Programa Cooperación Farma-Biotech

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

Use of the immune-modulator Ruti® for prevention and treatment of seasonal rhinitis and asthma

ARCHIVEL farma

Madrid, 12 July 2011

CataloniaBIO Associació catalana d'empreses de biotecnologia

MEDICAMENTOS INNOVADORES Plataforma Tecnológica Española

GOBIERNO DE ESPAÑA MINISTERIO DE CIENCIA E INNOVACIÓN

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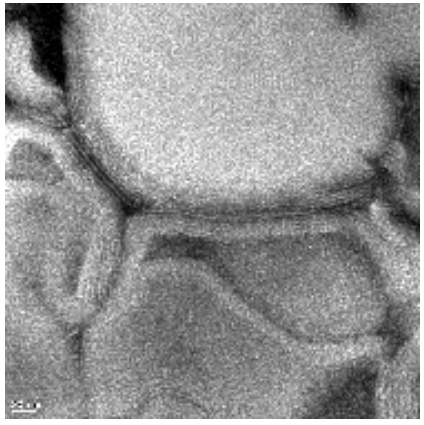
1. The Company

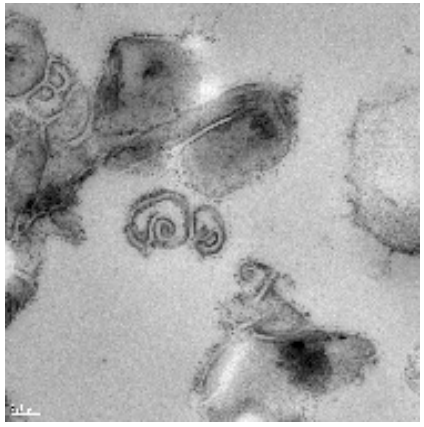
- Private Biopharmaceutical company, created in 2005
- Located in Badalona, at 10 km Barcelona, Catalonia, Spain
- Own production plant + R&D facilities + P3 lab 720 m^2
- STAFF: 17 employees + Chief Executive Officer + Chief Scientific Officer + Chief Operations Officer + Project Manager

2. The Product

RUTI®

- Invented at Institut Germans Trias I Pujol , ("Can Ruti")
- Made from *M. tuberculosis* grown under anoxic stress
- Non live: fragmented, detoxified and liposomed. SC route
- Poly antigenic
- Stable at room temperature
- CMC development finished: entering phase III





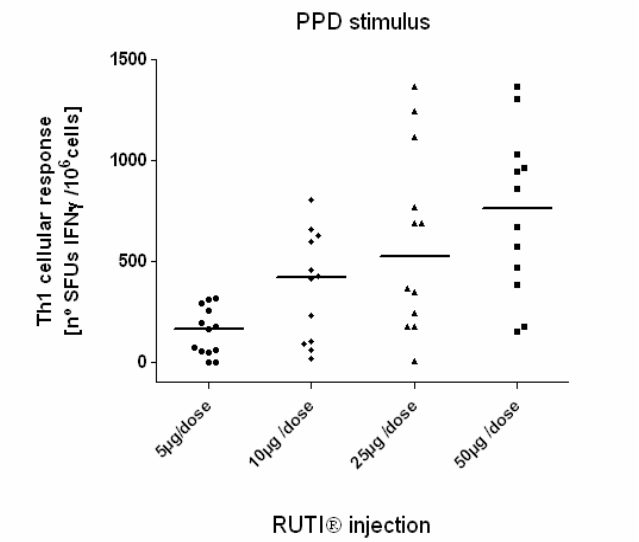
2. The Product: *Therapeutic focus*

- As therapeutic vaccine: prevention of active tuberculosis in individuals with non symptomatic infection (phase II completed, entering phase III)

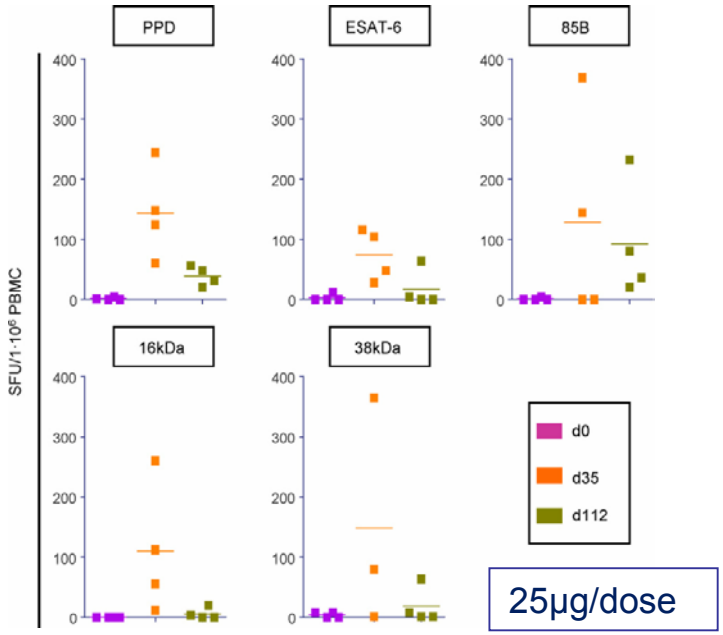
- **As immune-modulator: prevention and treatment of seasonal rhinitis and asthma ("seasonal vaccine" and symptomatic agent)**

2. The Product: *mechanism and safety*

Pre-clinical studies in mice



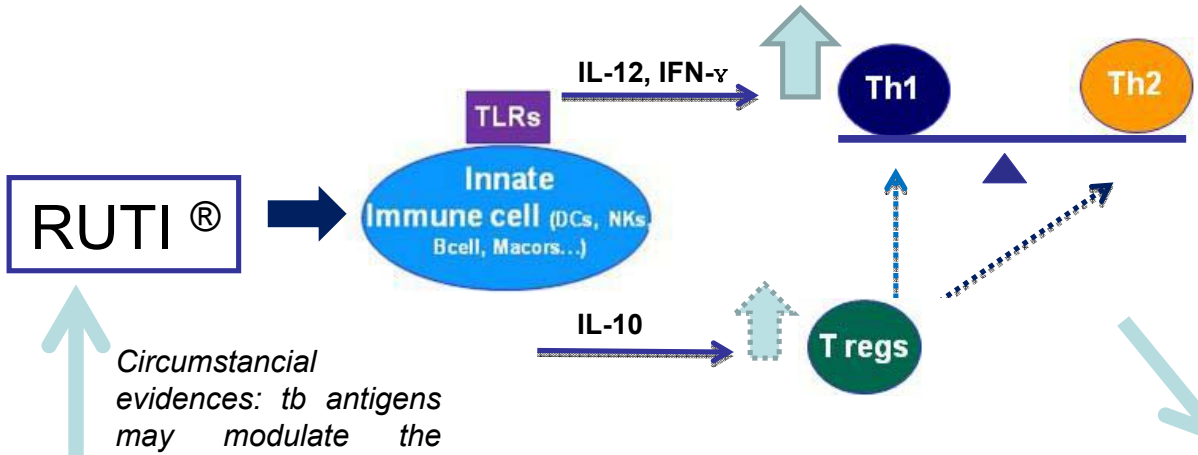
Clinical trial phase I



25µg/dose

RUTI ® has a good safety profile.
RUTI ® increases Th1 cellular response.

2. The Product: *innovative mechanism of action*



Circumstancial evidences: tb antigens may modulate the immune response related to allergies and asthma

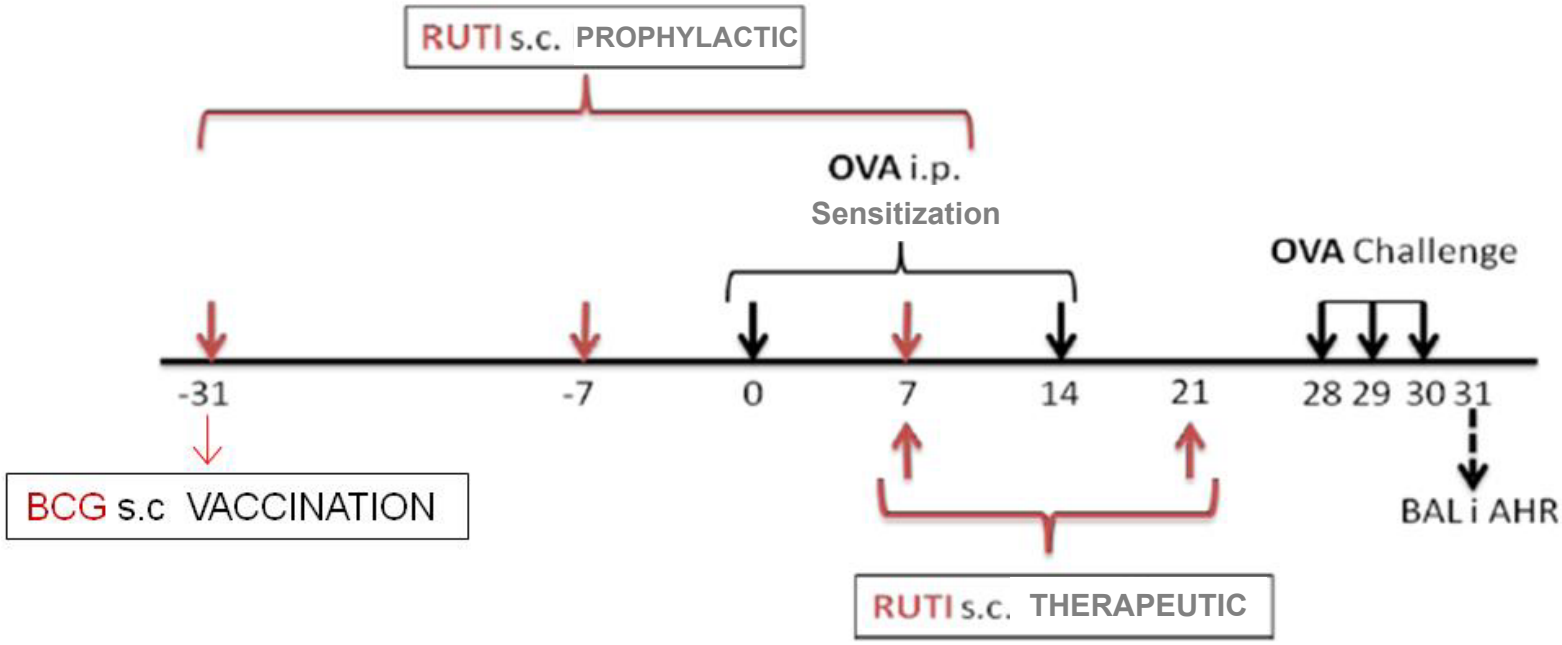
Previous evidences: "hygiene" hypothesis, BCG experience, Vaccae experience, CpG experience.

Improve allergic disease: RHINITIS & ASTHMA,

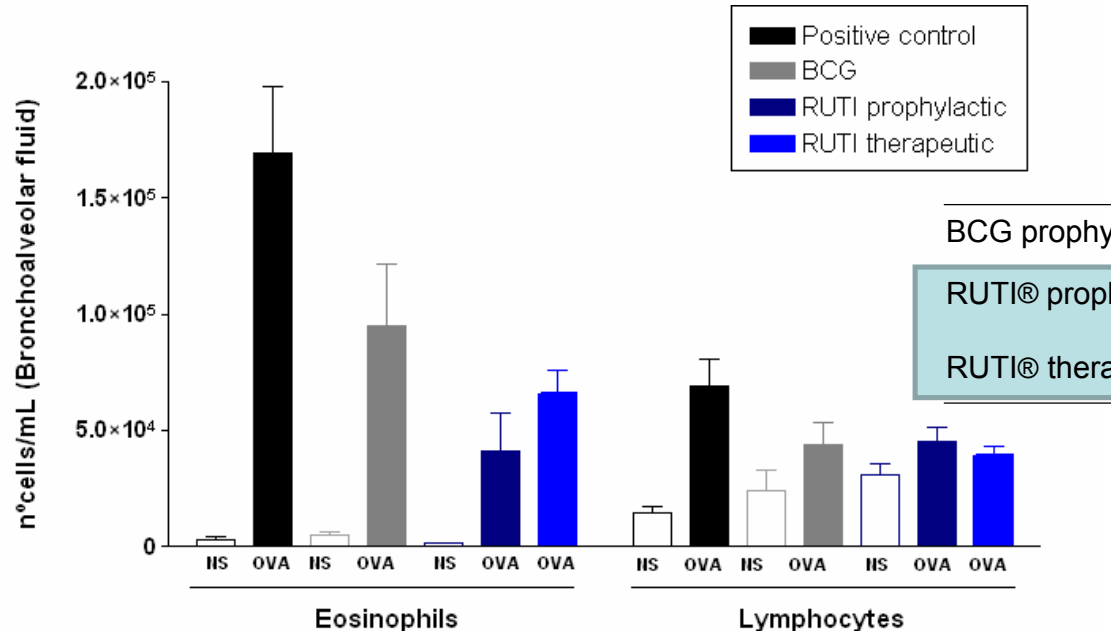
Proof of principle

Pre-clinical study in Ovalbumin-presensitized mice model

2. The Product: *preclinical proof of principle (design)*



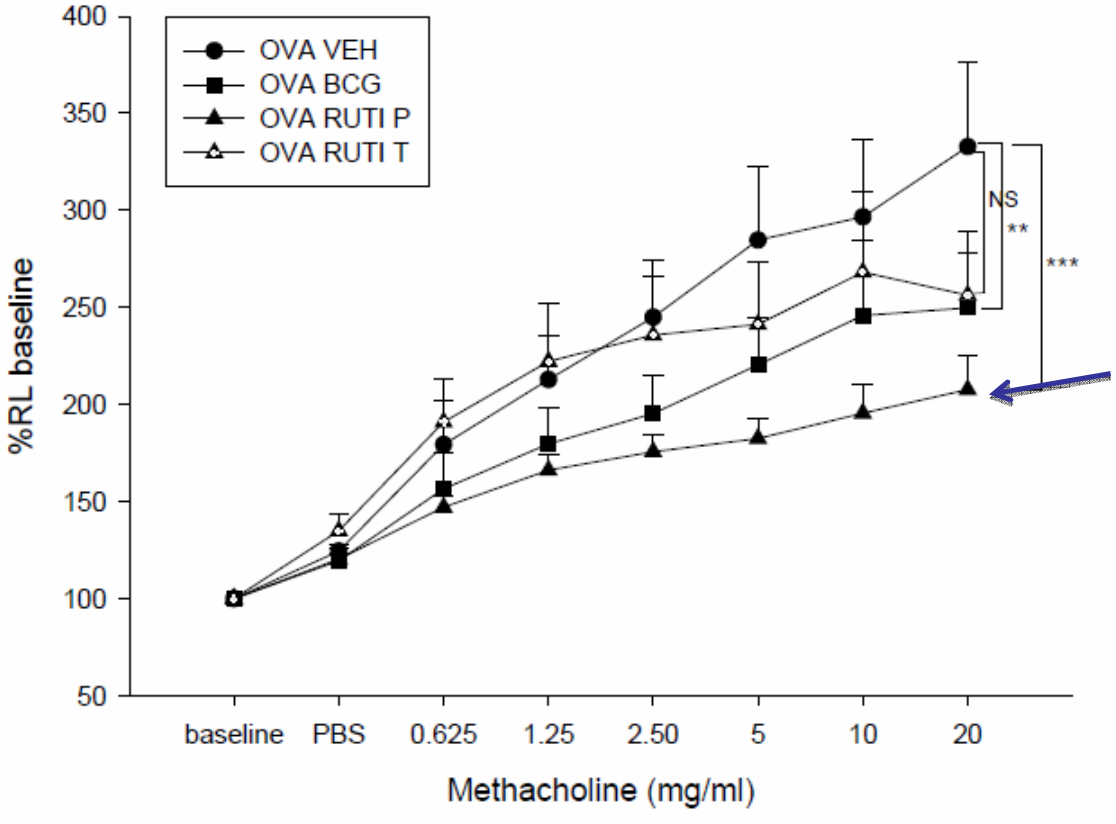
2. The Product: *Effect on BAL (cell infiltration in bronchoalveolar liquid)*



	eosinophil		lymphocites	
	Reduction	t-student test	Reduction	t-student test
BCG prophyl.	44 %	p= 0,08	63 %	NS
RUTI® prophyl.	76 %	**p= 0,005**	83 %	p= 0,09
RUTI® therap.	62 %	**p= 0,05**	72 %	**p= 0,05**

Eosinophilia infiltration of airway area is significantly suppressed with RUTI ® prophylactc and therapeutic treatment

2. The Product: *Effect on AHR (Airway Hypersensitivity Response)*



Prophylactic arm RUTI®

(100% efficacy with respect to control)

AHR is significantly suppressed with RUTI ® prophylactic treatment.

2. The Product: *conclusions on preclinical POP*

RUTI ® substancially reverses airway hyperreactivity and eosinophilia that is induced in ovalbumin-presensitized mice, both prophylactically and therapeutically

2. The Product: *Differential aspects*

TPP: one or two SC injections to reduce allergic rhinitis or asthma symptoms ("seasonal vaccine" and symptomatic treatment)

- Independent of allergen
- Potential to minimize use of corticoids, antihistaminics, antileukotriens, etc
- Safe and well tolerated (non systemic effects, mild effects on site of injection)
- Cost effective (ie: as compared with symptomatic medication)

2. The Product: *Current development status*

- CMC development completed and agreed with PEI
- Own manufacturing capability up to 9M doses / year
- Entering phase III in tb-related indication
- Strong preclinical POP in target indication
- Safe and well tolerated even in HIV+, tb infected individuals

2. The Product: *IPR*

- WO2005/042013 granted in most countries, process, product and therapeutic uses
- WO2008/053055 under accelerated review in EU, national phases worldwide, prophylactic use
- PCT/ES2009/000436, will enter national phases by Q1 2011, primary prophylactic use
- Patent application EP 11150072.4 covering composition of matter of phase III product filed January 4th 2011

2. The Product: *Risks, pitfalls, the opportunity*

- Lower than average process development risk
- Lower than average safety concern
- Higher than average mechanistic proof and circumstancial evidence of potential efficacy
- Proof of principle in the most astringent preclinical model available
- **Immediate availability for clinical proof of concept / phase II**

3. Availability for cooperation:

- Flexible approach to the indication. Archivel's expertise and priority is currently TB. Opportunity for codevelopment, option agreements, license.
- "Intelligent" financial support
- **Next step, proof of concept in humans: Seasonal allergic rhinitis for which primary cause is pollen (less than 1M€ and less than 1 year potential)**