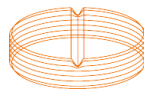


XII Encuentro de Cooperación Farma-Biotech

Santiago de Compostela, 26 de septiembre de 2014

Neuroprotective BN201 for multiple sclerosis and orphan CNS indications

bionure
Biomedicine heading to future



MEDICAMENTOS INNOVADORES
Plataforma Tecnológica Española

biospain
2014

farmaindustria

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

1. Early stage drug development co.

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1. Early stage drug development co.

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 **MEDICAMENTOS INNOVADORES**
Plataforma Tecnológica Española

bionure
Biomedicine heading to future

farmaindustria

1. Early stage drug development co.

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2010

2009

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1. Early stage drug development co.

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2010

2009



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- ▶ Small molecule
- ▶ NCE + First-in-Class
- ▶ Orphan Neuro-ophthalmology

2.1 Optic Neuritis

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Farma-Biotech

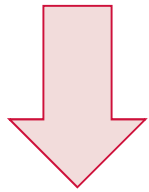
Inflammatory disease of the optic nerve

5/100,000

2.1 Optic Neuritis

Inflammatory disease of the optic nerve

5/100,000

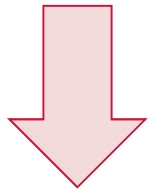


40,000
patients/yr

2.1 Optic Neuritis

Inflammatory disease of the optic nerve

5/100,000



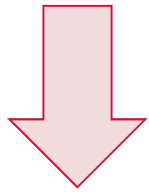
40,000
patients/yr

IV corticos

2.1 Optic Neuritis

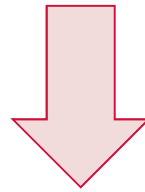
Inflammatory disease of the optic nerve

5/100,000



40,000
patients/yr

IV corticos

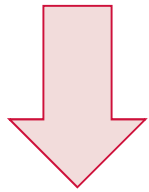


No improvement
visual outcome

2.1 Optic Neuritis

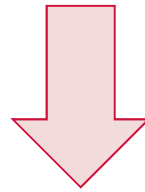
Inflammatory disease of the optic nerve

5/100,000



40,000
patients/yr

IV corticos



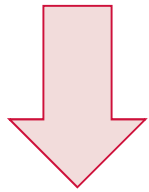
No improvement
visual outcome

Axon/Myelin

2.1 Optic Neuritis

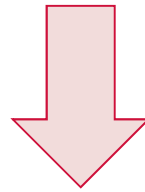
Inflammatory disease of the optic nerve

5/100,000



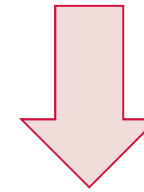
40,000
patients/yr

IV corticos



No improvement
visual outcome

Axon/Myelin



Pain
Vision loss

2.1 Optic Neuritis

Inflammatory disease of the **optic nerve**

5/100,000



40,000
patients/yr

IV



No improvement
visual outcome

Axon/Myelin



Pain
Vision loss

2.1 Optic Neuritis

Inflammatory disease of the optic nerve

5/100,000



40,000
patients/yr

IV



No improvement
visual outcome

Axon/Myelin

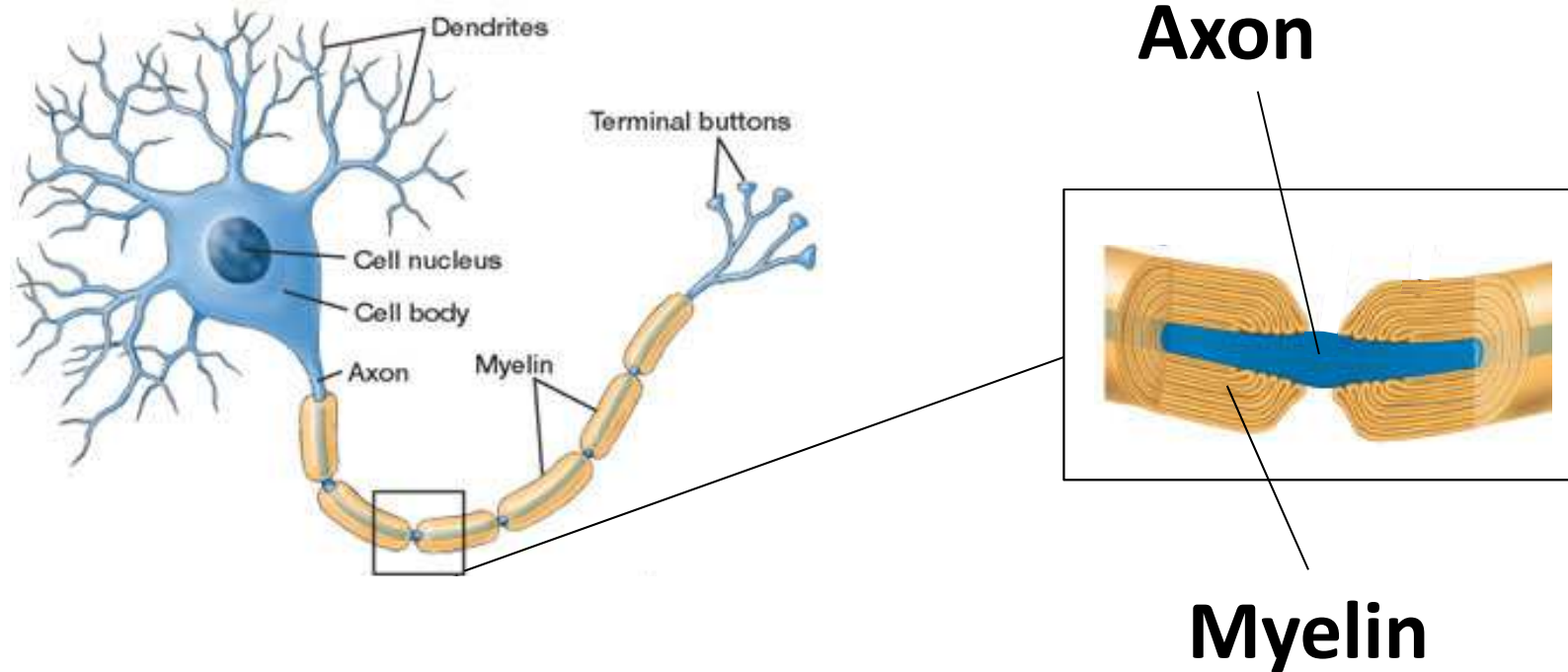


Pain
Vision loss

Also focused in Neuromyelitis Optica

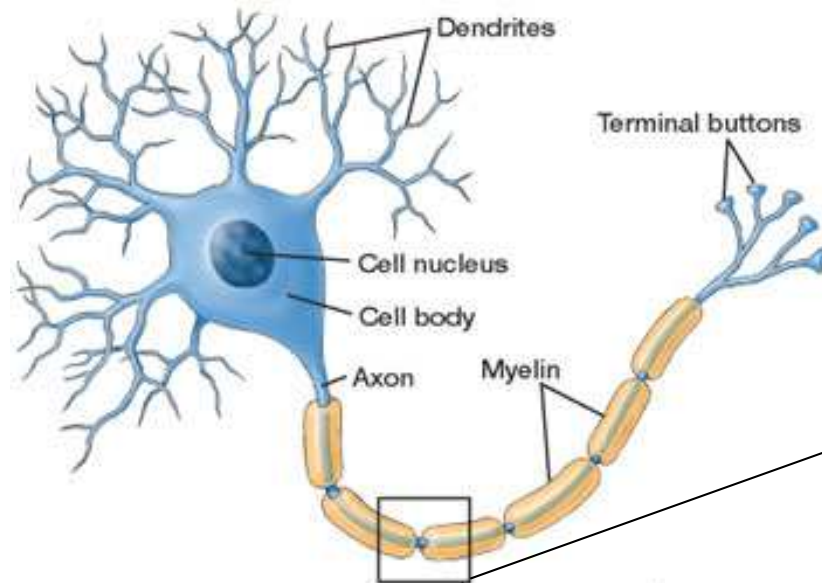
2.2 BN201: small molecule, NCE, FiC

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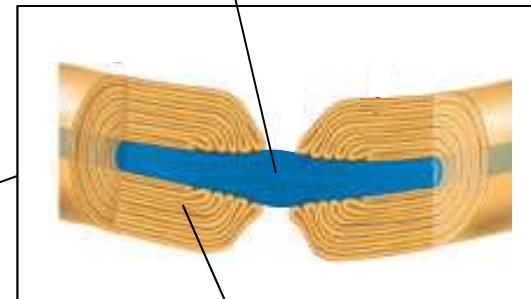


2.2 BN201: small molecule, NCE, FiC

XII Encuentro
Farma-Biotech



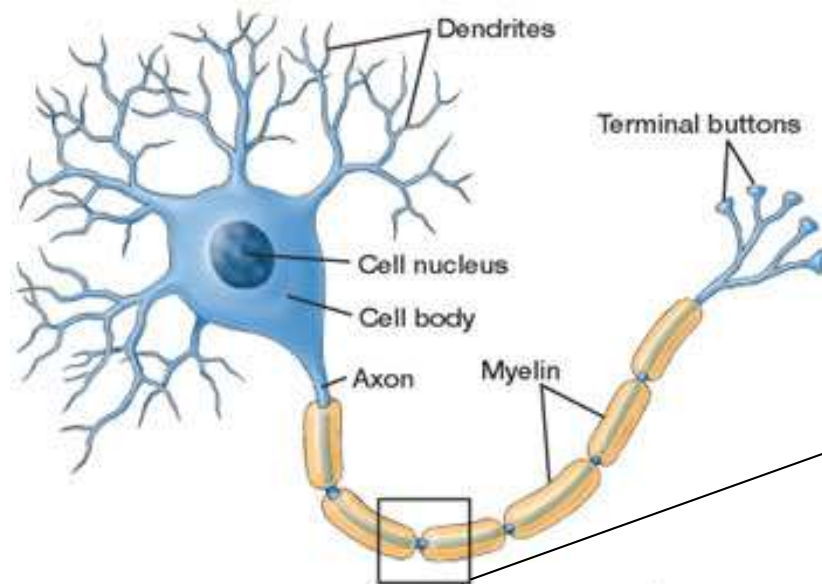
Neuroprotection



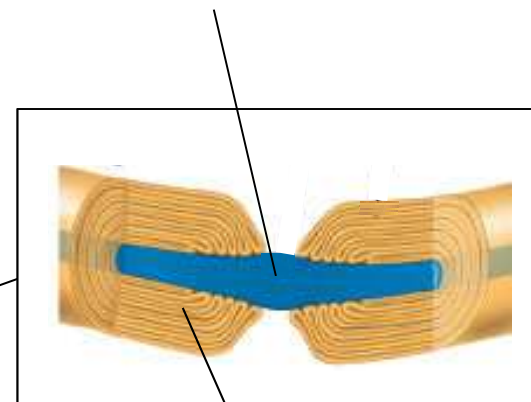
Remyelination

2.2 BN201: small molecule, NCE, FiC

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Neuroprotection



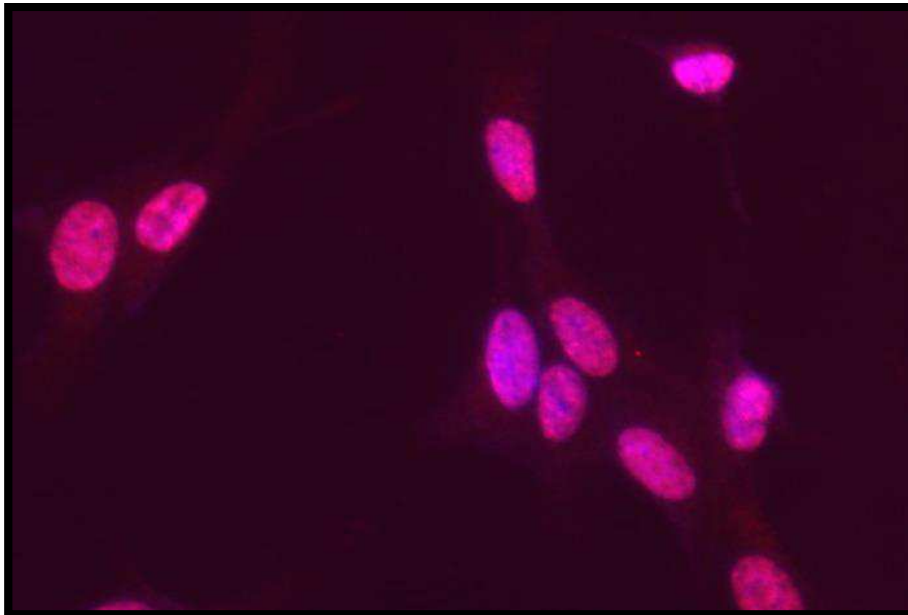
Remyelination

- ▶ Novel MOA: SGK agonist (FOXO3) -> cell survival

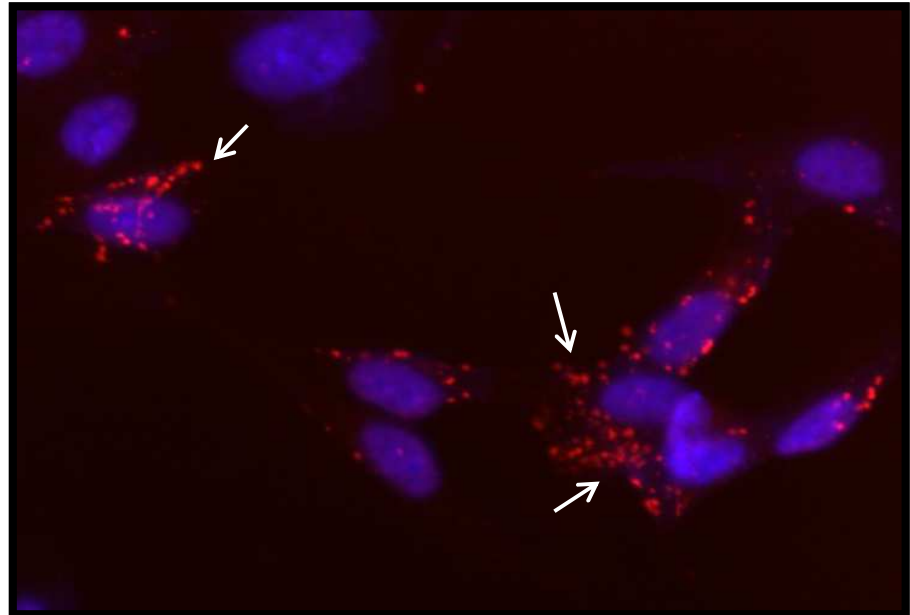
2.3 BN201: novel MOA

SGK agonist -> FOXO3 translocation

No treatment 40X



BN201 2 μ M (15') 40X



FOXO-3 staining by immunofluorescence in SH-SY5Y after BN201 stimulation

2.4 Why Optic Neuritis?

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Acute

2.4 Why Optic Neuritis?

XII Encuentro
Farma-Biotech

Acute



2.4 Why Optic Neuritis?

Acute



Eye

**“In God we trust.
All others must
bring data”.**

W. Edwards Deming



2.4 Why Optic Neuritis?

Acute



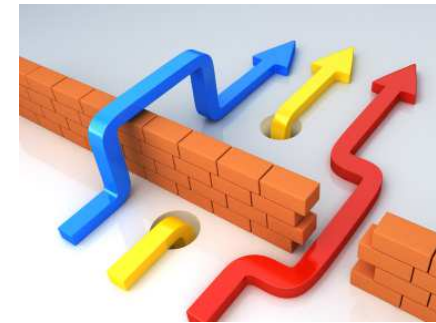
Eye

**“In God we trust.
All others must
bring data”.**

W. Edwards Deming



Rare



2.4 Why Optic Neuritis?

Acute

+

Eye

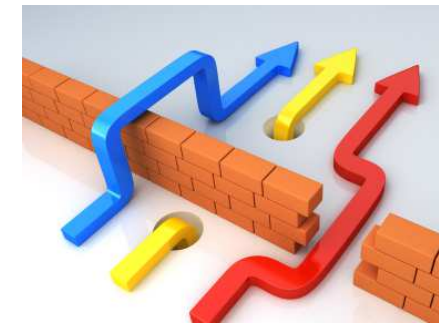
+

Rare



**“In God we trust.
All others must
bring data”.**

W. Edwards Deming



- ▶ Demonstrate neuroprotection in humans

2.5 Milestones

POC



2.5 Milestones

POC

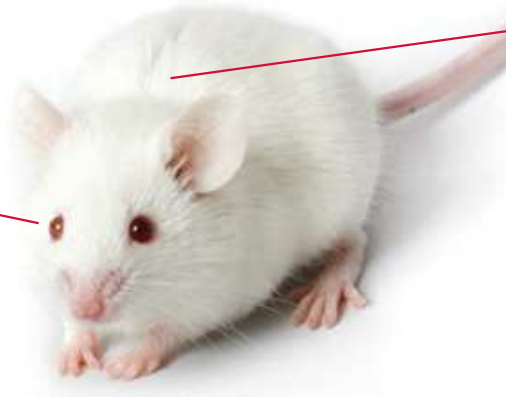
Optic Neuritis (x2)
(demyelination)



2.5 Milestones

POC

Optic Neuritis (x2)
(demyelination)

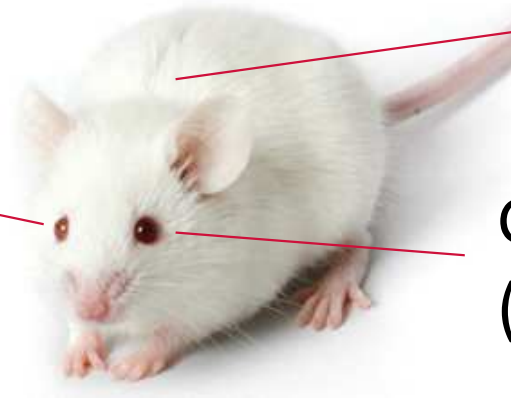


Multiple Sclerosis (x2)
(inflammation)

2.5 Milestones

POC

Optic Neuritis (x2)
(demyelination)



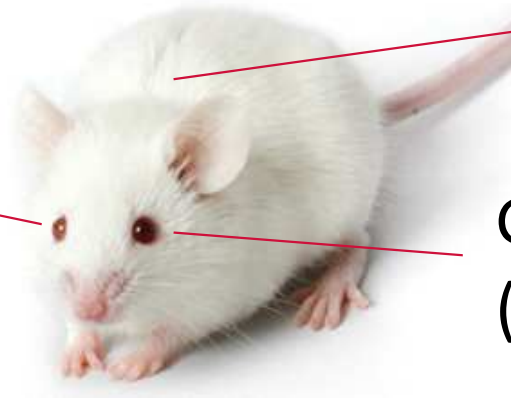
Multiple Sclerosis (x2)
(inflammation)

Glaucoma
(hypertension)

2.5 Milestones

POC

Optic Neuritis (x2)
(demyelination)



Multiple Sclerosis (x2)
(inflammation)

Glaucoma
(hypertension)

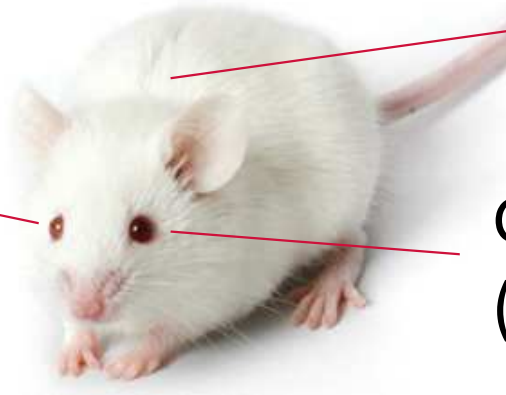
Patent



2.5 Milestones

POC

Optic Neuritis (x2)
(demyelination)



Multiple Sclerosis (x2)
(inflammation)

Glaucoma
(hypertension)

Patent



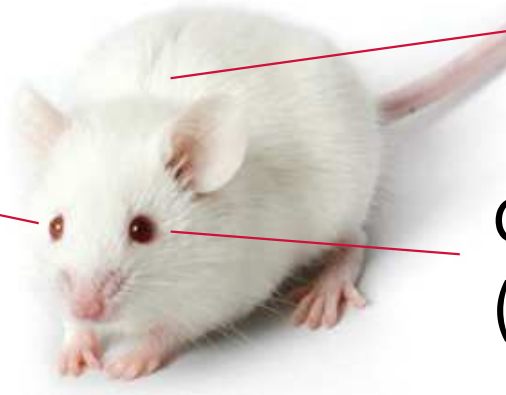
Orphan



2.5 Milestones

POC

Optic Neuritis (x2)
(demyelination)



Multiple Sclerosis (x2)
(inflammation)

Glaucoma
(hypertension)

Patent



Orphan



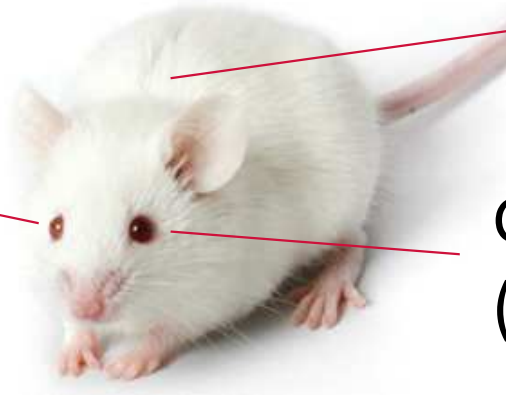
Pre-IND



2.5 Milestones

POC

Optic Neuritis (x2)
(demyelination)



Multiple Sclerosis (x2)
(inflammation)

Glaucoma
(hypertension)

Patent



Orphan



Pre-IND



Regulatory Preclinical Toxicology for IND

2.6 Development strategy

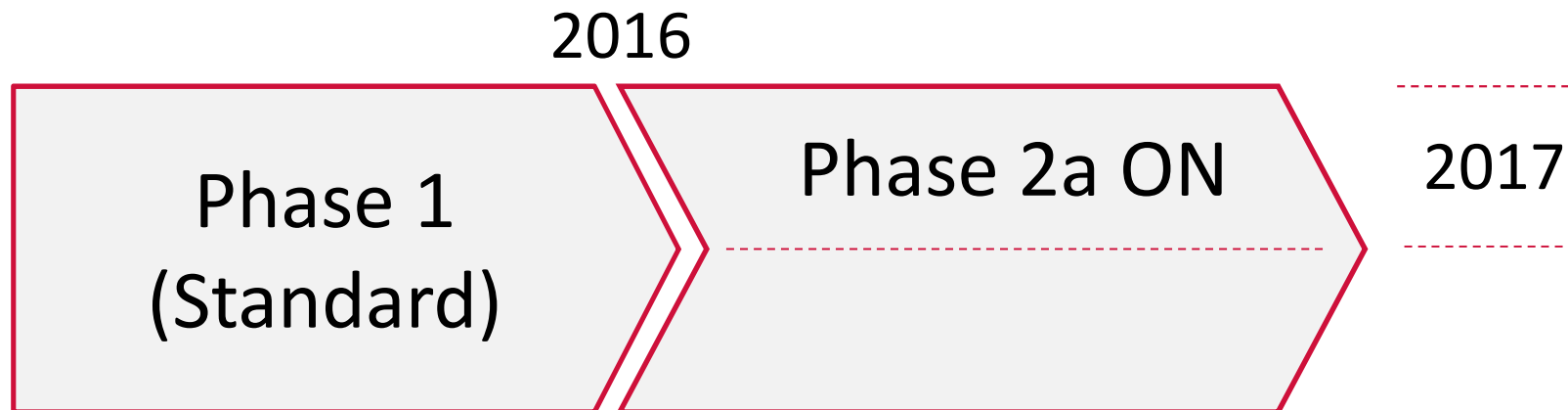
2.6 Development strategy

2016

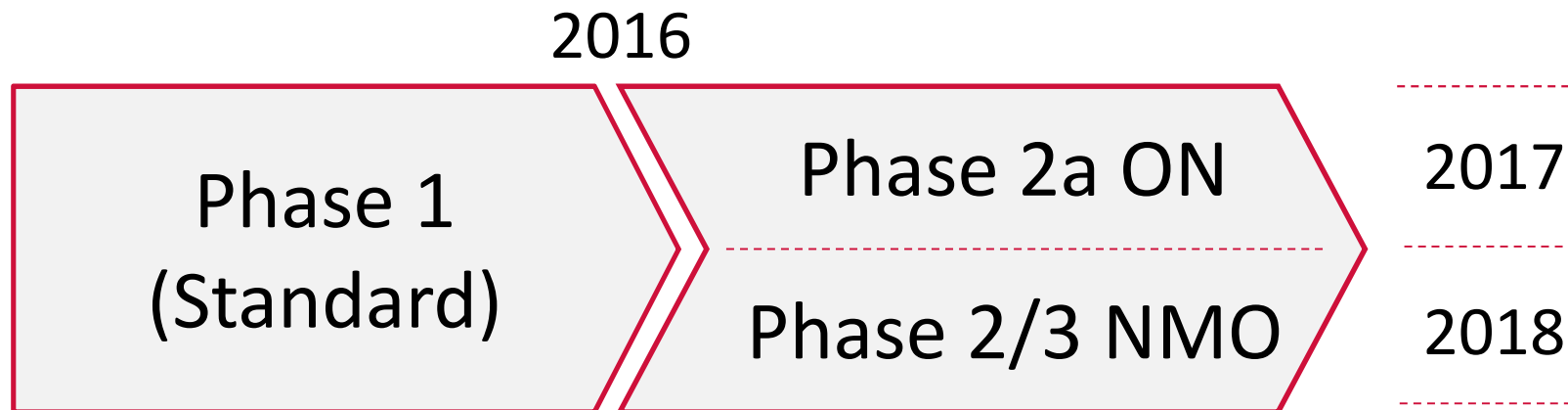


Phase 1
(Standard)

2.6 Development strategy



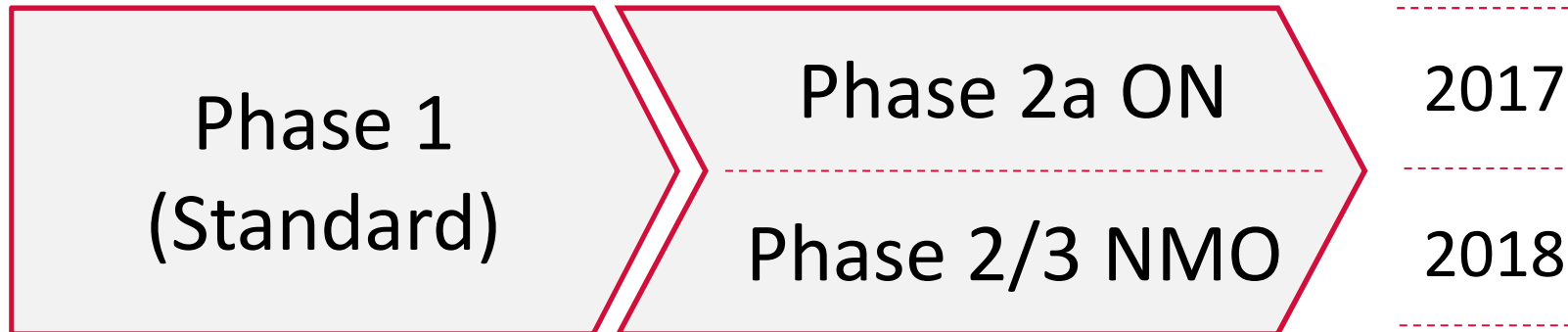
2.6 Development strategy



2.6 Development strategy



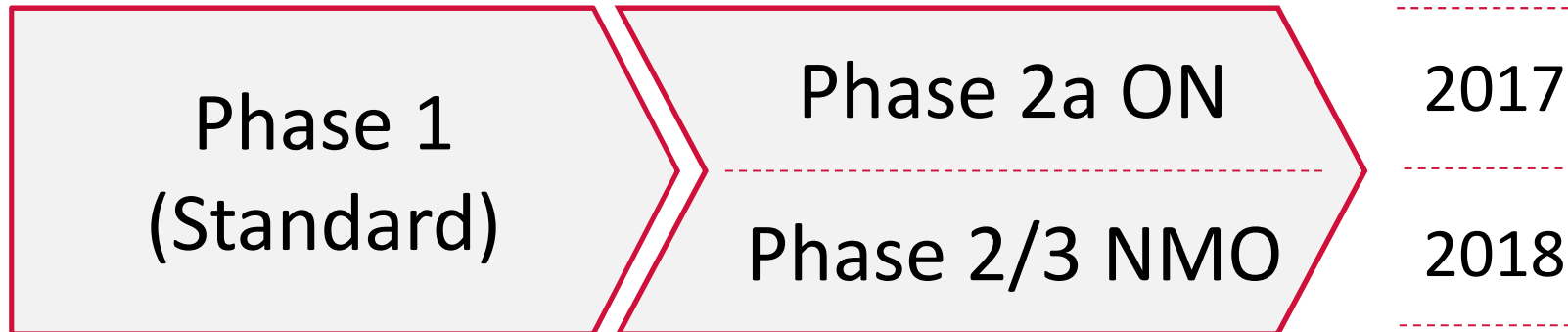
2016



2.6 Development strategy



2016



- ▶ **Biz potential after probing neuroprotection**

2.6 Pitfalls & Risks

XII Encuentro
Farma-Biotech

Management



Albert G. Zamora, MBA
Co-founder, CEO

2.6 Pitfalls & Risks

XII Encuentro
Farma-Biotech

Management



Albert G. Zamora, MBA
Co-founder, CEO



Pablo Villoslada, MD, PhD
Co-founder, CSO

2.6 Pitfalls & Risks

XII Encuentro
Farma-Biotech

Technical/Strategic risk

“Been There, Done That”

2.6 Pitfalls & Risks

XII Encuentro
Farma-Biotech

Technical/Strategic risk

“Been There, Done That”



Joaquim Trias



2.6 Pitfalls & Risks

Technical/Strategic risk

“Been There, Done That”



Joaquim Trias Larry Steinman



2.6 Pitfalls & Risks

Technical/Strategic risk

“Been There, Done That”



Joaquim Trias

Larry Steinman

Steve Hauser



2.6 Pitfalls & Risks

Technical/Strategic risk

“Been There, Done That”



Joaquim Trias



Larry Steinman



Steve Hauser



Craig Smith



2.6 Pitfalls & Risks

Technical/Strategic risk

“Been There, Done That”



Joaquim Trias



Larry Steinman



Steve Hauser



Craig Smith

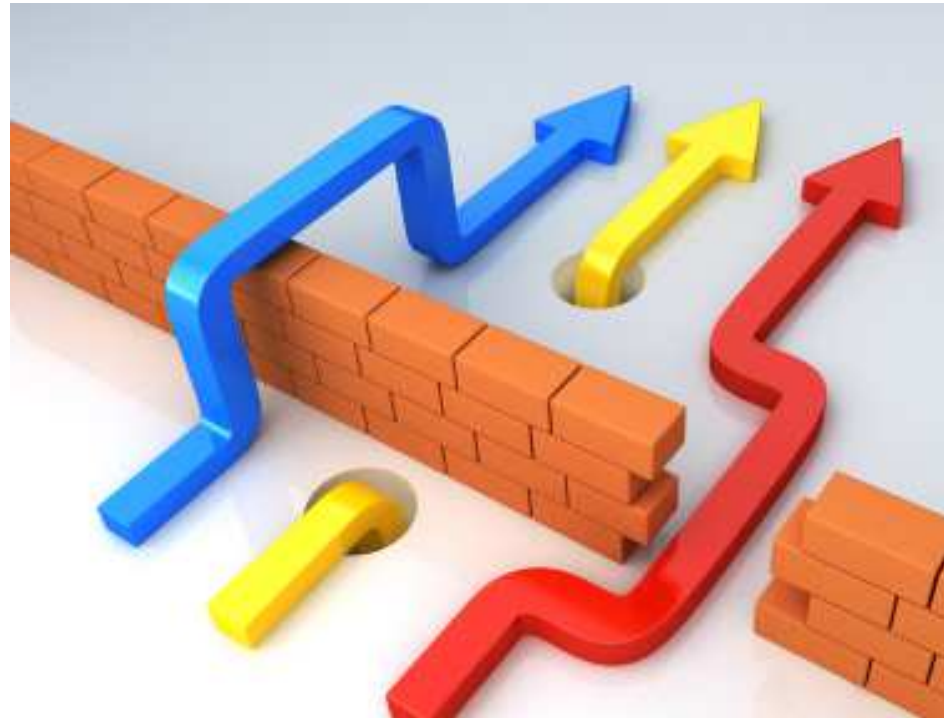
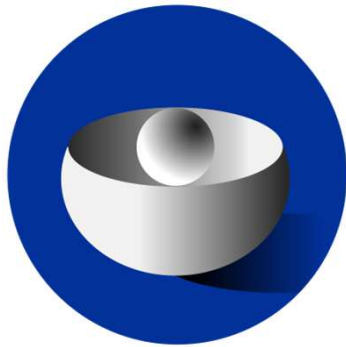


Ari Green



2.6 Pitfalls & Risks

Regulatory risk



2.6 Pitfalls & Risks

Financial risk

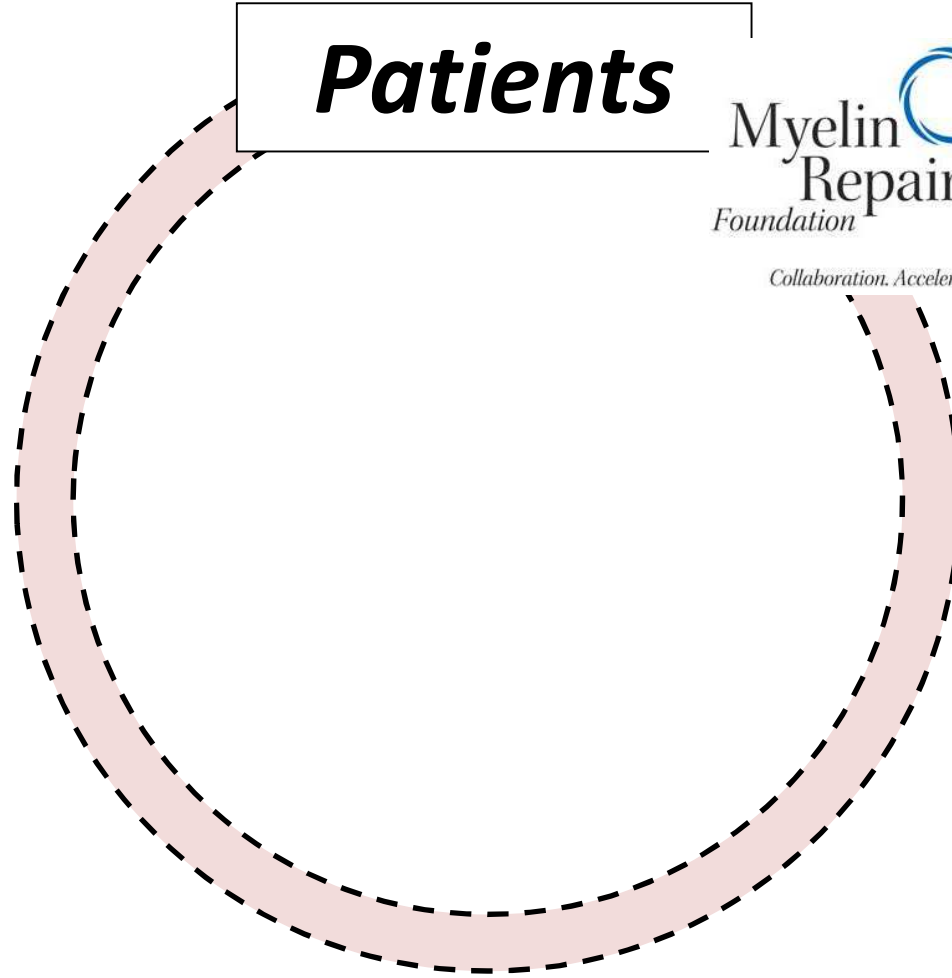
1. Equity round of funding completed
2. International validation by a third party

2.6 Pitfalls & Risks

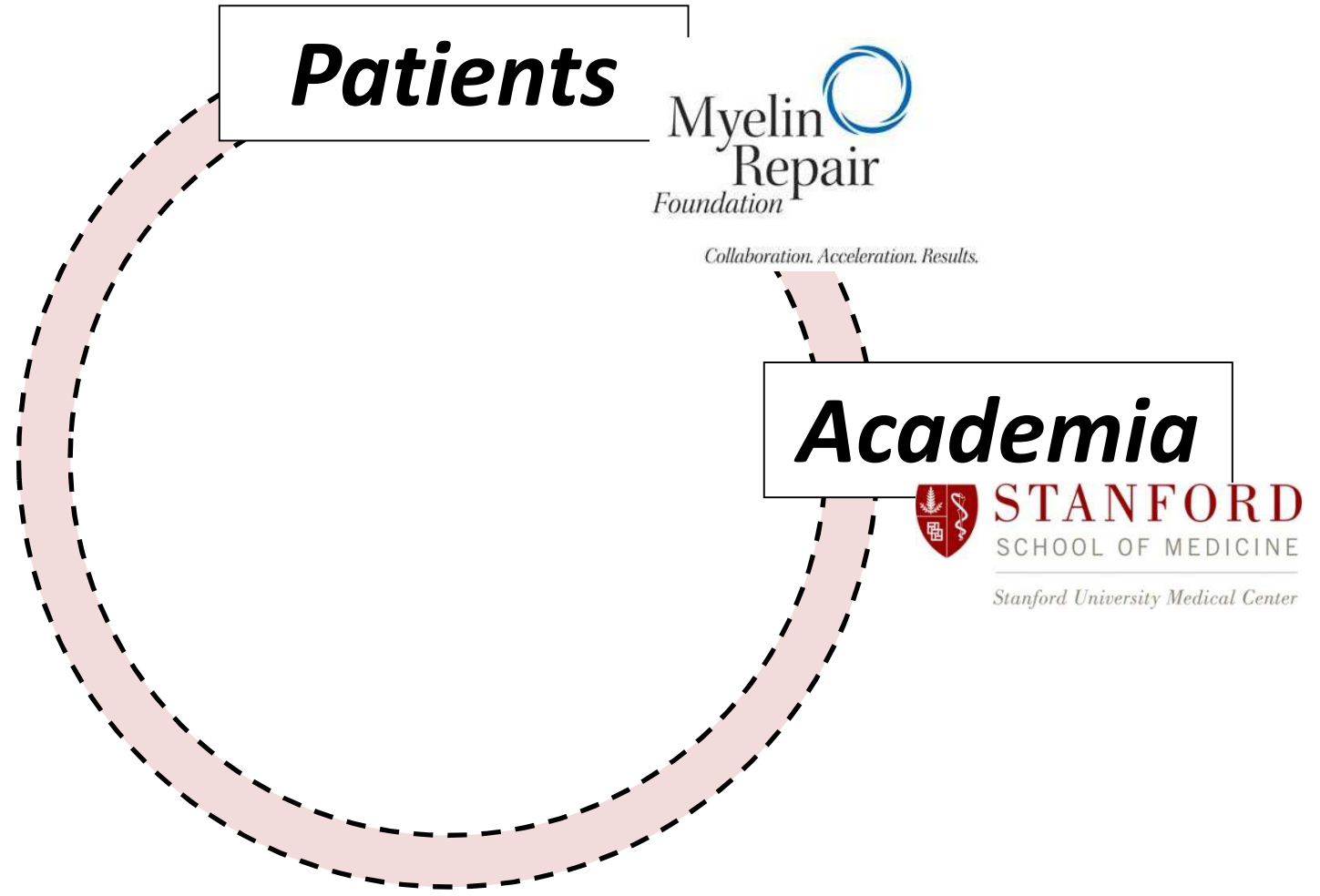
Patients

Myelin
Repair
Foundation

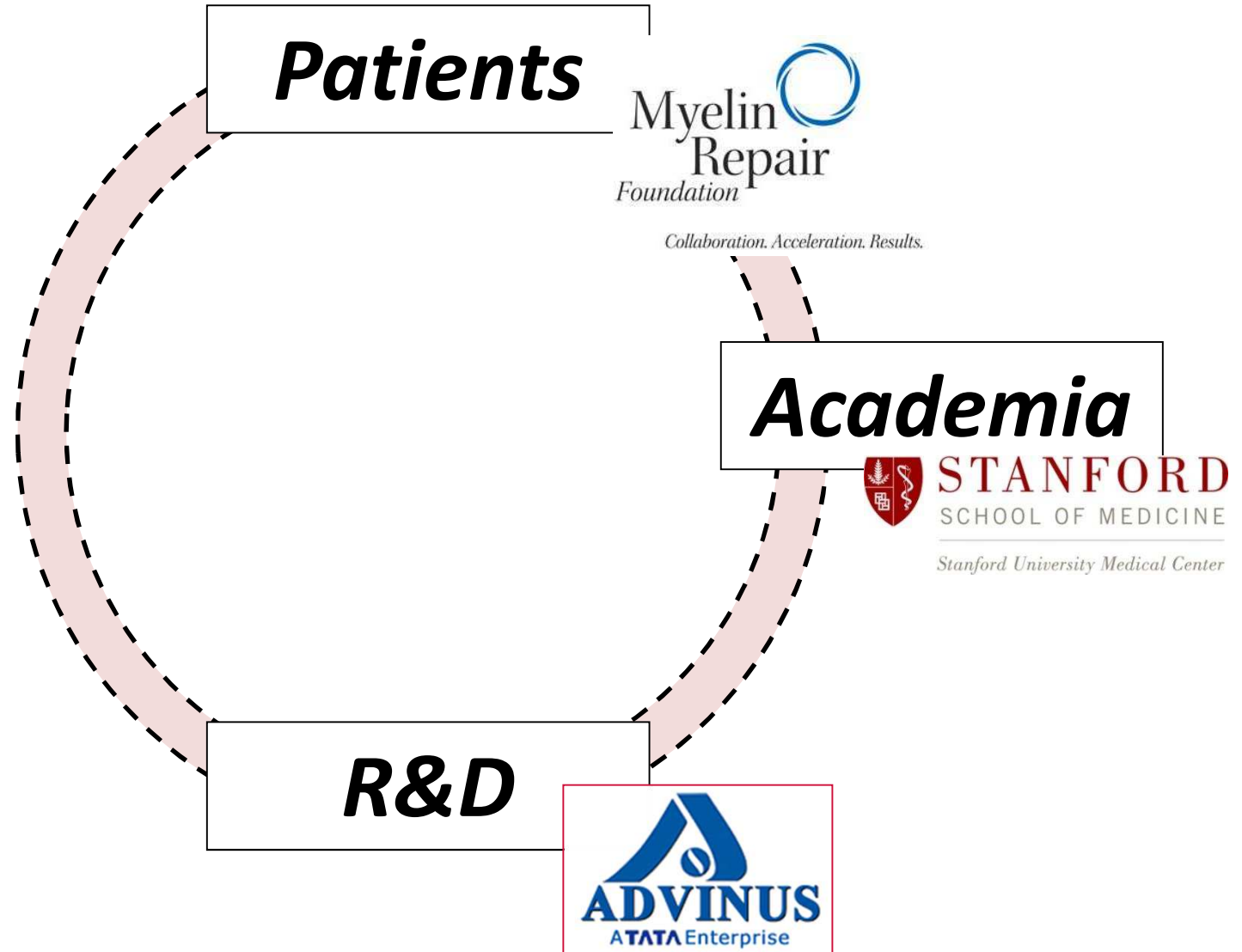
Collaboration. Acceleration. Results.



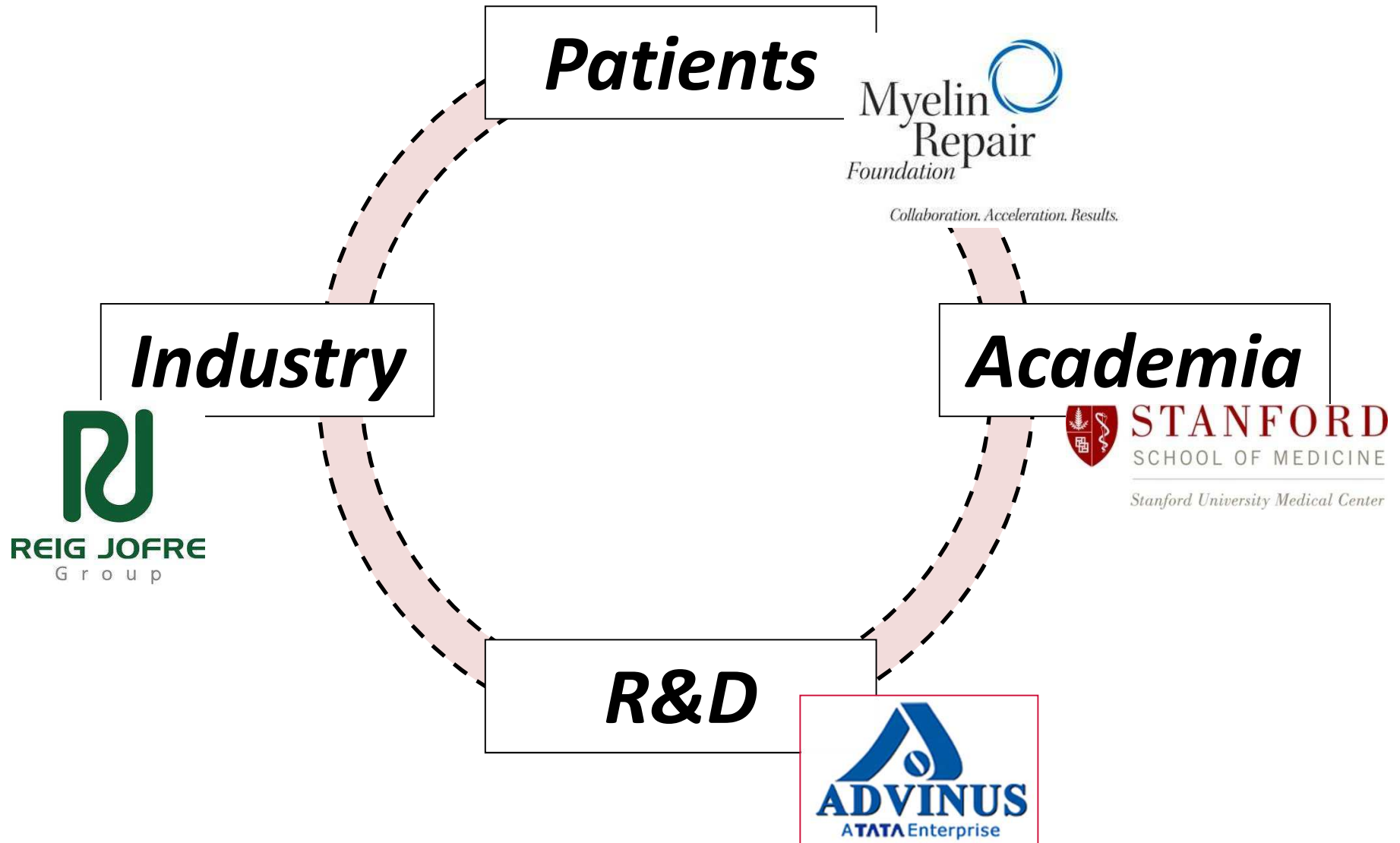
2.6 Pitfalls & Risks



2.6 Pitfalls & Risks



2.6 Pitfalls & Risks



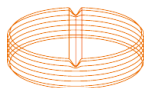
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Santiago de Compostela, 26 de septiembre de 2014

THANK YOU!

bionure

Biomedicine heading to future



MEDICAMENTOS INNOVADORES
Plataforma Tecnológica Española

biospain
2014

farmaindustria