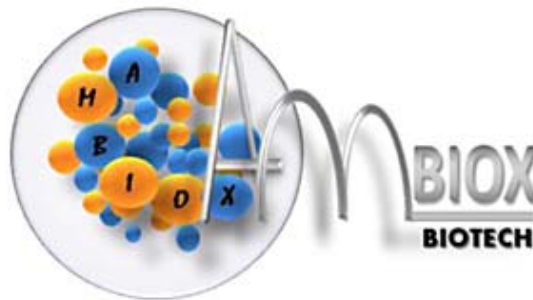


# Programa Cooperación Farma-Biotech

Jornada IV: Ámbitos terapéuticos relacionados con respiratorio,  
dermatología, nefrología, inflamación e infección

***Preventing the spread of HIV / AIDS through the use of nanotechnology***



Madrid, 12 July 2011

# Programa Cooperación Farma-Biotech

Jornada IV: Ámbitos terapéuticos relacionados con respiratorio, dermatología, nefrología, inflamación e infección

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## Content

### 1. The Company

### 2. The Product

- a) Therapeutic focus
- b) Innovative mechanisms of action
- c) Differential features facing the market
- d) Current status of development
- e) IPR protection
- f) Pitfalls & Risks to be considered

### 3. Availability for cooperation

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1.

# AMBIOX BIOTECH

# Ambiox's beginnings



- Dept Inorganic Chemistry,
- Expertise in dendrimer and polymer synthesis
- University of Alcalá, Madrid

- Laboratory of Molecular Immunobiology,
  - expertise in therapeutics against HIV/AIDS
- Hospital Gregorio Marañón, Madrid

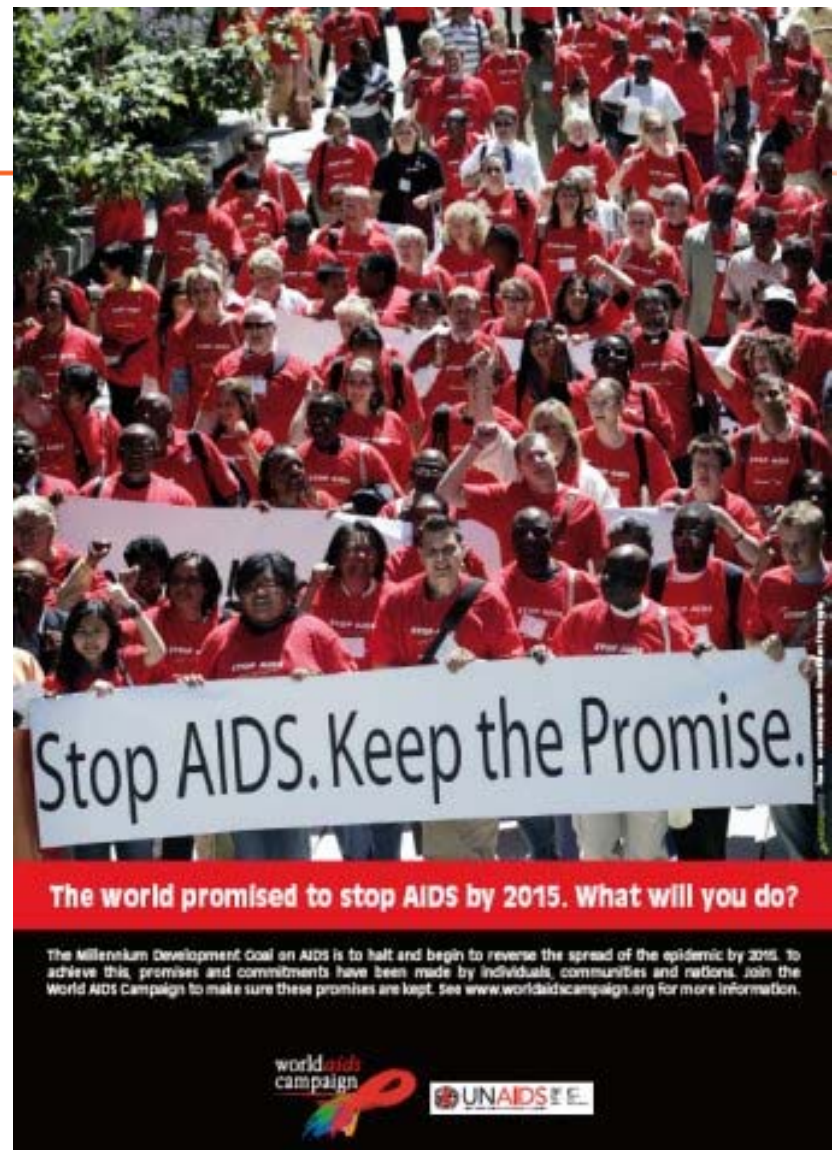


- Ambiox Biotech is a spin-off from the University of Alcalá.
- Ambiox is the result of the fruitful collaboration of these Research Centers for the identification of effective molecules against HIV

# Our vision

- **Stop AIDS impact on society**

- **→ with a product that allows the person to protect him/herself from HIV infection**



## Team

# Who we are?

Corporate  
strategy



**Javier de la Mata,**  
**PhD** Inorganic  
Chemistry Professor  
Alcala University  
Madrid

Operations



**Rafael Gómez,**  
**PhD** Inorganic  
Chemistry  
Professor Alcala  
University Madrid

Scientific  
Director



**Ma Angeles Muñoz,**  
**PhD**  
Director Molecular  
Immunobiology, Hospital  
Gregorio Marañón

General  
Director



**Teresa Gonzalo,**  
**PhD, IE MBA,** Postdoc  
Gene Therapy against  
AIDS, Hospital Gregorio  
Marañón, Madrid



# Resources of Ambiox

- Ambiox Biotech has an active R&D program led to the identification of novel compounds for the prevention and treatment of HIV / AIDS

- Pipeline of potential molecules (Inorganic Chemistry Dpt, Alcalá University)
- Patent: covered family of compounds
- Efficacy and Toxicity Screening, In vivo testing (Hospital G.Marañón)

- World renowned Scientist in **Dendrimer Nanotechnology and therapy against AIDS** involved in Ambiox Biotech
- Specific Training in **Management of Biotech Companies** (Master International in Biotechnology Management, IE Business School)

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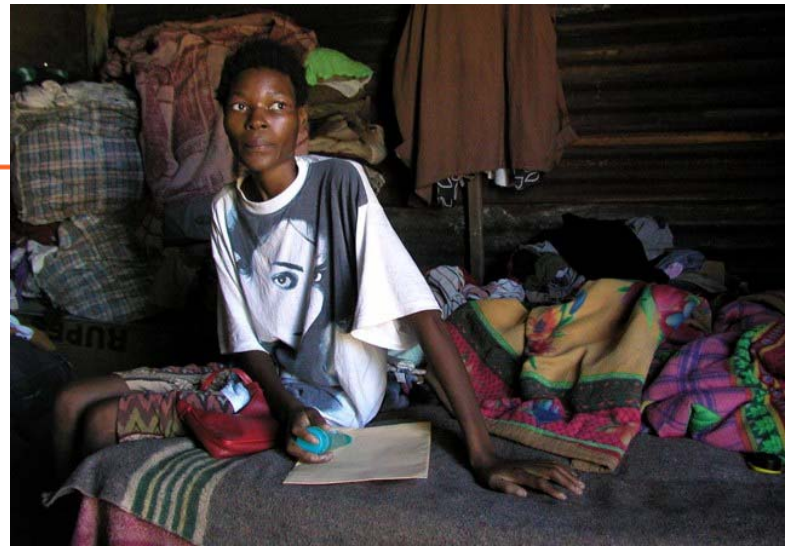
2.

# THE PRODUCT: MICROBICIDE



What is the global price due to HIV infection?

- **33 million people** affected by HIV
- **5 millions** are infected annually
- **80%** sexual transmission
- Annual cost in U.S **\$12 billions**



Nanotechnology

AIDS

↓

**PREVENTION OF VIRAL  
TRANSMISSION DURING SEXUAL  
CONTACT**

# a) Our product: microbicide

- A product that shuts the door to virus entry



Topical gel with dual action

- MICROBICIDE prevents HIV infection
- ANTI-INFLAMMATORY that prevents Immune system activation

# Our microbicide features

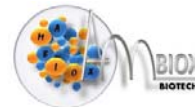
1. Microbicide is not toxic in vitro in cell lines or vaginal epithelium
2. Inhibits HIV infection more than 80% mainly by binding of the dendrimer to the protein gp120 of HIV.
3. No cause inflammation
4. No vaginal irritation and does not alter the bacterial flora in vivo.

## b) Innovative Mechanism of Action

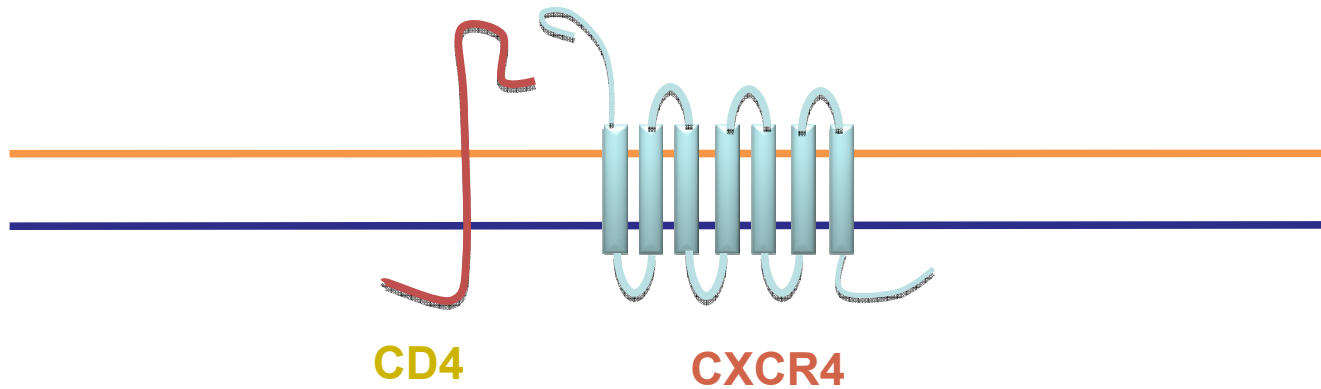
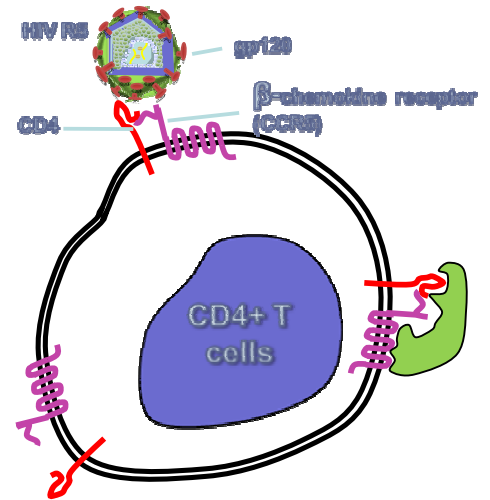
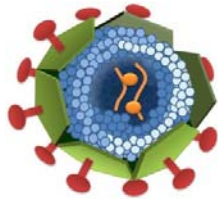
- It is possible to prevent the spread of HIV **using dendrimeric molecules that compete with the virus by their affinity to receptors on target cells, preventing their entry.**
- The ability of dendrimers developed by Ambiox Biotech to interfere with virus-cell interaction suggests that they might act as topical microbicides, ie compounds applied to the vaginal or rectal mucosa to prevent sexually transmitted diseases.
- A key difference of our system compared to others who are under study is its **anti-inflammatory feature**
  - **dual action anti-inflammatory and microbicide** to reinforce their anti-HIV action → which may result in decreased activation of the immune system and allow greater control of viral replication.

# Animation of Mechanism of Action

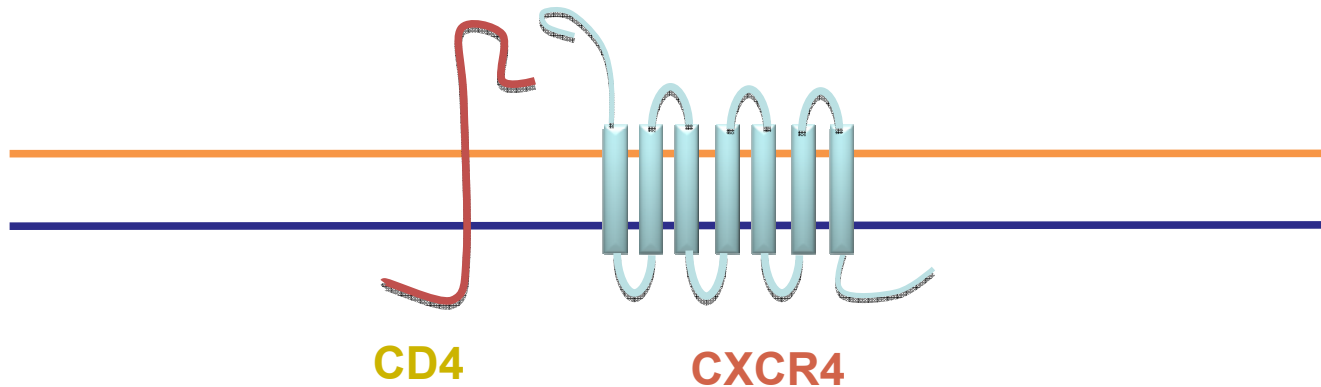
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# HIV entry

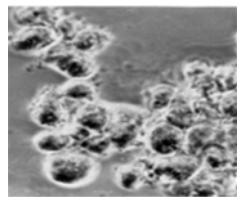
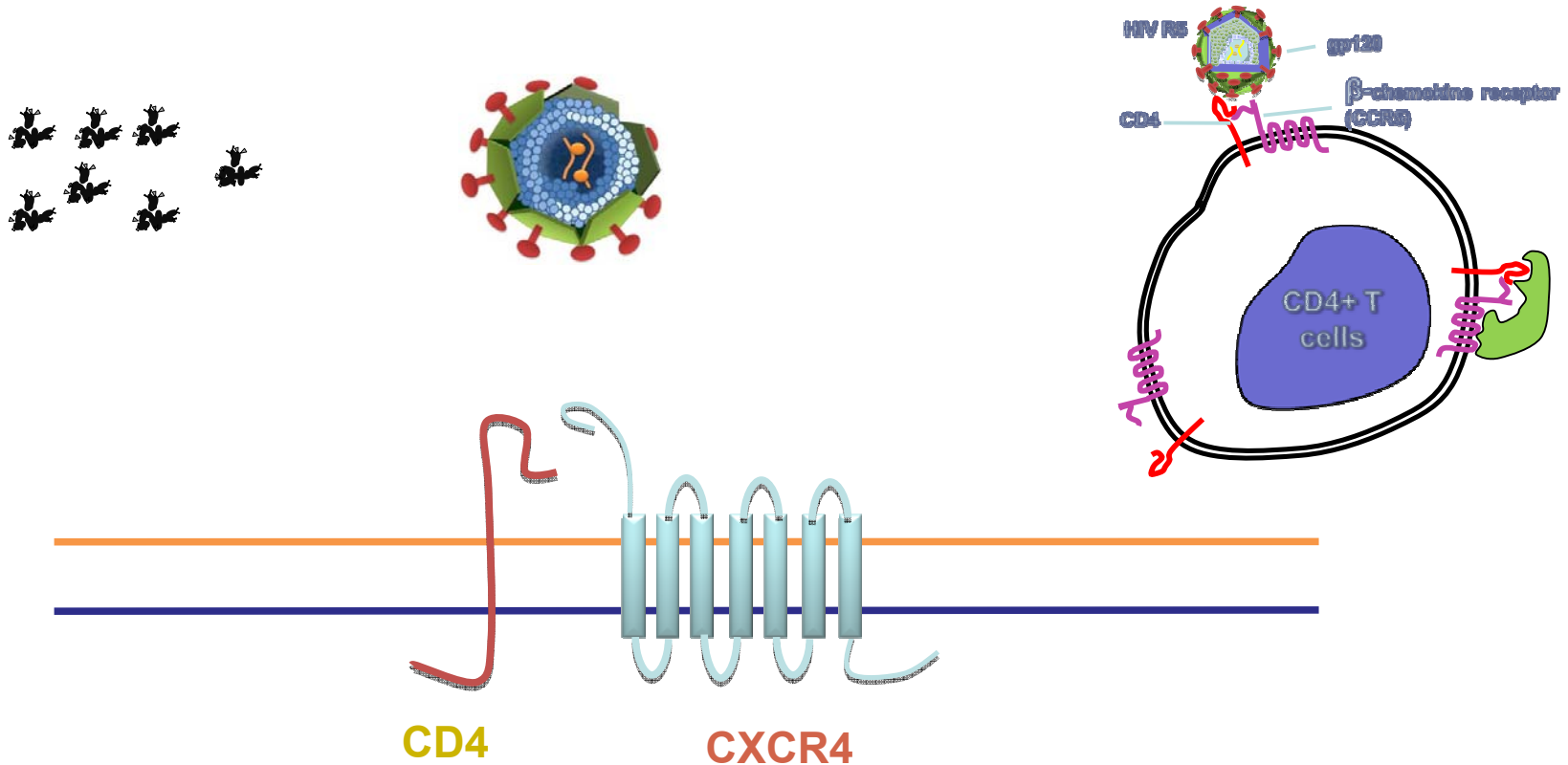


# Anionic dendrimer bound to the HIV



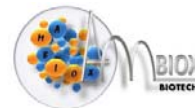


# Anionic dendrimer bound to the receptor and/or coreceptor



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# MARKET



# c) Market size and product

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Global Market: 2011-2020 **US\$0.9 -1.8 billions**

**A product for healthy people!**

Market segment daily hygiene, **\$5billions**

Product advantages:

- Topical product, non-injectable, easy to use
- **Treatment costs:**
  - **\$0,4** development countries
  - **\$1,3** industrialized countries

**article:** The Economics of Microbicide Development. A Case for Investment. Pharmaco-Economics Working Group of the Rockefeller Foundation Microbicide Initiative

# COMPETITORS (DENDRIMERS)

Microbicides in development

Product	Description	Company	Phase
<del>PRO 2000 (bis-aryl sulfonate)</del>	Polimer Polyanionic lipophilic	Indevus/Endo	Phase 3
VivaGel (SPL 7013)	Dendrimer with superficial groups sulphonated naphtalen acids	Starpharma	Phase 1/2

## Advantages:

- Open Pharmaceutical category: various products/category
- Antiviral therapy, multiple drug combinations

article: *Cash infusion for HIV Microbicide*, Nayanah Siva, Nature Biotechnology 27, 401 - 402 (2009)

# Our competitor Starpharma seizes a big market too



## VivaGel® - Anti-viral Condom Coating and Microbicide Gel

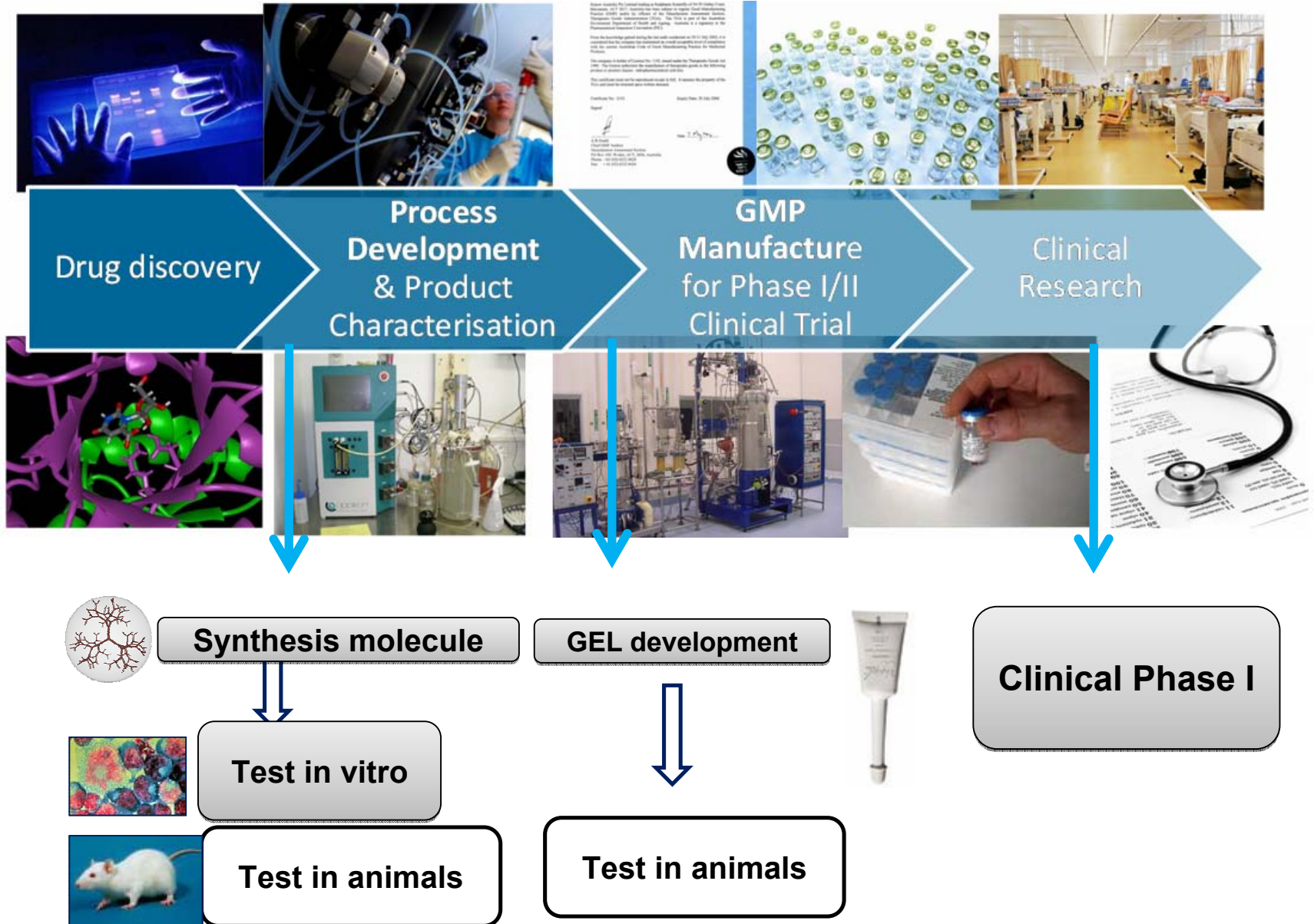
Product	Indication	Regulatory Path	Market Potential USD
VivaGel® Standalone Gel	STI (HIV/HSV2) Prevention Phase 2	Drug	\$3-6Bi <sup>1</sup>
VivaGel® Standalone Gel	BV treatment (& prevention) Phase 2	Drug	\$300-350M <sup>1</sup>
VivaGel® Coated Condom	Antiviral coating	Drug/Device combination	\$1.1 - \$1.7 Bi <sup>1</sup>

1: World Bank; UNAIDS; EC AIDS survey; various analyst and microbicide publications; industry reports and accounts of industry participants; SSL Annual Reports and condom industry data

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- Other applications for microbicide have a big market also:
  - With condoms
  - Bacterial Vaginosis treatment

# d) Current Status of development



## e) IPR protection

Degree of protection the product is very high:

- Intellectual property reviewed (PONS, Patent and Trademark Office) → solid foundation for Ambiox
- Analysis FTO (Freedom To Operate) and a study of "prior art" by Clarke & Modet
- Two patents filed in 2010 and PCT filed in March 2011.



## f) Pitfalls & Risks

- Existence of microbicides in clinical development as
- Possible development of a vaccine against HIV
- Non-acceptance by women or men who start using the first microbicides to reach the market

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3.

# AVAILABILITY FOR COOPERATION



# Business Model

## Ultimate Goal

**Global distribution of  
microbicide**

### **Asociation with big pharma**

- To develop the product in clinical phases, approval, launch, commercialization and distribution
- Business Model usual in this sector

# How do we reach our goal?

- HIV prevention is at the same time a human need and a business opportunity

- **Patents, Intellectual Property:** biotech lawyers specialized, PONS.
- Contacts with spanish and international investors showing interest in the project: **current negotiations with pharmaceutical company**
- **Public funding, european projects**
- **Colaborations agreed for clinical studies**



# Profitability

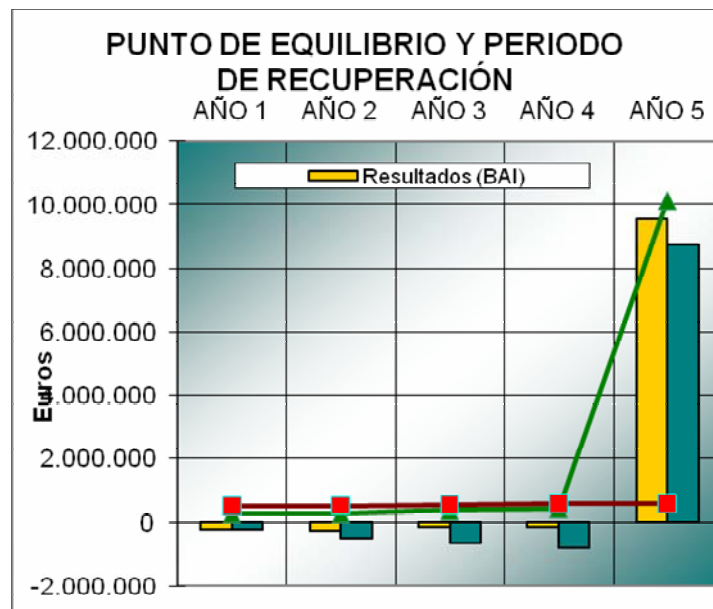
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- Income → licencing patent in clinical phase I → pharmaceutical company → milestones and royalties
- License agreement: Range 10-50 millions € year 5
- Ongoing payments when milestones are achieved in development of microbicide

# Investment? 1-3M€

Who is our ideal investor?

- Biotech investors with experience in the sector
- Pharma investor, brings also capacities complementary to biotech: production, clinical development, marketing, distribution



<b>VAN</b>	6.217.735
<b>TIR</b>	67%
Nº de veces que TIR multiplica la tasa de descuento	14,9

# Cooperative Approach with Pharma Industry

- The agreement through stages or milestones
  - Two options:
    - (1) Patent license to develop the **exclusive licensee** of the product.
    - (2) As **partners** seeking joint development of the product.



# Awards and Prizes

## We have been supported by:



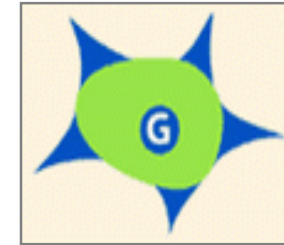
**Emprendedores magazine**  
**2ºFinalist Accesit**



**2009 Finalist Banespyme Orange**  
**IE Business School**



**Winners Entrepreneur Project category**  
**XV edition Bancaja Awards**



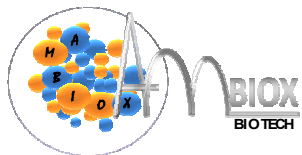
**Fundación Genoma España**  
**First prize to our Business Plan**  
**for starting a biotech company**



**2009 Finalist CIADE Award**  
**Universidad Autónoma Madrid**



**Third Prize**  
**Ideas competition UAH 2010**  
**to start-up a company**





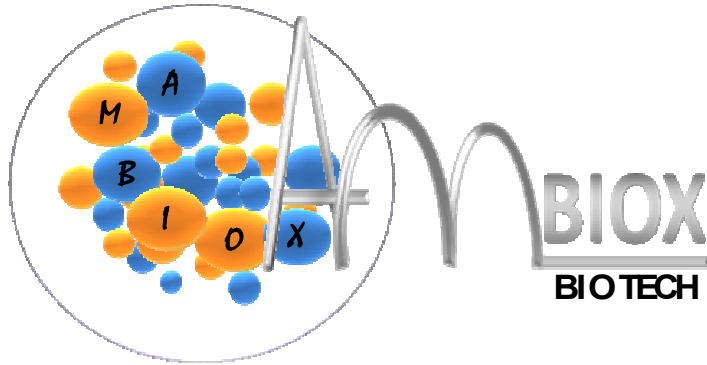
Ban Ki-moon, United Nations  
Secretary-General  
Excerpts from World AIDS Day  
message, December 2009

***“Investments in the AIDS response are  
producing results and saving lives.***

*We must continue doing what works, but we must  
also do more, on an urgent basis, to uphold our  
commitment to reach universal access to HIV  
prevention, treatment, care and support by 2010.*

*Let us use the AIDS response to generate progress  
towards the Millennium Development Goals.*

*Most of all, let us act now.”*



Gracias por su atención

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**Javier de la Mata**



**Rafael Gómez**



**Teresa Gonzalo**

