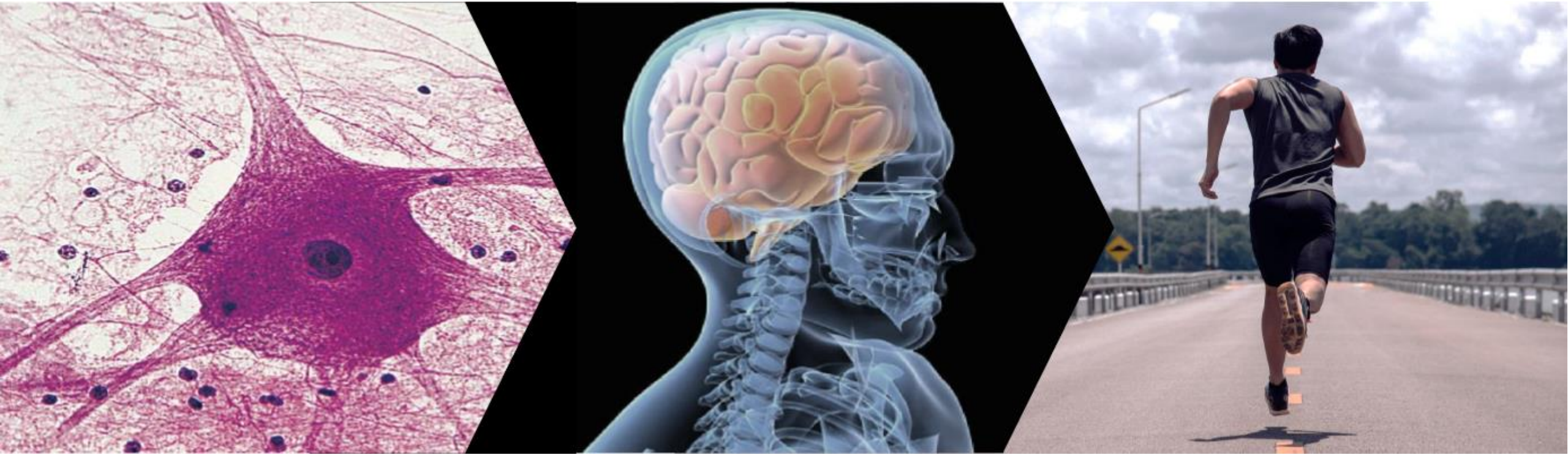


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Promoting Neuroprotection



Promoting Neuroprotection against Neurodegenerative Diseases

Dr Laurent NGUYEN, CEO

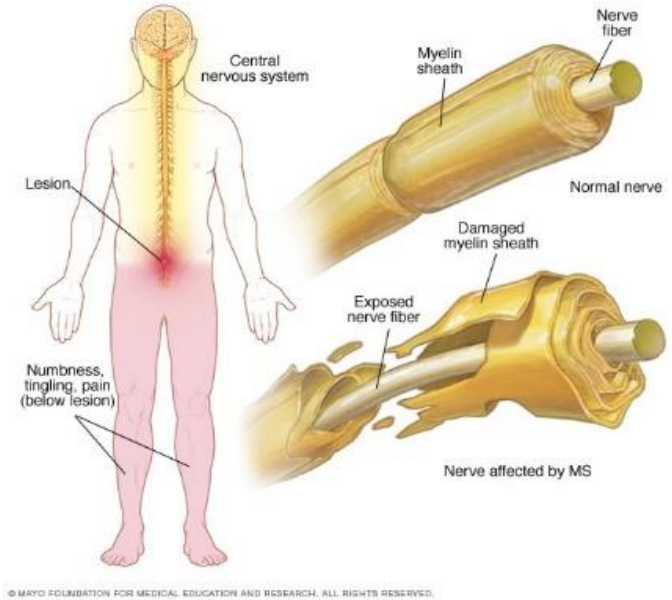
Biotech Showcase 2020

January 2020

Multiple Sclerosis

- ▶ **Immune-mediated CNS disease** with destruction of **myelin** and **nerve fibers**
- ▶ MS disable **2.5m people aged between 20 and 50** with **life expectancy** 5 to 10 years **lower** than average
- ▶ Patterns: **relapsing-remitting** and **progressive MS** courses
- ▶ Dozen **anti-inflammatory or immunomodulating drugs** help to better control relapses but disease progression continues and many drugs carry significant risks of side effects

A clear need for new disease modifier drugs as stand-alone or combo treatment



Disabilities



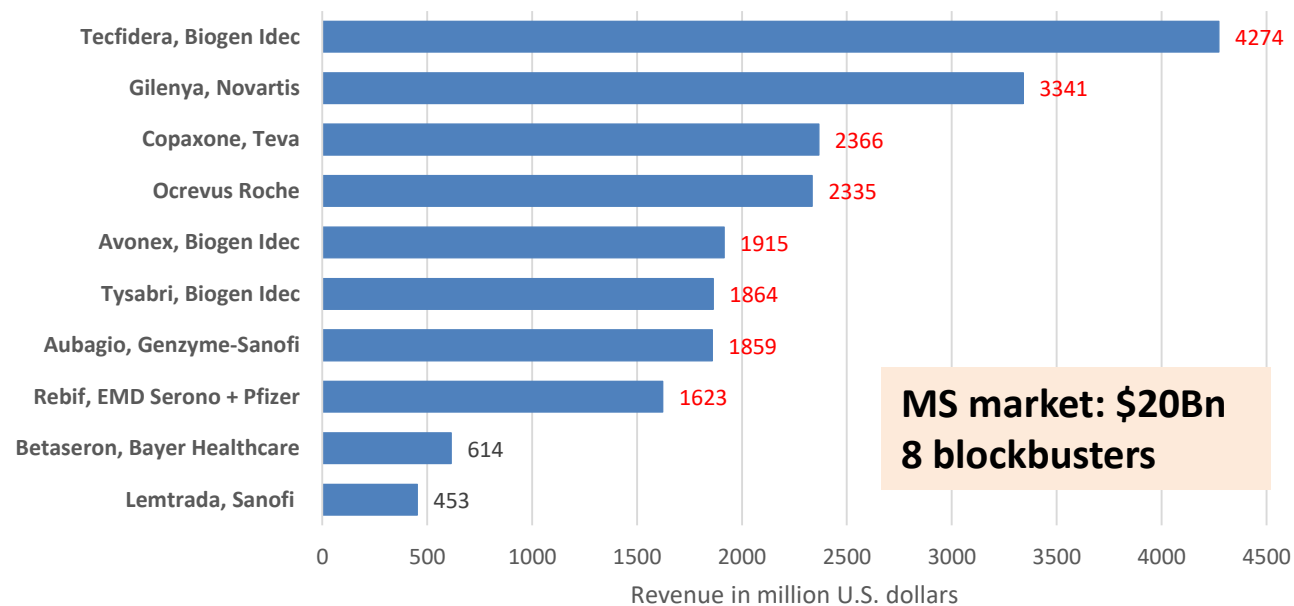
Expectations in Multiple Sclerosis



Patients & Doctors

Slow down, stop or reverse the evolution into disability

Top 10 Multiple Sclerosis drugs by revenue in 2018 (\$m)



Pharmas

Create, preserve or expand Neuro franchises

- Relapse-Remitting MS
- Progressive MS (next battle)

Lead Drug Candidate: BN201

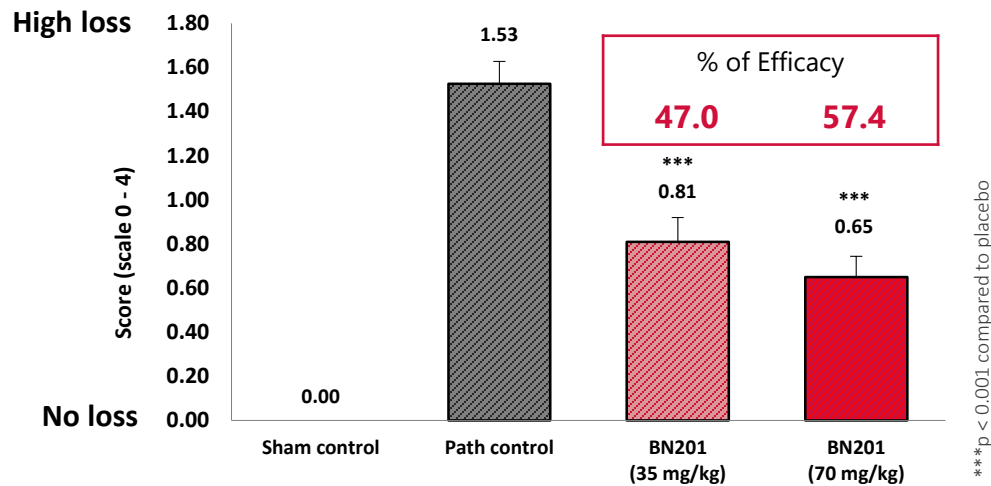
- ▶ **Small molecule** (peptoid class)
 - ▷ Highly soluble **New Chemical Entity** easy to synthesise
 - ▷ Crosses the blood brain barrier
 - ▷ Selected by phenotypic screening to **promotes cell survival and endogenous natural repair of neurons** (remyelination) as a **stand-alone drug and in combo** with anti-MS immunomodulator drugs
 - ▷ **Unique MOA: activation of trophic pathways (IGF1)**
- ▶ **Protection**
 - ▷ **Composition of Matter and method-of use patents** granted (expiry in 2031 + extension)
 - ▷ **Orphan drug designation** granted in EU/US in Acute Optic Neuritis

BN201 promotes axonal protection and prevents demyelination

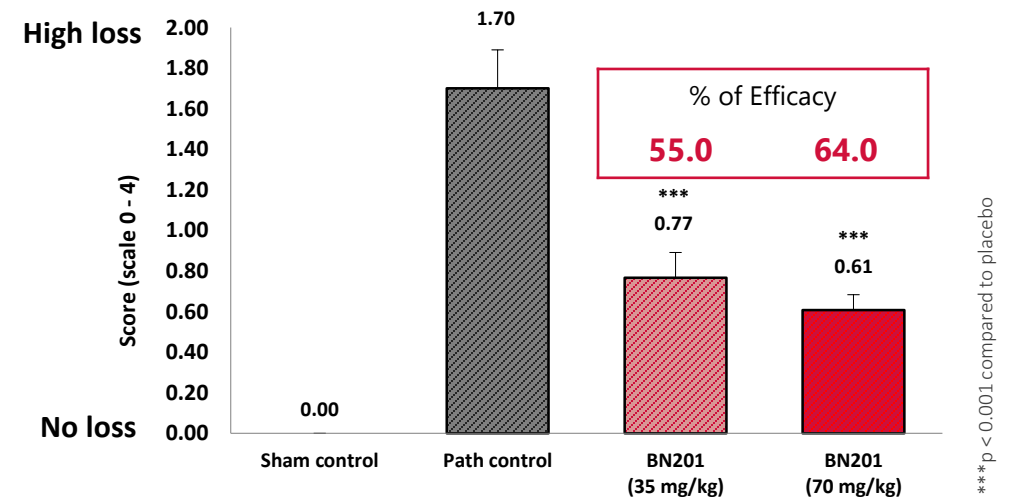
Pre-clinical data

Experimental acute optic neuritis in rats (model of Lysolecithin induced demyelination)

Reduced optic nerve axonal loss

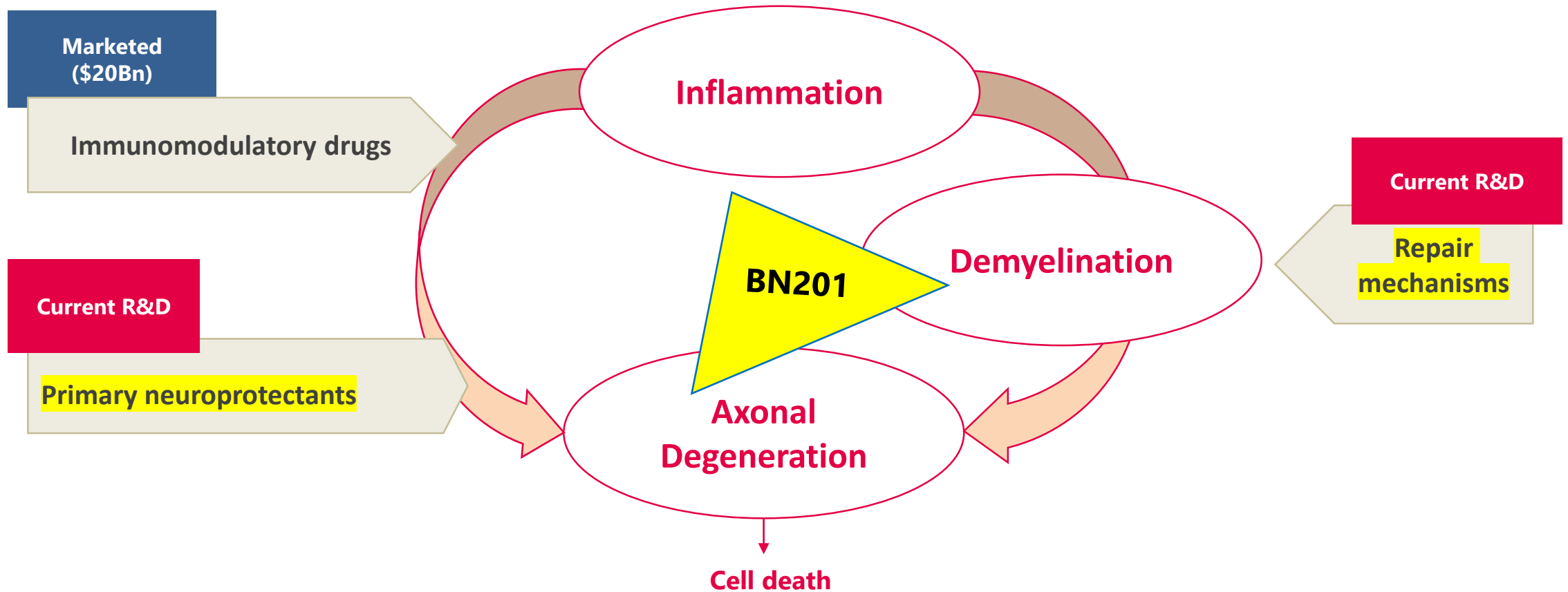


Reduced optic nerve demyelination



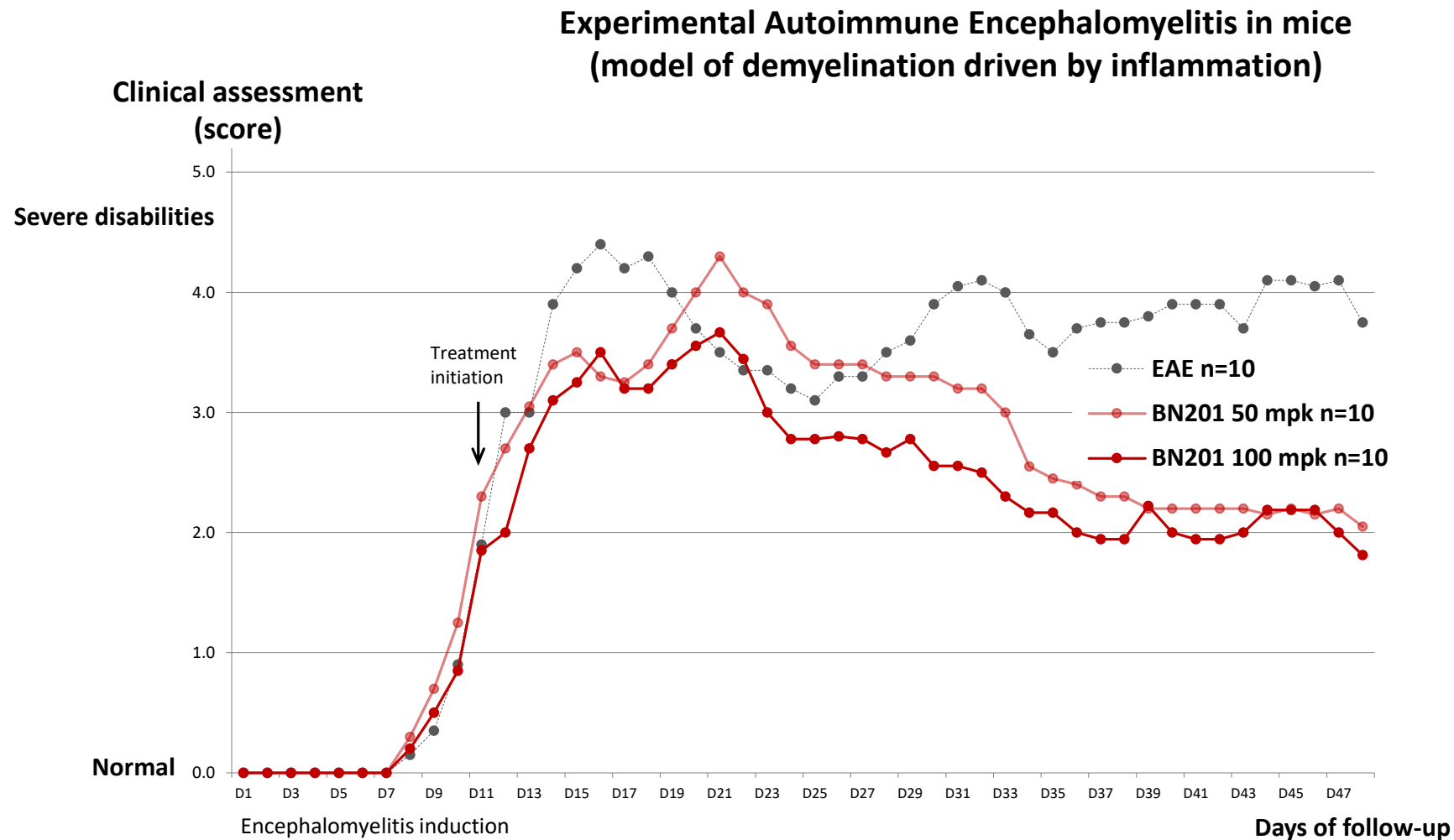
Villoslada P. et al. Neurotherapeutics, published online: 27 February 2019

BN201: Unique neuroprotective & repair actions



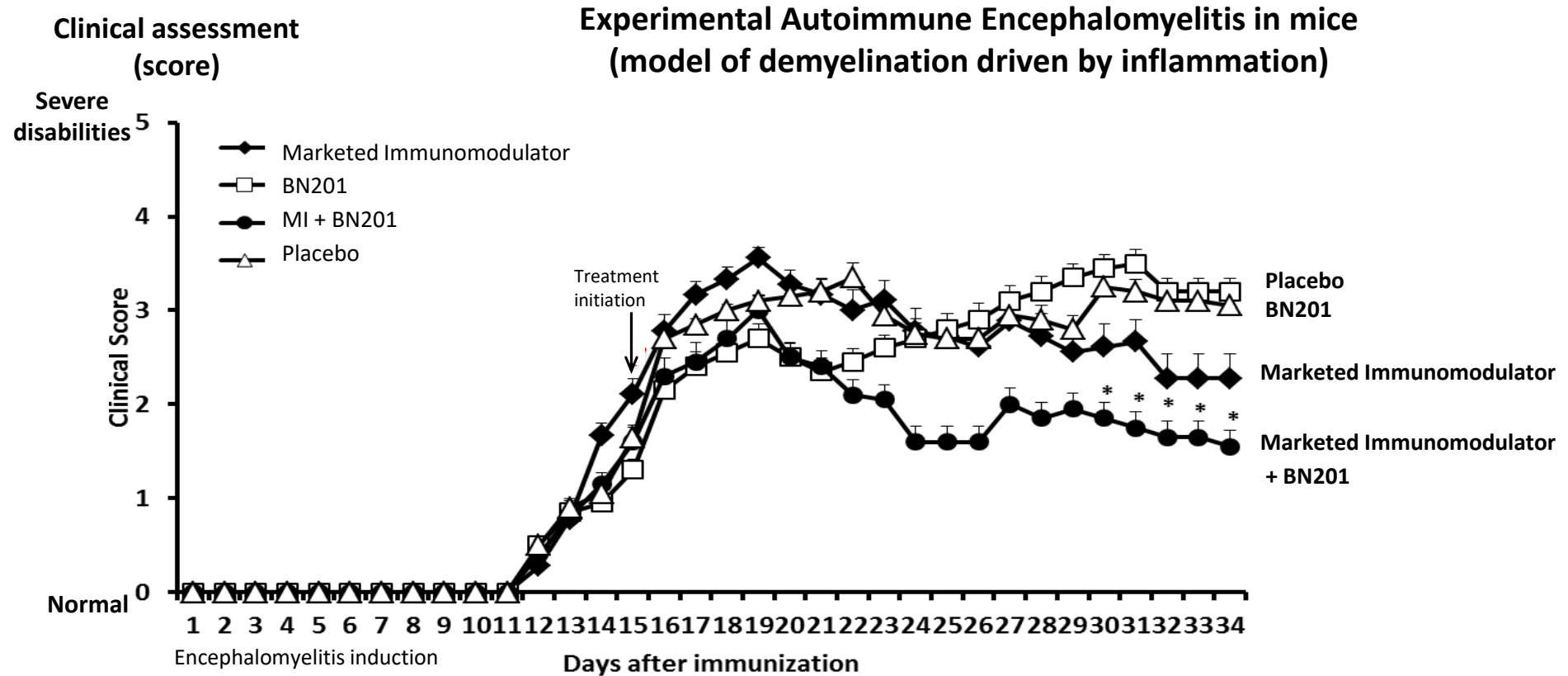
BN201 promotes improvement of clinical function

BN201 as a Stand-alone Drug



BN201 promotes improvement of clinical function

BN201 in Combo with Reference Disease modifier Drugs



► **Potential patient benefits: same efficacy with less side effects or superior efficacy**

Lead candidate: BN201

Pharma drug development steps



“Test tube” (In-vitro)



- BN201 protects the neurons under stress conditions
- BN201 favors neuronal differentiation



Animal testing (In-vivo)

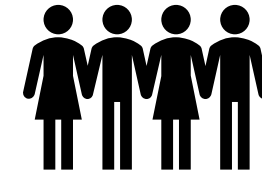


BN201 achieves axonal protection and remyelination in 3 reference animal models

- multiple sclerosis
- optic neuritis
- glaucoma



Human testing (In-vivo)



Double-blind, placebo-controlled clinical Phase 1 study in 48 adult healthy volunteers

- PK data in humans correlate with animal data
- No drug related safety issues

Next step: Clinical Phase 2 studies

Acute Optic Neuritis

A Phase 2 Paradigm for Neuroprotection Therapies in MS



- ▶ **Rare disease** (130K patients/year in EU-US) **with acute inflammation of the optic nerve**
 - ▷ Sub-acute **loss of vision and eye pain** occurring over few days with **long-term vision deficits** in > 2/3 of patients
 - ▷ **Mainly related to multiple sclerosis** but idiopathic origin also seen
 - ▷ **No approved treatment for repair** – Standard of care is IV corticosteroids with no effect on long-term protection

- ▶ **Acute optic neuritis is a suitable condition to test neuroprotective and remyelinating therapies** after acute inflammation (Magi Andorra et al. *JAMA Neurol.* doi:10.1001/jamaneurol.2019.3283)
 - ▷ Gold Standard vision-related outcome measures consider structure and function of the visual pathway including routine non-invasive OCT (**Optic Coherence Tomography**) and LCVA (**Low-Contrast Visual Acuity**)

- ▶ Clinical **Phase 2 study planned in 2020**
 - ▷ **Randomized double-blind placebo-controlled study** to evaluate the safety and efficacy of BN201 compared to placebo in patients diagnosed with a first unilateral AON with a demyelinating origin

Corporate highlights



- ▶ **Academic spin-off** in the field of **neurodegenerative diseases** (IDIBAPS, Hospital Clinic of Barcelona, Spanish National Research Council CSIC, Spain)
- ▶ **Developed into a lean R&D pharma**
 - ▷ **Inception: 2009**
 - ▷ **Privately-held by Business angels, Alta Life Sciences (VC) and Managers**
 - ▷ **7-FTE professional team highly experienced in drug development and corporate growth + biotech ecosystem**
 - ▷ **Raised €12m to date (R&D/corporate spending 75%/25%)**
 - ▷ **Business model: bring candidates to completion of Proof-of-concept Phase 2 study and assess options (partnering, M&A, IPO)**

Seasoned Team & Advisors to Execute Strategy and Plans



Dr. Laurent Nguyen · CEO
(Hoechst-Roussel, Merck KGaA, F. Hoffmann-La Roche Ltd, Pierre Fabre, Sensorion)



Dr. Larry Steinman



Stanford University



Dr. Lucia Septién-Velez · CMO
(Pfizer, Wyeth, GSK, Servier, IPSEN, DBV Technologies)



Dr. Steve Hauser



Dr. Pablo Villoslada · Co-founder, CSO, Chairman of the SAB
(IDIBAPS-University of Barcelona, Roche-Genentech, Neurotech, Stanford University)



Dr. Amit Bar-Or



Dr. Ari Green



Investment thesis

- ❑ **Vision: To develop disease modifier drugs to cure patients suffering from CNS diseases (huge medical needs & \$billion markets)**
- ❑ **Lead program BN201, a small molecule neuroprotectant** with clinical **Phase 1 completed** in healthy volunteers and **Orphan drug designation** in Acute optic neuritis in EU & US
- ❑ **A seasoned team with sound Pharma/biotech experience to execute strategy and plans**
- ❑ **Financing round (Series B) led by Alta Life Sciences to execute a clinical Phase 2 study in neurodegenerative diseases** (acute optic neuritis/multiple sclerosis, 2.5-y Business Plan)

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