING DRUG DISCOVERY TECHNOLOGIES  
FOR HEALTH



# EXPLORING BIODIVERSITY FOR HEALTH



## Management

LTI Biotech-Biopharma is a private biotech company specialized in HTS-DD and has a two-tier management structure consisting of the Consulting Board of Scientific Directors and Executive Management.

The Consulting Board of Scientific Directors is composed by Prof. Dr. Fernando Goldbaum (former President of Agencia Nacional de Promoción Científica y Tecnológica del Ministerio de Ciencia y Tecnología of Argentina) and Prof Dr. Fernando Vilella (Professor of Agronomy Faculty of Buenos Aires University, Dept of Bionegocios).

Executive Management, in turn, has responsibility for the company's daily operations. The two bodies are separate, and no person serves as a member of both.





# Biological Activity High Throughput Screening Drug Discovery Technology

## LTI BIOPHARMA'S HTS PLATFORMS

*"We have created five different families of HTS including thirteen platforms"*

### **Hemostasis Platforms™**

Whole Blood FibrinCoag™  
Plasma FibrinCoag™  
Assembly ProT Complex™  
Platelet Adhesion and Aggregation™

### **Thrombogenicity Tumor Platforms™**

Tumor-associated TF activity™  
Tumor-associated Factor X activators™  
Tumor Cell-mediated assembly of ProCom™  
Tumor-associated Plasmin/MMTs activity™  
Tumor cell-mediated PAI-1/uPA/UPAR activity™

### **SchizoReceptor Platform™**

D2/5H1A/mGlu2 receptors platform™

### **Alzheimer's Disease Platforms™**

BA-FibrinRes™  
BA-Astroglial Platforms™

### **Antioxidant Platform™**

CIO-CD platform™



(Myers et al. Nature 403, 853-858, 2000).

TABLA 1. Especies estimadas e identificadas

Especies	Estimadas	Identificadas	% Iden
Insectos	8.750.000	1.025.000	11,71%
Bacterias y Arqueos	1.000.000	4.000	0,4%
Virus	4.000	1.550	38,75%
Protozoos	200.000	40.000	20%
Nematodos y Gusanos	400.000	25.000	6,25%
Moluscos	200.000	70.000	35%
Crustaceos	150.000	43.000	28,6%
Otros organismos	250.000	110.000	44%
Peces	35.000	27.000	77,14%
Aves	10.000	9.700	97%
Mamíferos	4.809	4.650	97%
Anfibios	4.780	4.780	100%
Plantas	320.000	270.000	84,3%
Hongos	1.500.000	72.000	4,8%
Algas	400.000	40.000	10%
TOTAL	13.800.000	1.800.000	13%



# Conflicto Bioquímico

- Enzimas
- Péptidos
- Hormonas
- Neurotransmisores
- Activadores
- Inhibidores
- miRNA
- siRNA
- HRV's

**Guerra Bioquímica**  
*Dos organismos vivos*

**Supervivencia Bioquímica**  
*Un organismo vivo y su medio hostil*

# EXPLORING BIODIVERSITY FOR HEALTH





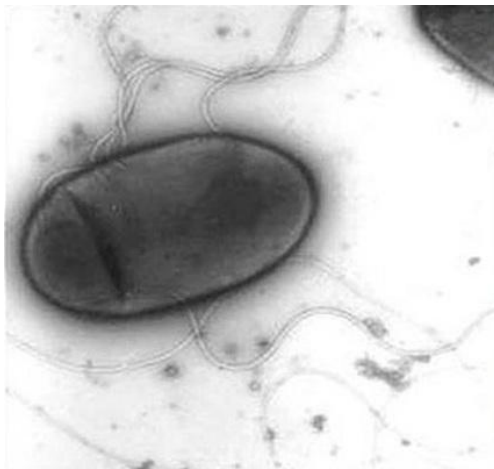
# EXPLORING BIODIVERSITY FOR HEALTH



# EXPLORING BIODIVERSITY FOR HEALTH



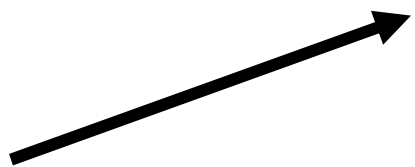
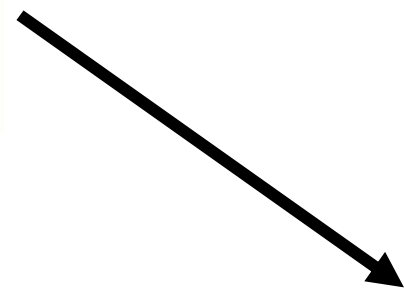
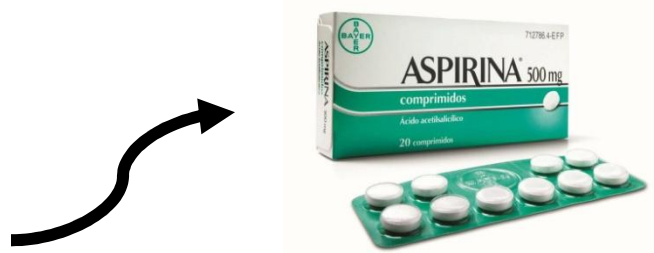
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# EXPLORING BIODIVERSITY FOR HEALTH



# EXPLORING BIODIVERSITY FOR HEALTH





## Partnering & Crowdfunding

- Raising funding privado y público
- Campañas de Crowdfunding locales y globales
- Partnering entre países ricos en biodiversidad

Maria Alejandra Pérez Bisbal . MA  
Global Partnership Development Director

[mariabisbal@ltibiotech.com](mailto:mariabisbal@ltibiotech.com)

# EXPLORING BIODIVERSITY FOR HEALTH



# EXPLORING BIODIVERSITY FOR HEALTH

