Programa Cooperación Farma-Biotech
9º encuentro (4 de julio de 2013)

BN201: a paradigm-shift (neuroprotection) in the treatment of neurodegenerative diseases

bionure
Biomedicine heading to future

Barcelona, 4 de julio de 2013

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1) The company: Origins

Bionure is an early-stage drug development company founded in 2009:

- Spin-Out from IDIBAPS (Hospital Clínic de Barcelona) and CSIC.
- Based in Barcelona and San Jose, California.
- Virtual company. Strong expertise in Project Management and Business Development.
- Aimed at developing new therapies for neurodegenerative diseases with special focus in Multiple Sclerosis and Glaucoma through an innovative approach: neuroprotection.
- Bionure has raised 3-4M € in public and private funding.

1) The company: Management & Adv. Board

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Founders & Management Team

Albert G. Zamora, CEO
Co-founder, Chairman and CEO of Bionure
MBA EADA Business School
Director of Innovation, Fundació Clínic

Pablo Villoslada, CSO
Co-founder and CSO of Bionure
MD, PhD in Neuroimmunology
Director Neuroimmunology IDIBAPS

Advisory Board

Joaquim Trias
Top-name in American biotech
>20 years experience Silicon Valley

Larry Steinman
Professor Neurology, Stanford
Co-inventor Natalizumab (Tysabri®)

Craig Smith
Ex-Clinical Science Unit Head, Ophthalmology and eHealth Global Strategic Lead, Novartis.

Stephen L. Hauser
Professor Neurology, UCSF. Rituxan for MS
Presidential Bioethics Commission by Obama

Joaquin Uriach
Grupo Uriach, pharma company founded in 1838

Juan Bigorra
Innovation Director, Hospital Clínic Barcelona

Michelle Messmer
Director of Customer & Healthcare Programs in Italian MS Society.

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2) The product: Overview

BN201

- Small molecule/Peptoid
- Targeting Neuroprotection
- First-in-Class: novel Mechanism of Action
- Development Status: Preclinical; IND completed Q4 2013

2) The product: Target indications & Strategy

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The aim of Bionure is to develop neuroprotective drugs by targeting the neurotrophin pathway and its receptors

Multiple Sclerosis, MS

Neurological/Inflammatory disease

Myelin damage and axonal loss

Therapies target inflammation

Glaucoma, GL

Neurological/Ophthalmologic disease

Retinal Ganglion Cell (neurons) loss

Therapies target intraocular pressure

Acute Optic Neuritis, AON

Inflammatory disease of the optic nerve, highly related to multiple sclerosis

BN201 for acute, i.v administration: reduces costs and timings, opportunity for ODD

Strategy to obtain PoC of Neuroprotection in humans asap

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2) The product: Target indications & Strategy

To develop in niche

1. Intravenous administration for Acute Optic Neuritis
2. Gain exception from Orphan Drug Exclusivity
3. Clinical PoC of Neuroprotection in humans (PhIIa) in 2016
4. After that, BN201 will be extended to bigger indications (i.e. Multiple Sclerosis and Glaucoma)

2) The product: MoA

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MS marketed drugs – Immunomodulators	GL marketed drugs – IOP-lowering
Only target inflammation	Only target intraocular pressure
No neuroprotection	No neuroprotection
Half of patients not treated	Half of patients not treated

BN201 exerts its neuroprotective function targeting the multiple neurotrophin pathway and its receptors

- Targets brain damage and neurodegeneration
- Has shown to prevent and protect the brain and neurons from damage
- Crosses the Blood-Brain Barrier and reaches the CNS
- Is potentially effective for all the patients (i.e. progressive forms in MS, low/normal-IOP in GL)

2) The product: Market differentiation

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Marketed drugs and the majority of drugs in development for MS are immunomodulators.
In the AON market, there are not satisfactory treatments for patients.
Drugs in the market and in development for GL are basically IOP-lowering drugs.

Current therapies	Bionure
Only target inflammation (MS)/intraocular pressure (GL)	Targeting neuroprotection
MS: Only benefiting patients in the early to medium phase of the disease & RRMS patients (not progressive) In GL: Only benefiting patients with high-IOP	Benefiting patients in all stages (including progressive forms in MS and not high IOP patients in glaucoma)
Do not prevent brain damage	Preventing brain damage
Do not prevent neurodegeneration	Preventing neurodegeneration
Limitation for combination therapy due to side/effects	Suitable for combination therapy

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2) The product: Science & Development

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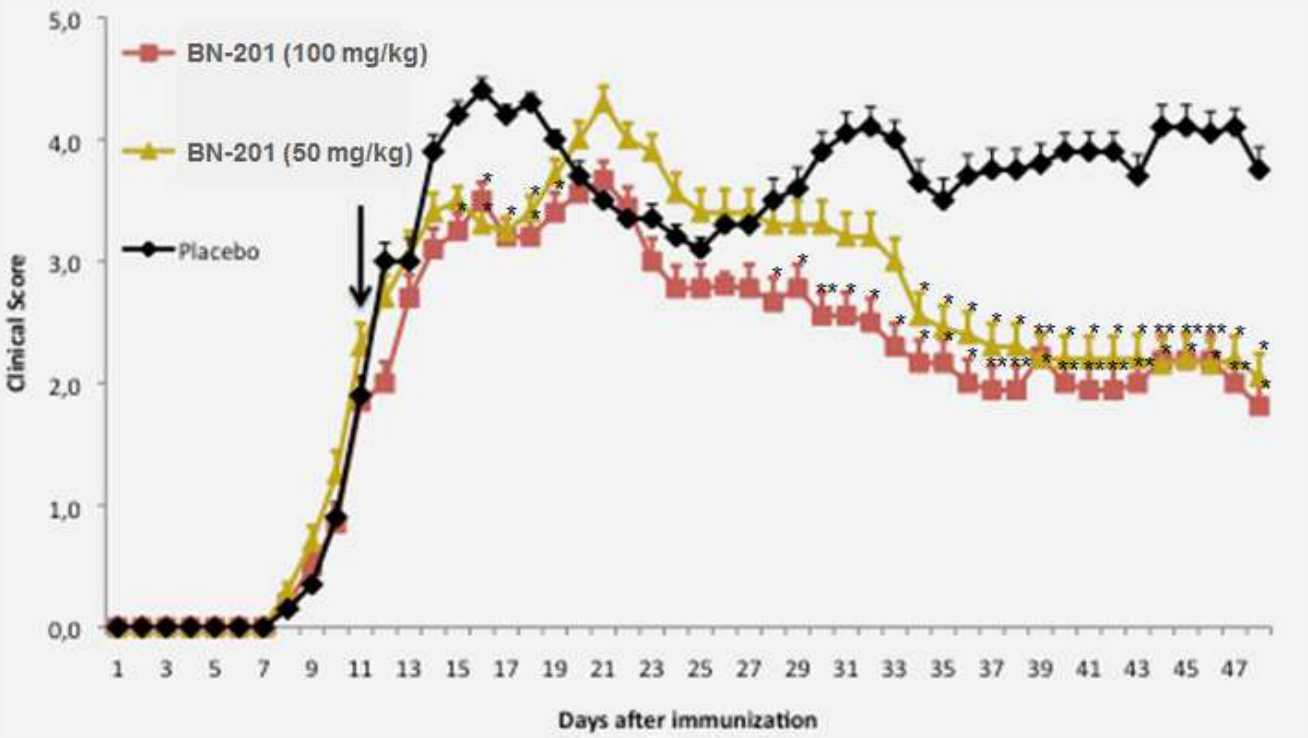
Effect of BN201 in the EAE curative model



BN201 delays the onset of the relapse and ameliorates the clinical course of animals suffering EAE

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2) The product: Science & Development

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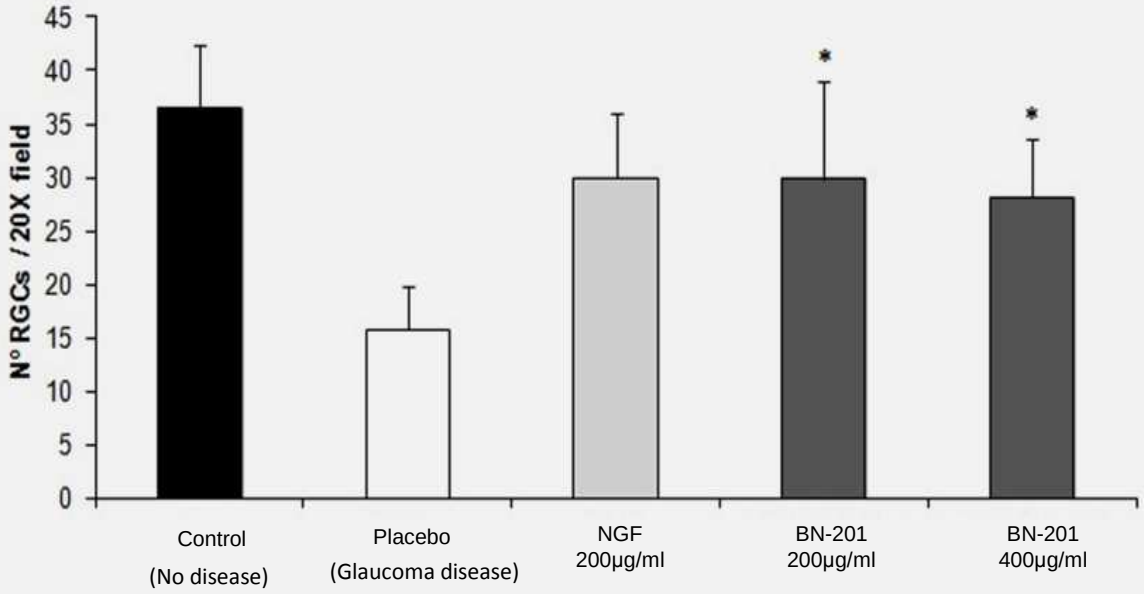
Efficacy of BN201 in the animal model of glaucoma



BN201 eye drops protected neurons (RGC) from death in the animal model of glaucoma to a similar extent than NGF

2) The product: Science & Development

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After having obtained good results in Efficacy, Tox & Safety and Blood-Brain Barrier crossing, preclinical regulatory studies are ongoing (to be completed by late 2013)

DEVELOPMENT PLAN	Status
Preclinical Package	***Ongoing***
ADME studies	*Ongoing*
Toxicological studies in rats & dogs	*Ongoing*
Genotoxicity (2 studies)	*Ongoing*
Safety Pharmacology (CV, CNS, Respiratory)	*Ongoing*
The preclinical package is expected to be completed by Q4 2013	

IND submission	***Q4 2013***

Clinical Trials	*Planned to start by Q1 '14*
A Phase I study (SAD+MAD) planned	*First Patient In (FPI) Q1 '14*

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2) The product: IP protection

- 1 patent filed by the academic institutions (IDIBAPS and CSIC) was presented to the European Patent Office in August 2009, and was then licensed to Bionure
  - "New peptoids agonists of nerve growth factor and their use as medicaments". Priority date: August 31 2009. – Extension in August 2010.
- In August 2010, Bionure filed a new patent covering 3 new peptoids (G79*, G80, G81) and several indications for brain and retina diseases in the US. Currently in national phases.

Bionure trademark was registered by March 29th 2010 #1036701 for 10 years in the US and Europe.

**BN201 was formerly named G79*

2) Risk and Financials to reach milestones

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The main risk would be the lack of capital that would allow Bionure to finance the clinical phase development.

Bionure has opened a 7M € series A round of equity funding:

- 2M€ to complete IND by 2013 for AON, possible Orphan Drug Designation.
- 5M€ to complete Phase IIa of Neuroprotection in AON (BN201 i.v. and acute intervention)

After demonstrating neuroprotection in humans, we will extend to bigger indications: MS (oral) and Glaucoma (topic)

3) Partnering Opportunities

Bionure is open and flexible to a wide range of options to collaborate with Pharma: direct investment, co-development and risk-sharing approaches, licensing agreement, etc.

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