Innovative pharmacological treatment for retinal dystrophies

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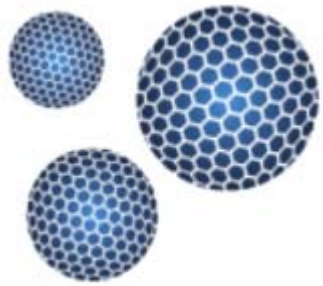
2. AP-1

- 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



The Company

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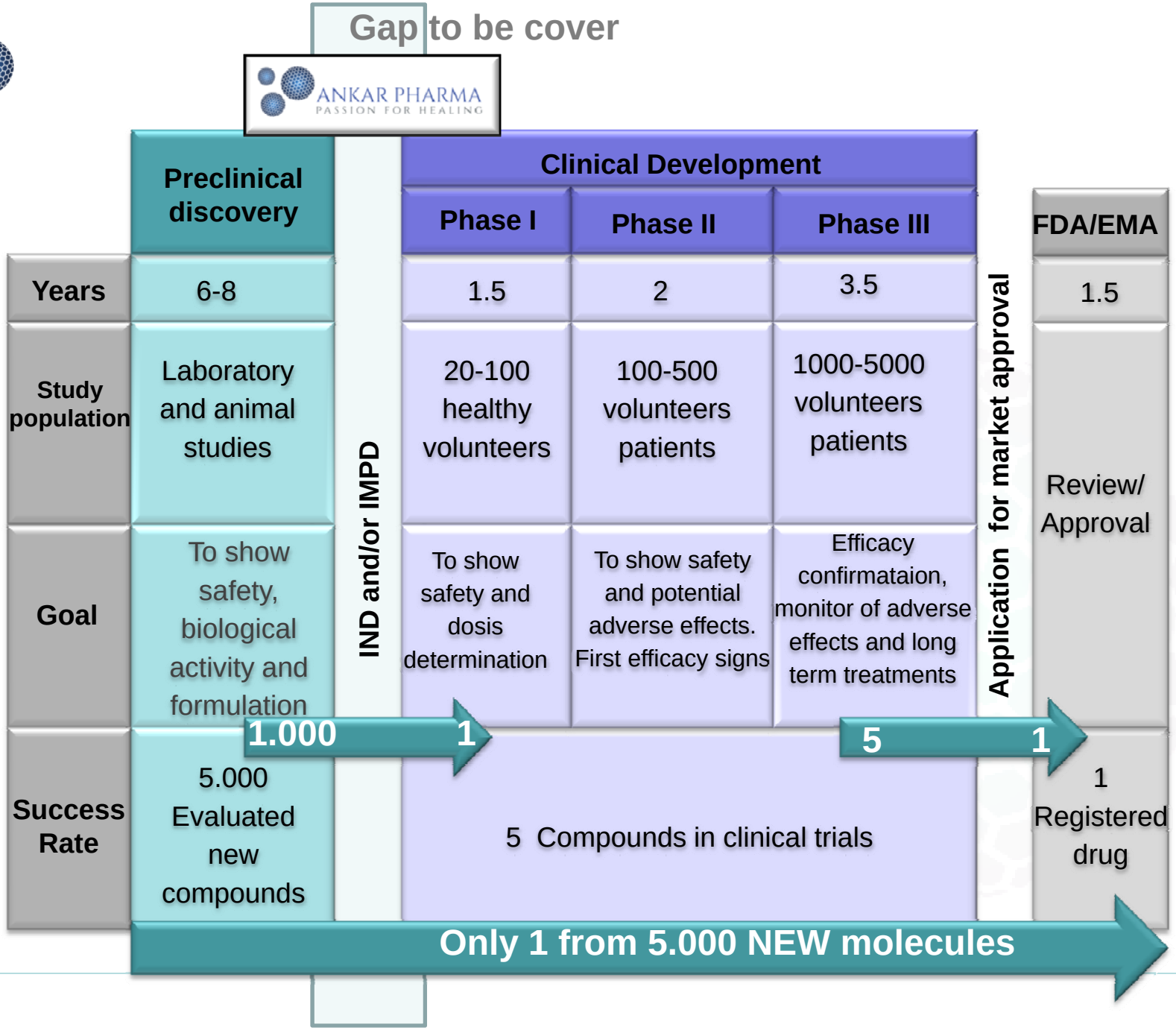
ANKAR PHARMA is a Spanish biotech company, a spin-off of CSIC founded in February 2014

Mission	*Develop drugs that cure or increase the quality of life of patients suffering from neurodegenerative diseases, reducing the time span between drug investigation and market introduction*
Objectives	*Develop successful pre-clinical trials for new innovative drug candidates for neurological diseases and metabolic disorders*
Business Model	*Acquired promising innovative drug molecules from CSIC, develop successful IND-enabling tasks and offer them to the pharmaceutical industry for clinical development and market registration*


Gap to be cover
ANKAR PHARMA
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Preclinical discovery
Clinical Development
Phase I
Phase II
Phase III
FDA/EMA
Years
6-8
1.5
2
3.5
1.5
Study population
Laboratory and animal studies
20-100 healthy volunteers
100-500 volunteers patients
1000-5000 volunteers patients
Review/ Approval
Goal
To show safety, biological activity and formulation
To show safety and dosis determination
To show safety and potential adverse effects. First efficacy signs
Efficacy confirmataion, monitor of adverse effects and long term treatments
IND and/or IMPD
Application for market approval
1.000
1
5
1
Success Rate
5.000 Evaluated new compounds
5 Compounds in clinical trials
1 Registered drug
Only 1 from 5.000 NEW molecules

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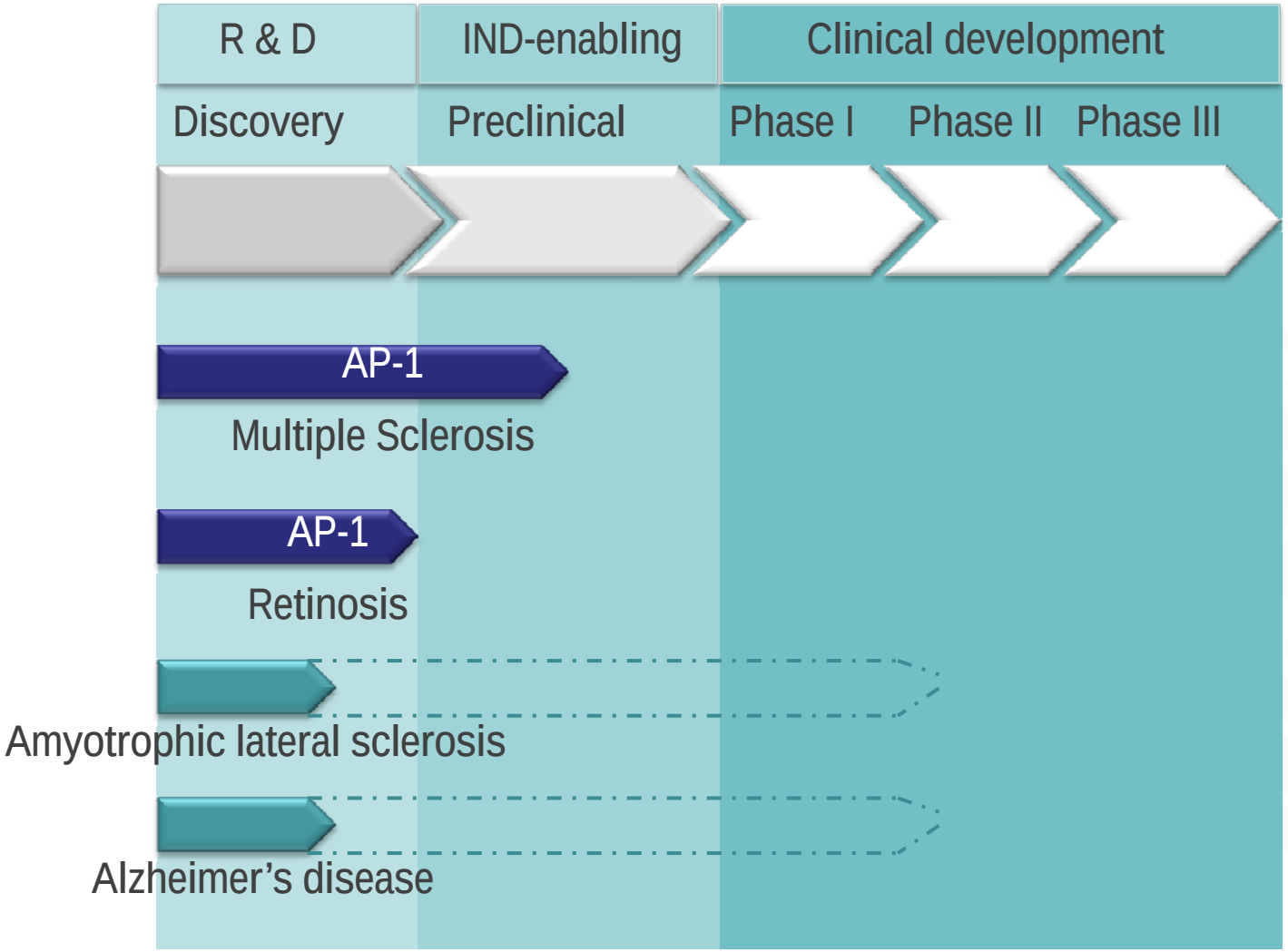

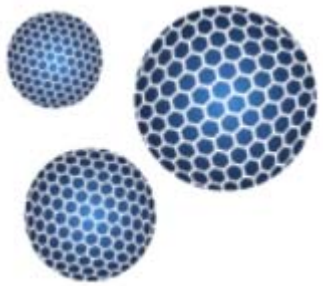

Drug development: AP-1

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First Targeted disease: Multiple Sclerosis

- MS is the most common chronic, no-traumatic disabling CNS disease affecting young adults, affecting 2 Million persons worldwide.
- Age and gender:
  - MS is diagnosed frequently between ages of 20-40 years
  - It is more frequent in women than in men (2:1 ratio up to 3:1 ratio in most recent reviews)

Ratio per 100.000	Prevalence	Incidence
Europe region	>30	
Scotland and Northern Ireland	>200	
UK	203	9.6
Iberian peninsula	72-77	2.2-5
France	95	7.5
Central European countries	62-128	
US	135	
China	1	
India	3	
Asia	1-5	
Germany	149	
Global	**30**	**2.5**

- Prevalence:
  - It is estimated that 2 million people have MS worldwide: 0.6 M in Europe and 0.4 M in US
  - Europe is a high prevalence region with >30/100,000
  - Prevalence of >200/100,000 inhabitants were reported in Scotland and Northern Ireland
  - UK (1990-2010): 286/100,000 in women and113/100,000 in men (2.4% growth per year)

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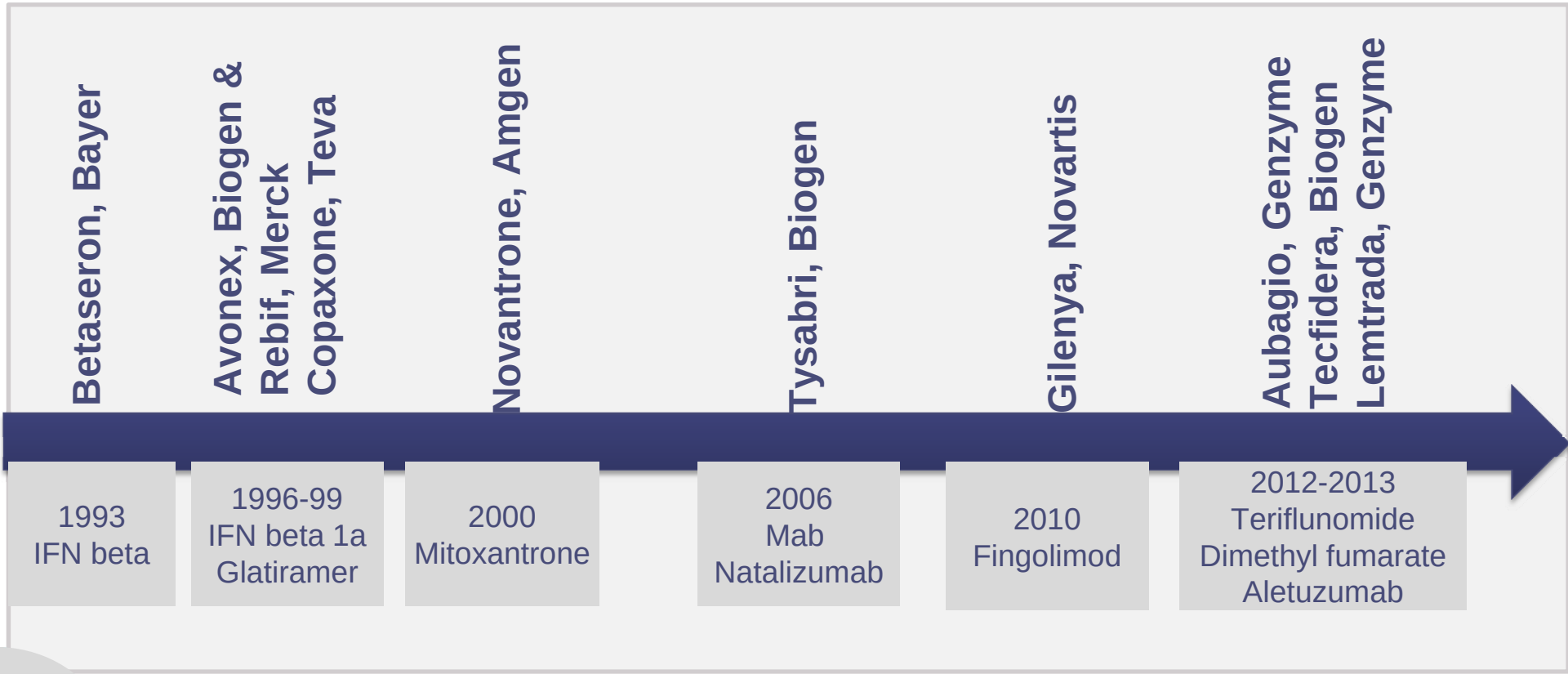
MS: drug competitors

- Several improvements have been made in the recent years in the treatment of MS patients, thanks to the introduction of new therapies.



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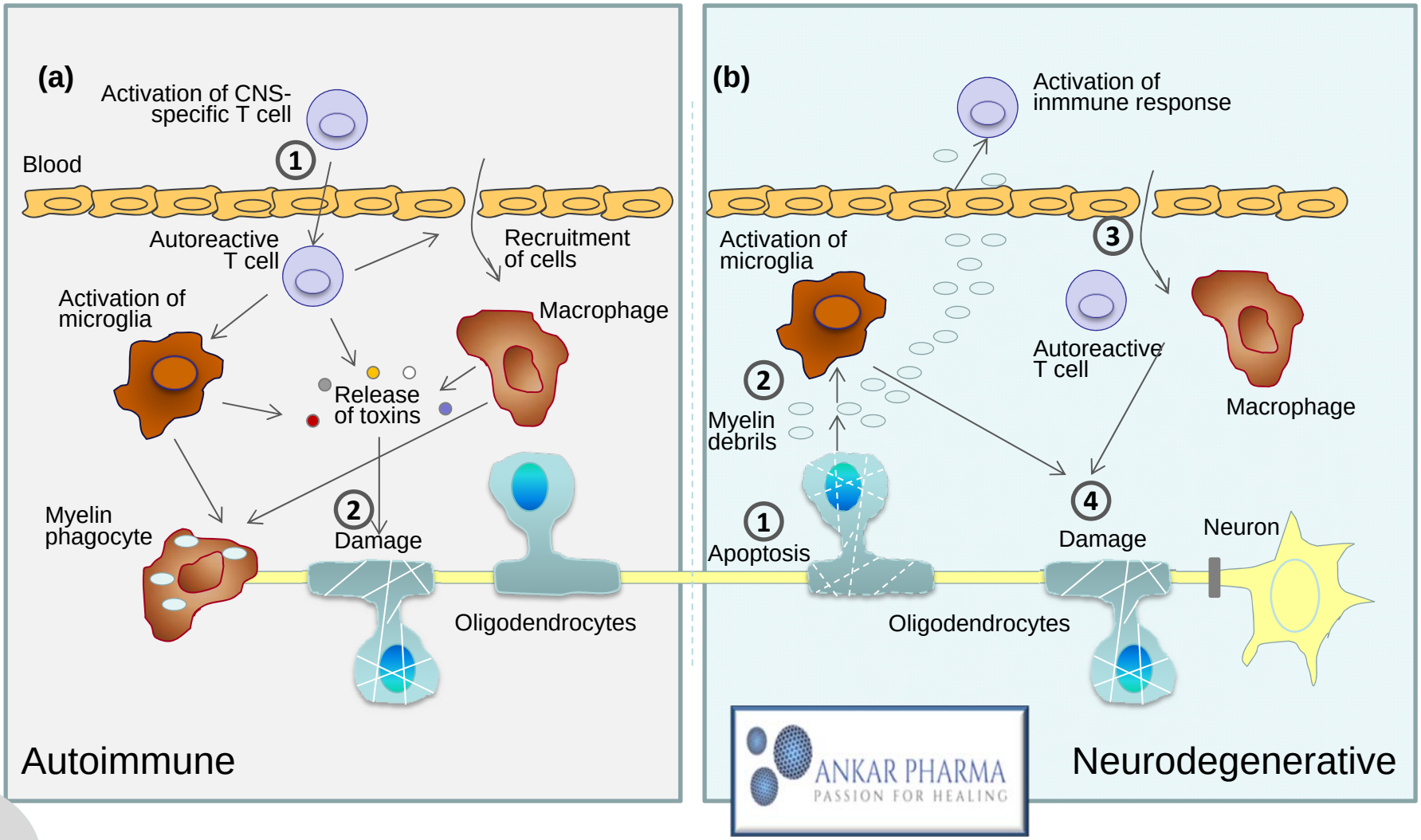
Multiple Sclerosis: Innovative approach



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New Treatment: AP-1

AP-1
first-in-class product candidate for the treatment of MS

Innovative MoA: neuroprotection, remyelinization and antinflammation
high activity in all key components associated to MS (EAE model)
- **decrease of clinical symptoms (neurological score)**
- **reduce inflammation**
- **increase neuroprotection and remyelinazation**

good pharmacokinetic profile compatible with clinical trials

Patent protection

•**AP-1** compound is strongly protected by national patent covering all ITDZs compounds and its use in neurodegenerative disease.
•Initial patent (**ES**2360783 (**B1**) 22.06.2012) is now in national phase for the following territories:
- **USA**: US2012225879 (A1) (02.04.2012)
- **EU:** EP2484670 (27.04.2012)
- **Australia:** AU2010302536 (A1) (27.04.2012)
- **Canada**: CA2780695 (27.04.2012)

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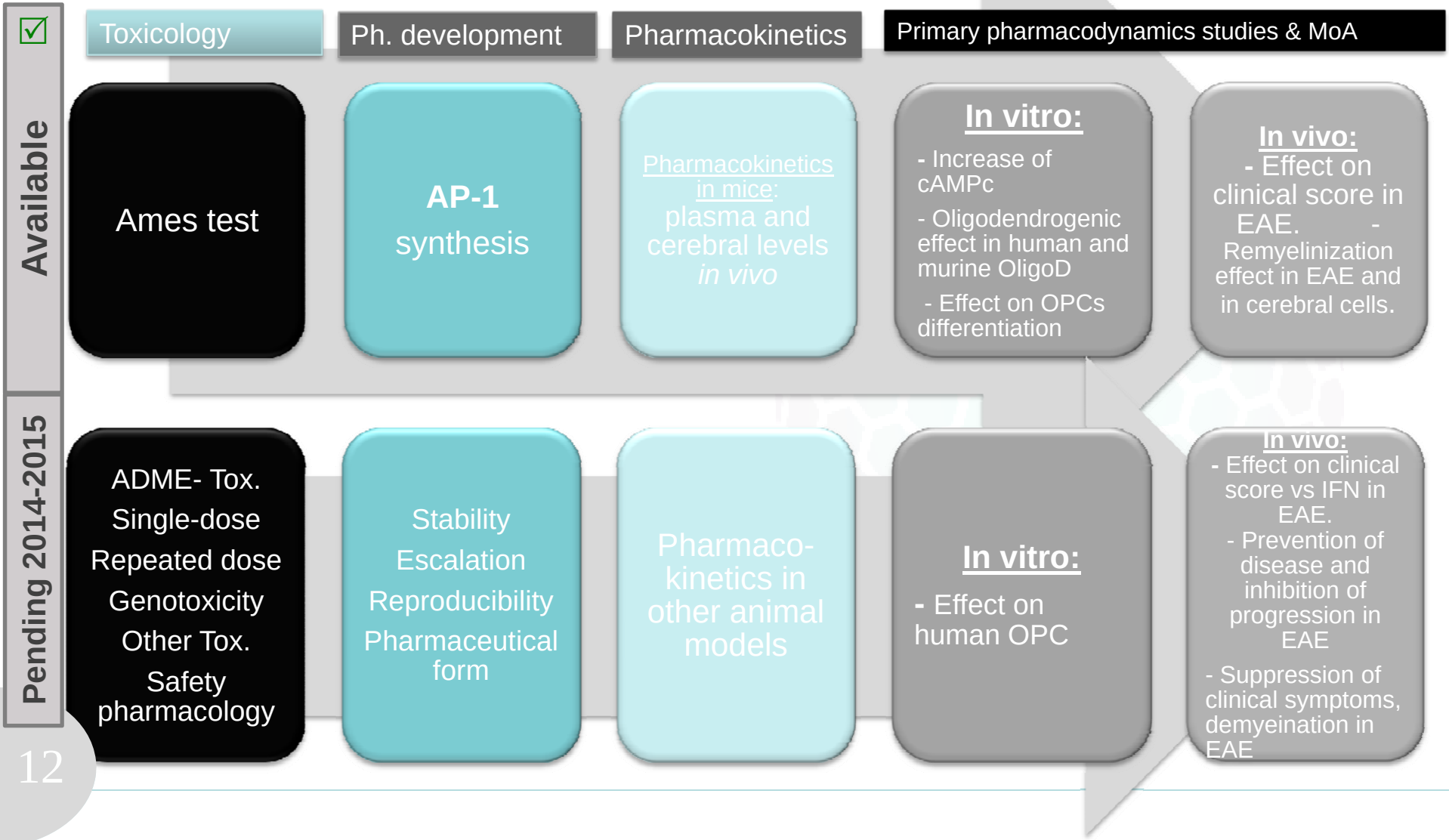
AP-1 Development plan

Until 2014 Investment in Research reached a total of 2 Million euros to obtain all the evidence supporting the efficacy of this triple mechanism of action in EAE, PD and AD.



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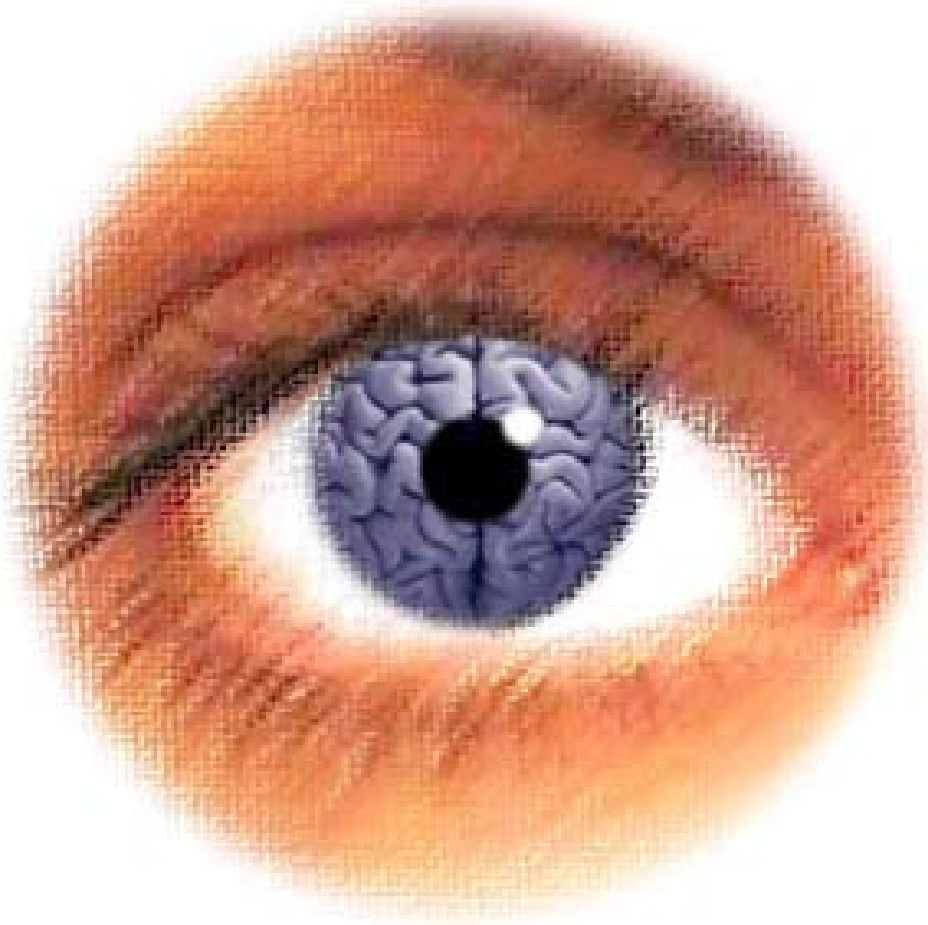
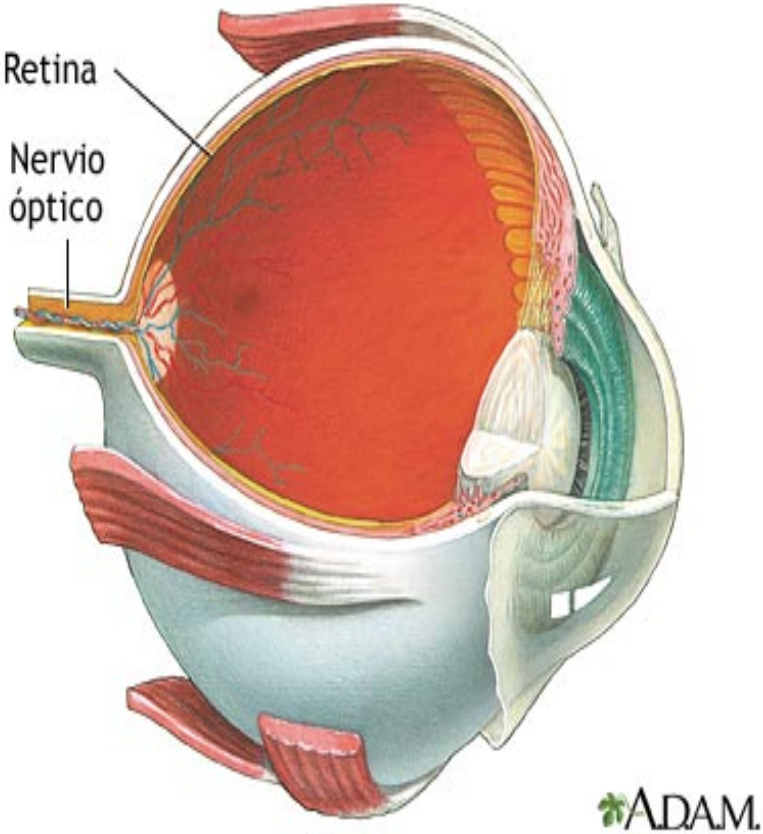
Second Targeted disease: Retinitis pigmentosa





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Second Targeted disease: Retinitis pigmentosa

Retinitis pigmentosa (**RP**) is an inherited, degenerative eye disease that causes severe vision impairment and often blindness. RP is characterized by the progressive loss of photoreceptor cells.

Currently there is no cure for retinitis pigmentosa,

Territorio	Tasa de prevalencia por mil habitantes	2005	2010	2015	2020	2025
España	*3.000*	*14.466*	*15.036*	*15.333*	*15.482*	*15.541*
Unión Europea	4.000	115.370	116.000	116.750	117.325	117.525
EE.UU.	4.000	74.912	78.673	82.253	85.637	88.733
Canadá	4.000	8.068	8.438	8.798	9.147	9.478
Australia	4.000	5.078	5.341	5.599	5.855	6.098
Japón	4.000	31.974	31.940	31.652	31.122	30.404
Total		**235.401**	**240.391**	**245.051**	**249.086**	**252.237**

Rare disease

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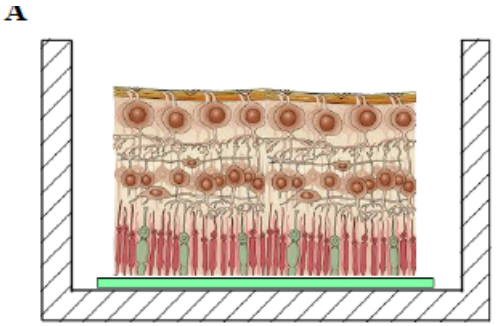
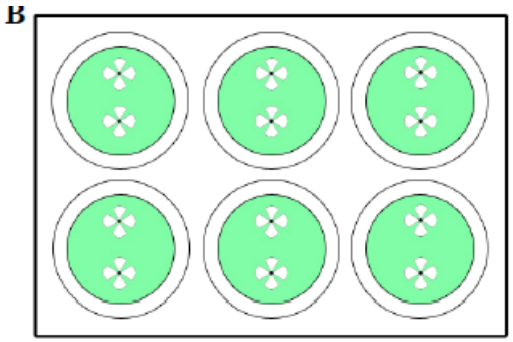
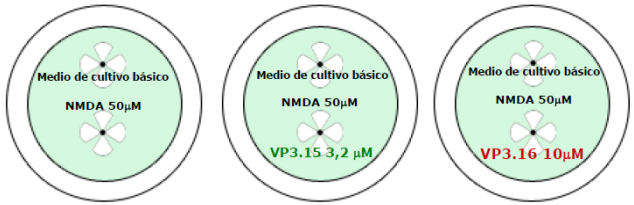
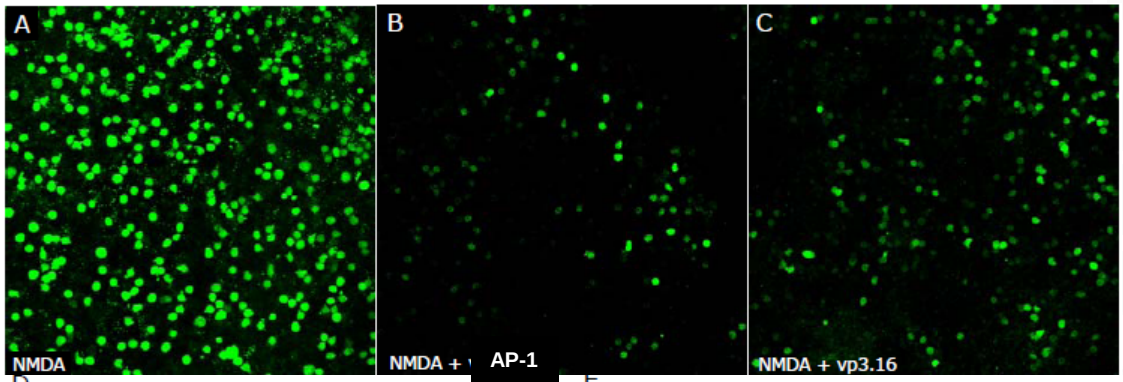
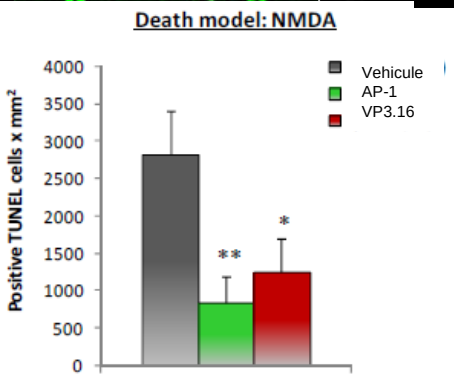
New Treatment: AP-1

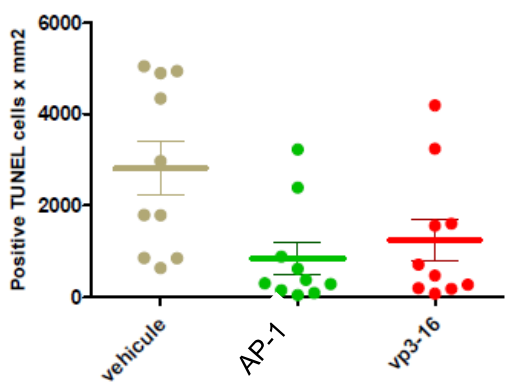












GSK-3 inhibitors, including **AP-1,** protect the ganglionar cells of the retina after the damage with NMDA.

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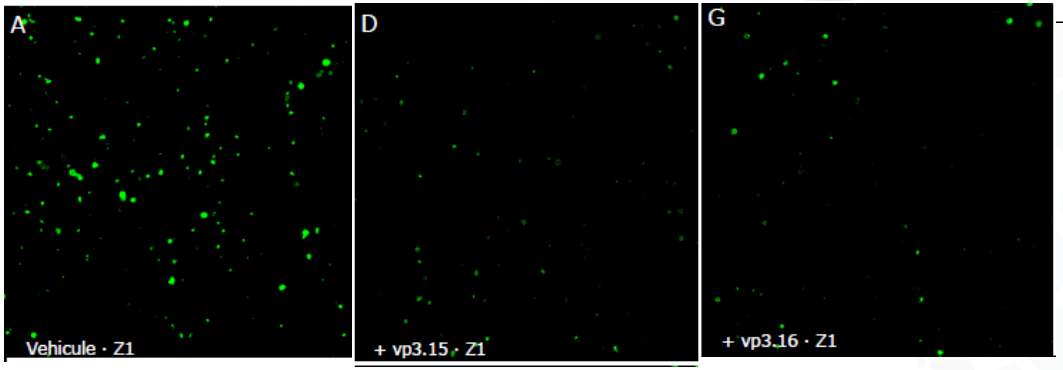
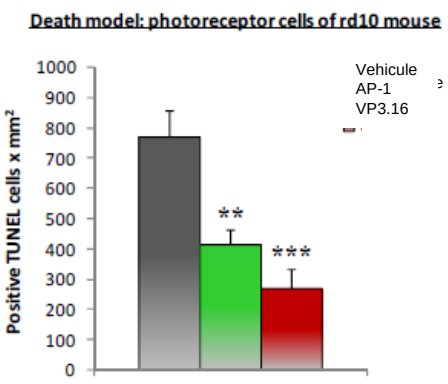
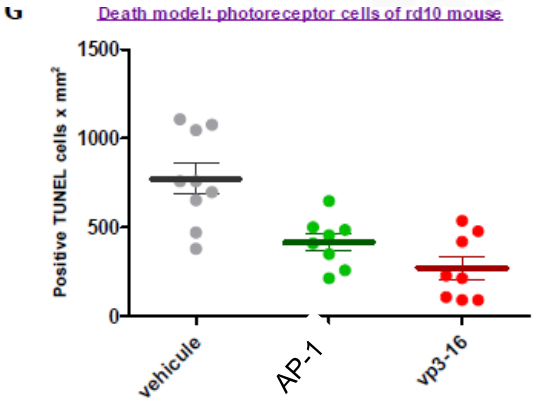
New Treatment: AP-1

rd10 mice







GSK-3 inhibitors, including **AP-1,** protect the photoreceptor cell of rd10 mice retinas.

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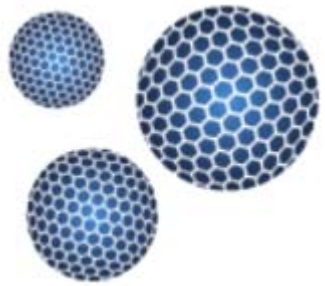
In vivo functional evaluation of **AP-1** in rd10 mice.



Strategic Business Leverages

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Strategic Business Leverages

Qualitative actions that improve P&L

Close relationship
R&D institutions

Cost
synergies &
optimization

Drug portfolio
management

Alliances with
Pharma & Biotech
companies

De-risking
Strategies

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Strategic Business Leverages

Qualitative actions that improve P&L

Close relationship
R&D institutions

- In-depth knowledge of CSIC patent opportunities
- Focused knowledge in international researches pertaining neurodegenerative diseases
- Members of different international consortiums for research on neurodegenerative disorders

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Strategic Business Leverages

Qualitative actions that improve P&L

Alliances with Pharma & Biotech companies

- Reaching Co-research agreements with several biotech companies for the application of AP-1 in several diseases:
  - Retinitis Pigmentosa
  - Alzheimer
  - Autism
  - ALS

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Strategic Business Leverages

Qualitative actions that improve P&L

Cost synergies & optimization

- Same trials costs for developing more than one therapeutical target
- Alternative global suppliers vs. local
- Active search for public and private grants

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Strategic Business Leverages

Qualitative actions that improve P&L

- MS with AP-1. Objective: Clinical trial phase
- Secondary objective: short-term results. Initiate orphan drug status for retinosis pigmentosa
- De-risking strategy: Obtain patent of a drug in clinical phase to begin neurodegenerative orphan drug clinical trials with patients.

Acknowledgements

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CSIC CIB

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