

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

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

Portfolio

Scientific results

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

Selling package

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

VII Encuentro de Cooperación Farma-Biotech

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

VII Encuentro de Cooperación Farma-Biotech

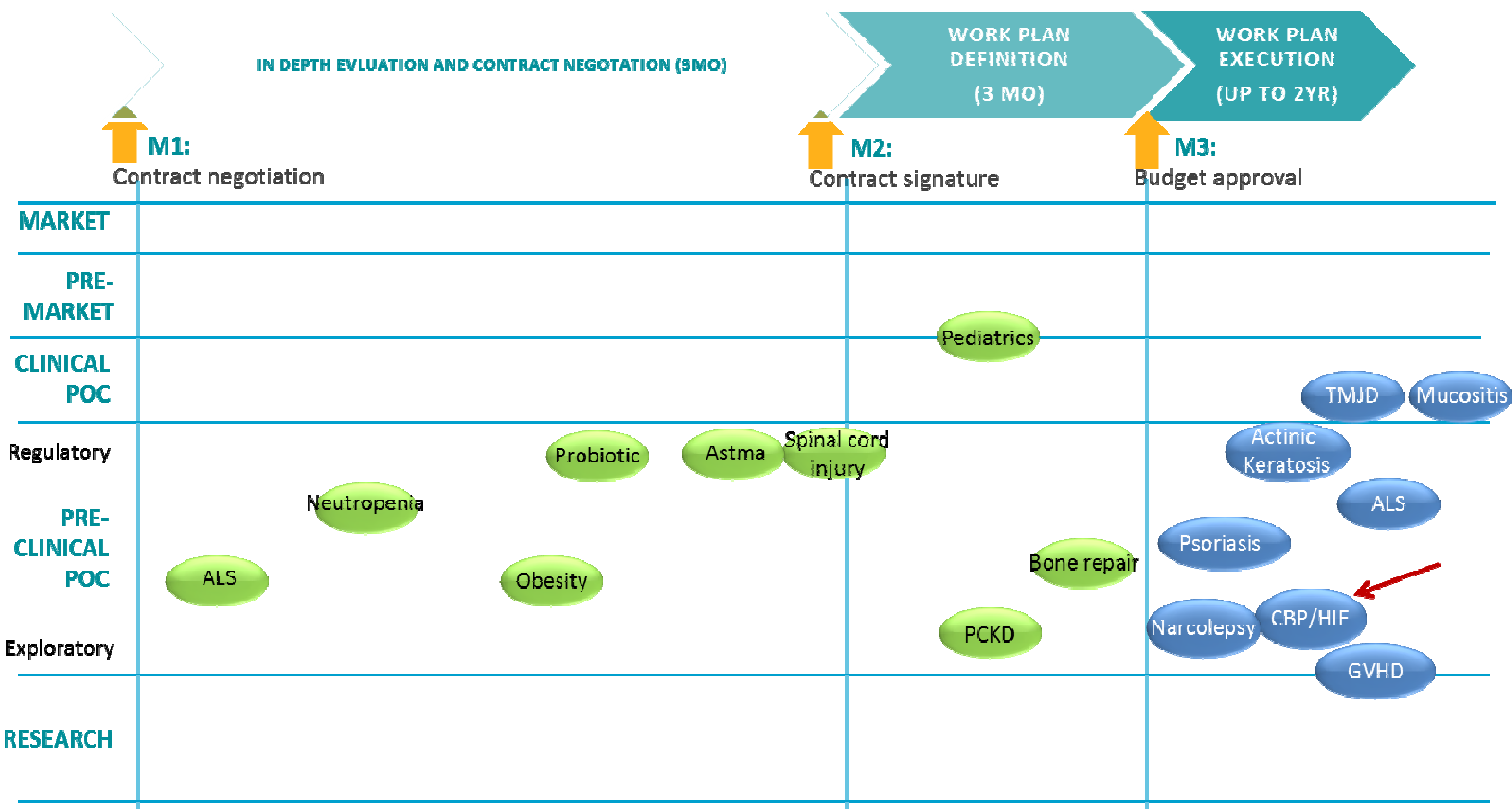
Company

Mission

Unmet need

Approach

Portfolio



CB1

Canviaria CBP per CPB Cardio Pulmonary Bypass

Montserrat Cano Biosca; 23/09/2014

VII Encuentro de Cooperación Farma-Biotech

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

Product

Neuroprotection for CPB

(Cardio-Pulmonary Bypass)

Indication

> Etiology

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

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.

> Unmet need

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

Business Opportunities

IP Protection

Product

Indication

Innovative
Aspects

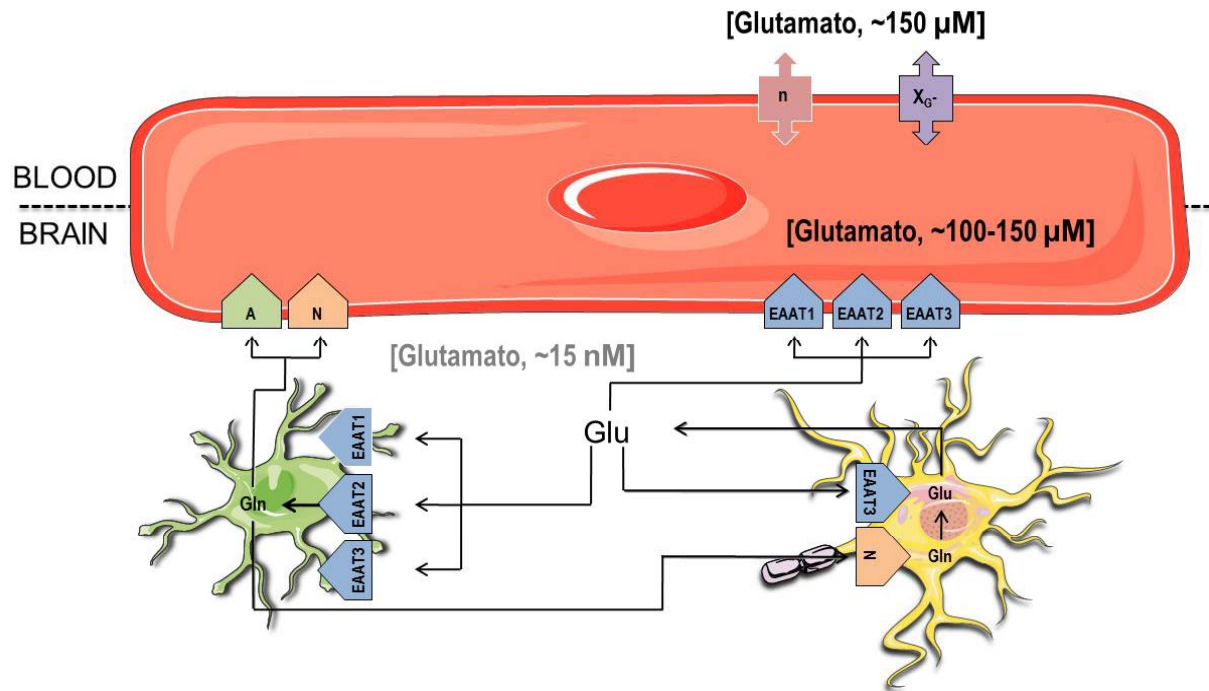
Current
status

Business
Opportunities

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

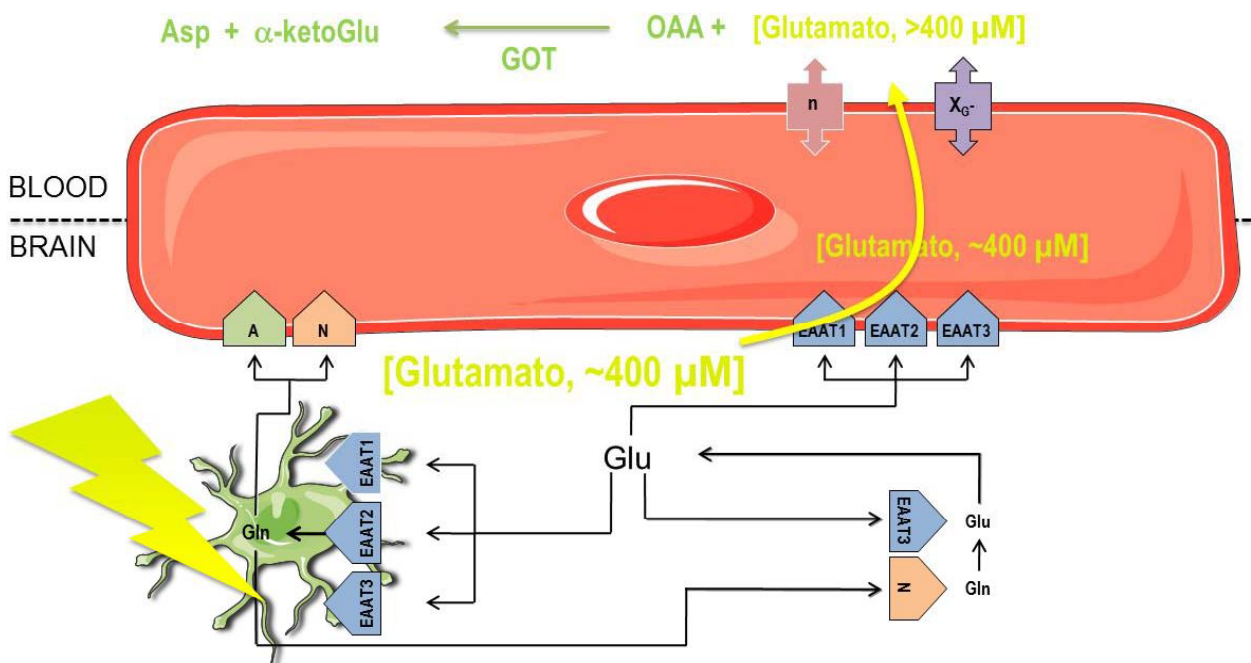
Current status

Business Opportunities

IP Protection

First in class mechanism of action

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



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

VII Encuentro de Cooperación Farma-Biotech

Product

Indication

Innovative Aspects

Current status

Business Opportunities

IP Protection

- Extensive exploratory, mechanistic evidence to support neuroprotection upon ischemic insults (*Castillo, Teichberg...*)
- Clinical evidence of correlation between high blood glutamate and bad outcomes (stroke, Cardiopulmonary Bypass)
- TPP defined in two indications: HIE and CPB
- Preclinical models in the two indications available, protocols and providers well defined and ready
- Final pharmaceutical form of OAA donor not available yet (**project bottleneck**)

VII Encuentro de Cooperación Farma-Biotech

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

VII Encuentro de Cooperación Farma-Biotech

Product

Indication

Innovative Aspects

Current status

Business Opportunities

IP Protection

- International patent application under review in US and EU (method and dose protection)
- Regulatory barriers: data protection, Orphan Drug status
- Further IP down the road: pharmaceutical composition

VII Encuentro de Cooperación Farma-Biotech

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