

# XII Encuentro de Cooperación Farma-Biotech

Santiago de Compostela, 26 de septiembre de 2014

## OXALOACETATE DONORS AS NEW NEUROPROTECTIVE AGENTS



A FERRER  
& JANUS COMPANY

[www.spheriumbiomed.com](http://www.spheriumbiomed.com)

## Content

### 1. The Company and the current pipeline

### 2. The Product

- Target Indications
- Innovative aspects
- Current status of development: proof of concept and trials already performed
- Differential features facing the market and business opportunities
- IPR protection
- Pitfalls & Risks to be considered

### 3. Looking for...

# VII Encuentro de Cooperación Farma-Biotech

Company

Company

Mission

Unmet  
need

Process

Portfolio

To turn basic **biomedical knowledge** into  
**social**  
**and economic value**

Company

## To turn results into products

Mission



Unmet  
need

- **Key experiments**
- **Efficient Project Management**



Process

### **Scientific results**

- Oriented to knowledge
- Preliminary IP
- No data on production feasibility
- No regulatory definition

Portfolio

### **Selling package**

- Oriented to market (defined TPP)
- IP strategic development
- Manufacturing feasibility
- Defined regulatory path.

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Company



- Biomedical projects:
  - No specific indication required
  - Vet and human health
  - Therapeutics, diagnostics and medical devices

Mission

Unmet  
need

Approach



- Innovative technology capable to reach the next value milestone:
  - SCOPE: covering a de-risking step
  - COST: with an investment around 300-500 k€
  - TIME: in a 1 – 2 years framework

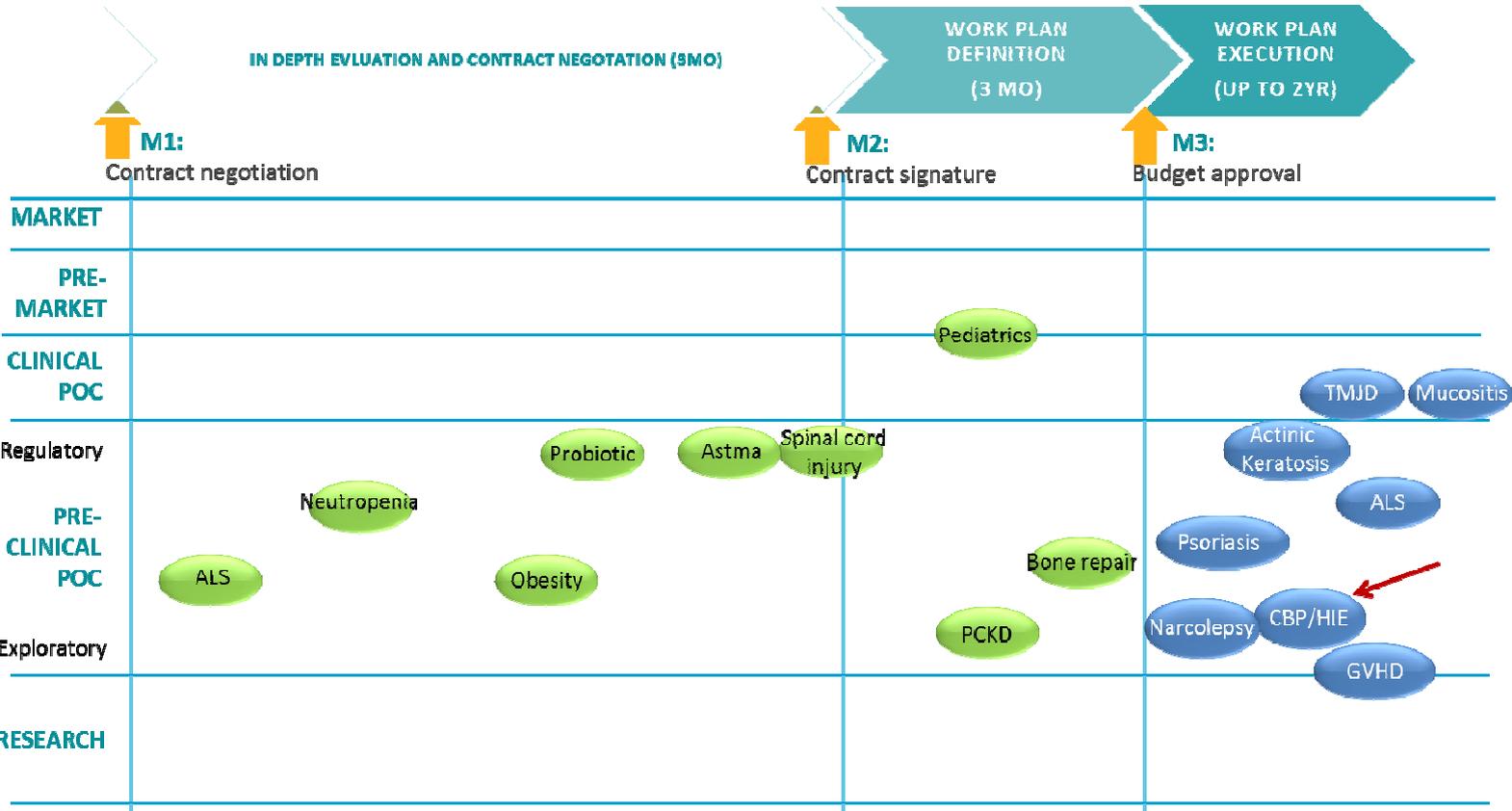
Portfolio



- Typical key value inflection points are
  - Confirmation of competitive preclinical candidate
  - Confirmation of clinical candidate
  - Confirmation of Phase IIB ready candidate

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**Company**  
**Mission**  
**Unmet need**  
**Approach**  
**Portfolio**



CB1

Canviaria CBP per CPB Cardio Pulmonary Bypass

Montserrat Cano Biosca; 23/09/2014

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**Product**

### Product

# Neuroprotection for HIE

(Hypoxia-Ischaemic Encephalopathy)

### Indication

#### > Etiology

Evolving process initiated by the hypoxic-ischaemic insult leading to decreased blood flow to the brain (**primary lesions**), followed by the reperfusion injury (**secondary lesions**).

### Innovative Aspects

#### > Incidence

Niche indication. Prevalence: 0.01%, <10/100.000(from orphanet).

### Current status

#### > Current Treatment

Therapeutic hypothermia by a **cooling cap**, only partially effective, with almost 50% of treated infants having adverse outcomes.

### Business Opportunities

#### > Unmet need

**No pharmacological treatment available.**

### IP Protection

# Neuroprotection for CPB

(Cardio-Pulmonary Bypass)

### Product

### Indication

#### > Etiology

2 types of perioperative cerebral injury after CPB: Type I, **stroke** (1-5% incidence); Type II, Postoperative **cognitive decline** (POCD, 50% incidence).

### Innovative Aspects

Cognitive dysfunction is less devastating but the 20-fold greater incidence is **far more significant** in terms of impact on quality of life and overall healthcare resource utilization.

### Current status

#### > Incidence

80% of all cardiac surgery is conducted with CPB: 1,2M patients ww.

### Business Opportunities

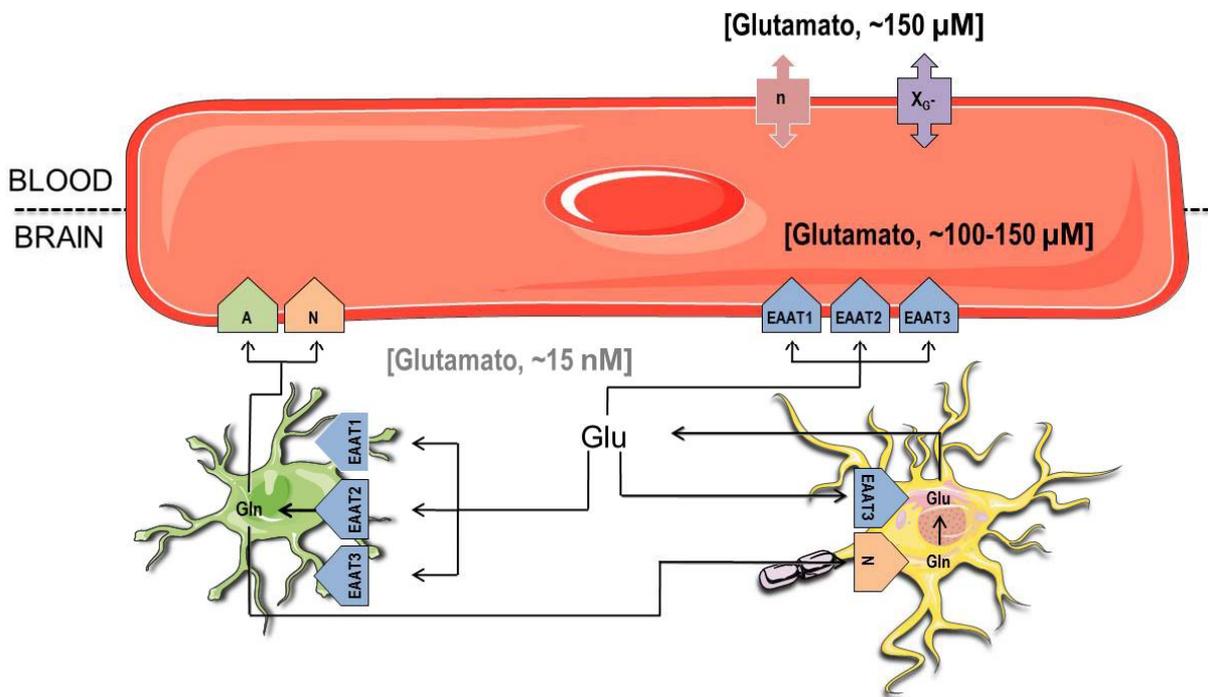
#### > Unmet need

**No pharmacological treatment available** for long-term neuroprotection to reduced incidence of POCD.

### IP Protection

# First in class mechanism of action

(BLOOD target to lower BLOOD glutamate to lower BRAIN excess glutamate, the bad guy)



Teichberg VI et al. Neuroscience 2009; 158:301-308.

Product

Indication

Innovative Aspects

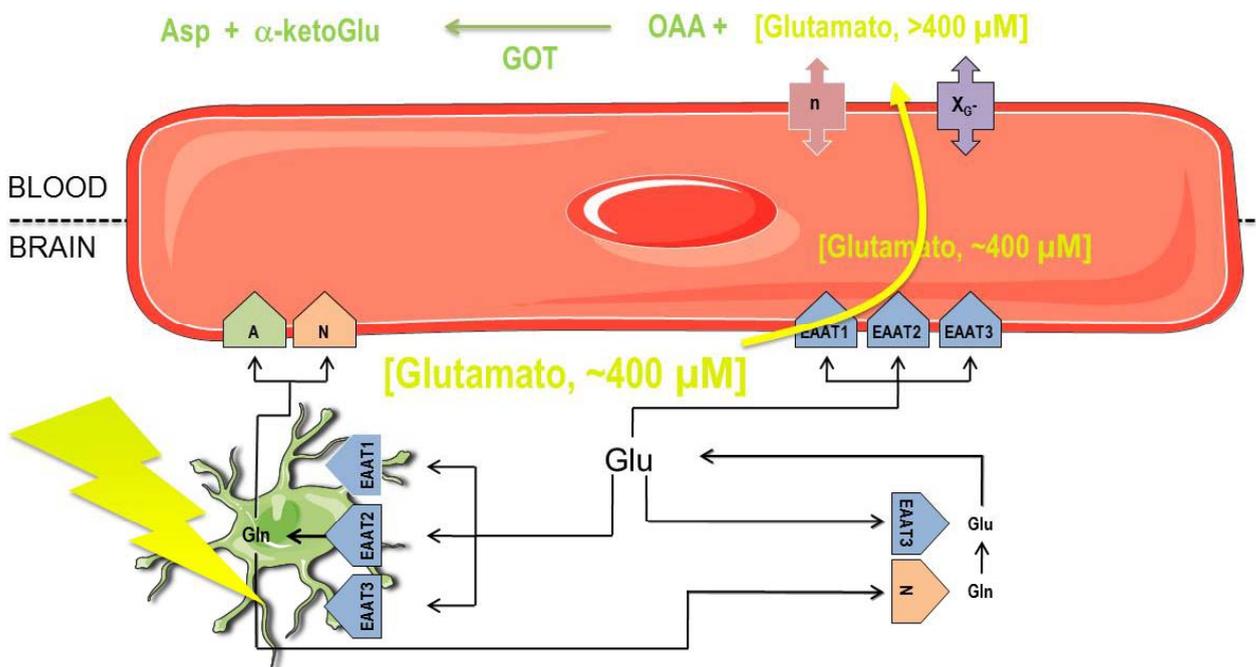
Current status

Business Opportunities

IP Protection

# First in class mechanism of action

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Product

Indication

Innovative Aspects

Current status

Business Opportunities

IP Protection

## VII Encuentro de Cooperación Farma-Biotech

### Product

### Indication

- Extensive exploratory, mechanistic evidence to support neuroprotection upon ischemic insults (*Castillo, Teichberg...*)

### Innovative Aspects

- Clinical evidence of correlation between high blood glutamate and bad outcomes (stroke, Cardiopulmonary Bypass)

### Current status

- TPP defined in two indications: HIE and CPB

### Business Opportunities

- Preclinical models in the two indications available, protocols and providers well defined and ready

### IP Protection

- Final pharmaceutical form of OAA donor not available yet (**project bottleneck**)

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### Product

- HIE is an orphan pediatric, underserved indication with <10/100.000 cases per year.

### Indication

- Postoperative Cognitive Decline after CPB is a well recognized unmet need, affecting up to 50% of patients (out of 1,2M annual interventions)

### Innovative Aspects

- The target population is well controlled and amenable to preventive administration

### Current status

- The product has potential in stroke (will make sense after niche indications)

### Business Opportunities

- Market access should not be a problem (unmet need, acute administration, huge economic impact)

### IP Protection

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**Product**

**Indication**

- International patent application under review in US and EU (method and dose protection)

**Innovative Aspects**

- Regulatory barriers: data protection, Orphan Drug status

**Current status**

- Further IP down the road: pharmaceutical composition

**Business Opportunities**

**IP Protection**

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Looking  
for ...

## VII Encuentro de Cooperación Farma-Biotech

### Looking for ...

- Partner after de-risking (preclinical GMP-like candidate product supported by specific animal model POC)
- Option agreement acceptable during de-risking phase