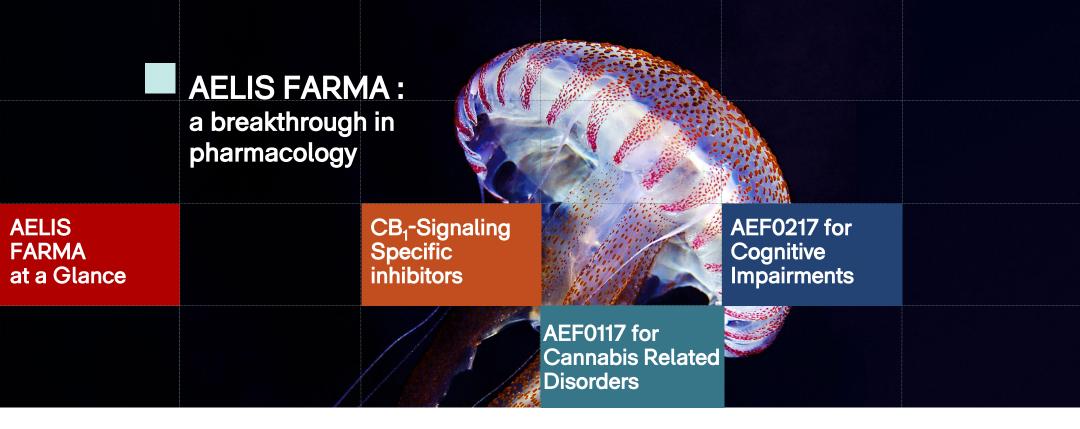


Signaling Specific inhibitors

# A New Pharmacological Class to treat CNS disorders





The company at a glance



#### **Aelis Farma Team**

#### Management



Pier Vincenzo Piazza (MD, PhD) CEO



Valérie Scappaticci (MBA) CFO



Stéphanie Monlezun (PhD) COO

#### Three utmost dedicated founders

- . Scientist-Entrepreneur, Aelis is the third biotech founded by Piervi
  - Former director of the Neurocentre Magendie of INSERM
  - Business savvy, cutting-edge vision on drug discovery and development (Grand Prix INSERM and French Academy of Science for CB<sub>1</sub>-SSi).
- . An associated director for 10 years at EY before founding Aelis
  - Perfectly mastering business, tax and grant regulations. Instrumental in securing record non dilutive financings for Aelis.
- . A neuroscientist with a unique talent for R&D management
  - Never taking no for an answer. 360° knowledge of drug development, the driving force behind the record timeline of AEF0117 and AEF0217 developments.

#### Headquarters



Bordeaux (France)

#### Neurocentre Magendie INSERM

- . One of the best Neuroscience institutes in Europe
- . State-of-the-art scientific infrastructure
- . Access to cutting edge scientist and facilities





# Scientific Advisory Board & Board of Directors

#### **Scientific Advisory Board**



Robert Malenka
Professor and Deputy Director
Neurosciences Institute
- Chairman of the SAB





**Keith Humphrey**Professor and Section Director for
Mental Health Policy Department of
Psychiatry





**Daniele Piomelli**Professor University of California Irvine
Former Director of Drug Discovery and
Development IIT







Markus Heilig
Director, Center for Affective
Neuroscience Linköping University
(Sweden), Former Clinical Director
NIAAA & NIDA



NIAAA

#### **Board of Directors**



Anders Gersel Pedersen
- Chairman of the Board Former Exc. VP R&D Lundbeck



François Thomas

- Board Member -CEO of Inserm Transfer Initiative (ITI) investment Fund



**Thibaut Richebois** 

- Board Member -Director of Economic Development of the Nouvelle Aquitaine Region



**Alain Sainsot** 

- Board Member -Former CEO of Amatsi Group



Pier Vincenzo Piazza

- Board Member -Former Director Magendie Neurocentre CEO AELIS FARMA



**Benedikt Timmerman** 

Censor Venture Partner at IRDI SORIDEC
 Gestion



#### **Overview of Aelis Farma Achievements**

2014

From discovery to end of phase IIa in 6 years

**→ 2021** 

#### A Growing Pipeline of Signaling Specific inhibitors of the CB<sub>1</sub>

#### A new pharmacological class of biased inhibitors

- . Reversing disease states without behavioral side effects
- . Proven efficacy in model of addiction & cognitive impairments
- . Provide pharmacologically differentiated new drug candidates

New CB<sub>1</sub>-SSi for multiple diseases

#### Cannabis Related Disorders: Addiction & Psychosis



#### Clinical stage

- . Extensive preclinical POC & FDA IND
- . Phase IIa completed at Columbia Univ. (NY)
- . Phase IIb will start in 2021

**AEF0117** Phase IIb in 2021

**Exclusivity** up to 2039

#### Cognitive Impairments: First target Down syndrome



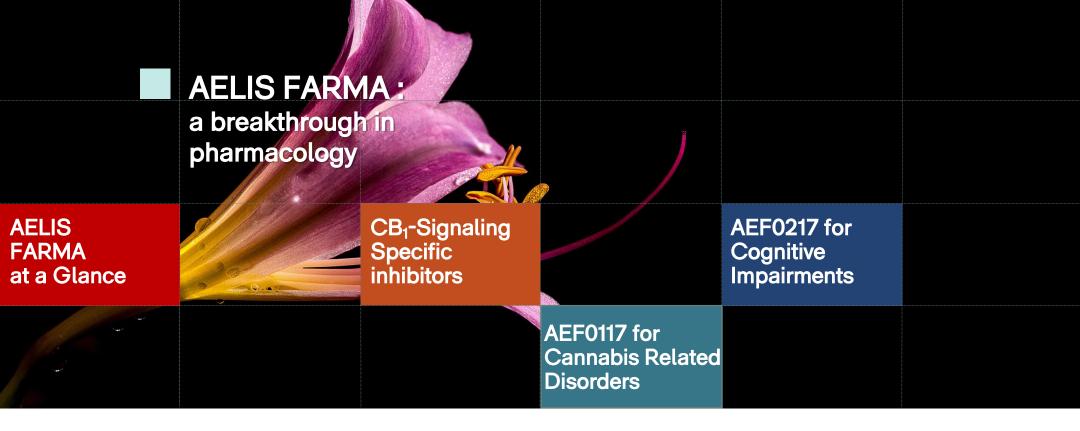
#### Will enter phase I in 2021

- . Very positive preclinical POC. Very favorable ADMET characteristics
- . Studies in Down syndrome subjects will start in 2022

**AEF0217** Phase I in 2021

**Exclusivity** up to 2039



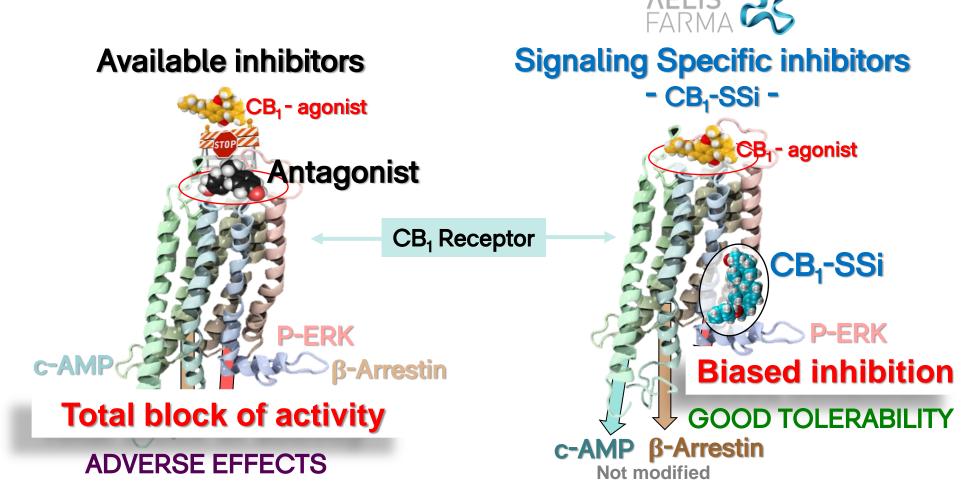


CB<sub>1</sub>-SSi

The first biased inhibitors



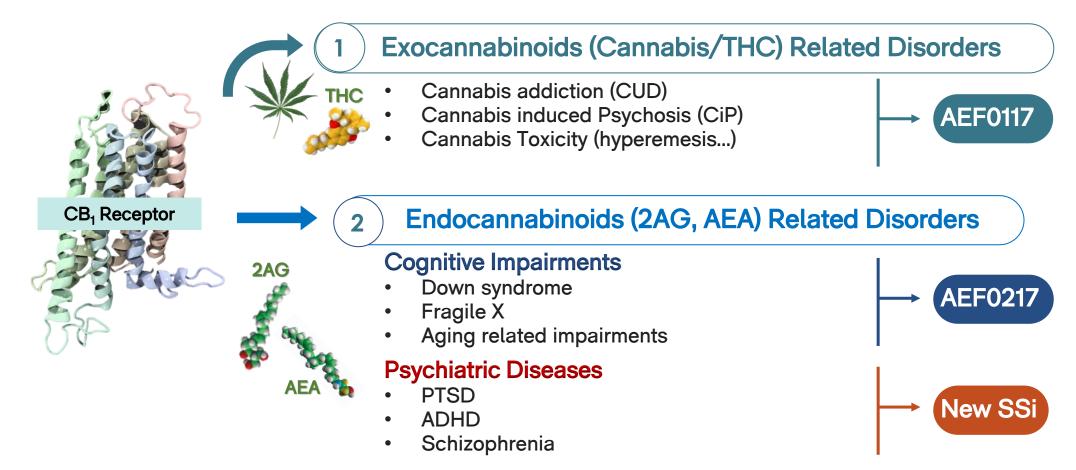
CB<sub>1</sub>-SSi biased inhibition decreases side effects and increases efficacy



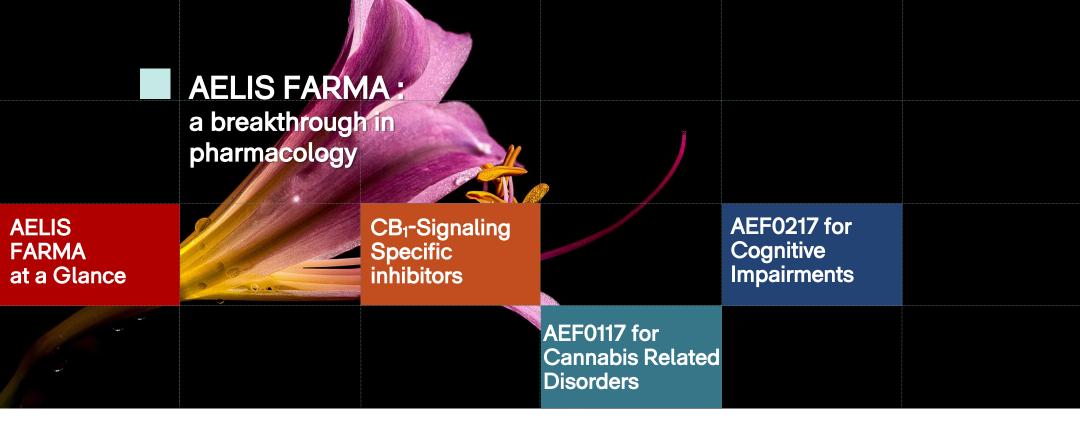


## CB<sub>1</sub>-SSi allow to target a large spectrum of indications

#### The CB<sub>1</sub>-Receptor is involved in several disease states







**AEF0117** 

# A First-in-class for Cannabis Related Disorders



#### **Cannabis Related Disorders**

#### Growing opportunity: increased medical needs because of legalization

#### **Cannabis Related Disorders**

#### Strong unmet medical needs

#### **Large Markets**



Cannabis Use Disorders (Addiction in the DSM-5) 12.4M patients with (CUD) worldwide 9M patients in US, EU, CA, AU

Highest demand for treatment in addiction clinics with no available therapy

Potential \$2Bn turnover per year for AEF0117 at peak sales



Cannabis induced **Psychosis** 

40% of Schizophrenics abuse cannabis and become resistant to neuroleptics 50% of European Emergency Room entries for psychosis

Cannabis induced psychosis are resistant to neuroleptics

Potential \$1Bn turnover per year for AEF0117 at peak sales



Cannabis induced **Toxicity** 

0.5M ER entry in the US due to cannabis

Recurring entries in ER after first visit with significative health care costs

Potential rapid market approval



# AEF0117: strong preclinical & clinical data package

#### **Preclinical**

- Very favorable ADMET profile
- Potent inhibition of THC effects
- Distinct pharmacological profile from CB<sub>1</sub> antagonists



#### Phase I

- Developments in the US
  - FDA IND obtained in 2016



- Phase I studies completed (US)
  - Single and multiple ascending dose (SAD & MAD)
  - Excellent safety profile



#### Phase II

- Phase IIa study in "Cannabis abusers"
  - University of Columbia
  - Cannabis subjective effects and self-administration COLUMBIA
  - No relevant adverse events reported
  - **Study Completed**
- Will enter phase IIb in 2021



#### Good CMC characteristics and advanced development

- Three steps synthesis
- Final formulation identified
- Low costs of goods
- Administration via oral capsules

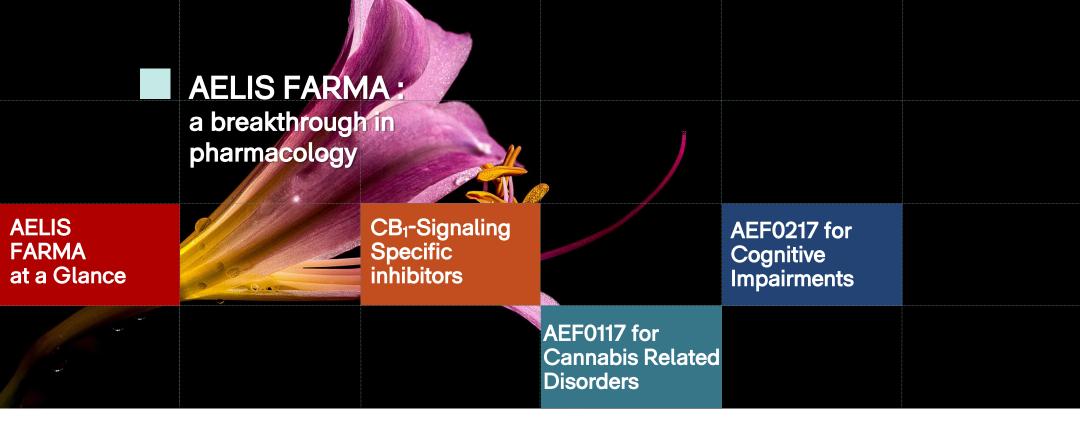


## Supported by NIH (NIDA)





UNIVERSITY



**AEF0217** 

A new approach to treat Cognitive Impairments





## Cognitive Impairments in Down syndrome (DS)

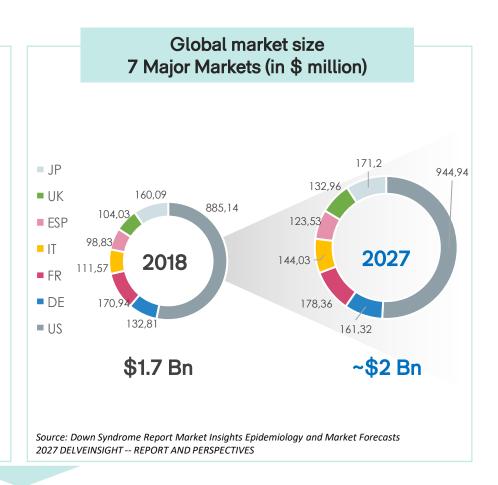
### A major unmet medical need

# Intellectual disability: the major unmet need in DS

- IQ < 70 in children and young adults</li>
- 50% of DS subjects develop Alzheimer disease after the age of 40
- High dependency, negative social and financial impacts



No pharmacological treatment available



Strong demand for a treatment of cognitive impairments and low competition in the space



# CB<sub>1</sub> receptors inhibition: a new target for treating Cognitive Impairments

Inhibition of the CB<sub>1</sub>-receptors by antagonists reverses cognitive impairments in models of neurodevelopmental disorders

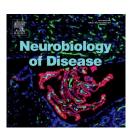
Fragile X syndrome

Targeting the endocannabinoids system in the treatment of fragile X syndrome. **Busquets-Garcia et al., 2013** 

medicine

Down syndrome

Cannabinoid type-1 receptor blockade restores neurological phenotypes in two models for Down syndrome. **Navarro-Romero et al. 2019** 



In animal models (Ts65Dn mice) and in Down syndrome subjects there seems to be a hyperactivity of the endocannabinoid system

# AEF0217: strong preclinical package & ready for Phase I

#### **Preclinical**

- Very favorable ADMET profile
- Very potent in reversing cognitive impairments in trisomic (Ts65Dn) mice
- Distinct pharmacological profile from CB<sub>1</sub> antagonists



#### Phase I (2021-2022)

- **Healthy volunteers** 
  - Single ascending dose (SAD)
  - Multiple ascending dose (MAD)
  - Food effect (Single dose)
- Down syndrome subjects
  - MD for safety, absorption and biomarker of changes in plasticity



#### Phase II (2022-2023)

- Phase II POC in Down syndrome
  - Efficacy on cognitive impairments in young adult Down syndrome subjects. Dose finding study
- Results expected in 2023





Institut Hospital del Mar d'Investigacions Mèdiques

#### Good CMC characteristics and advanced development

- Five steps synthesis
- Final formulation identified
- Low costs of goods
- Drinkable formulation

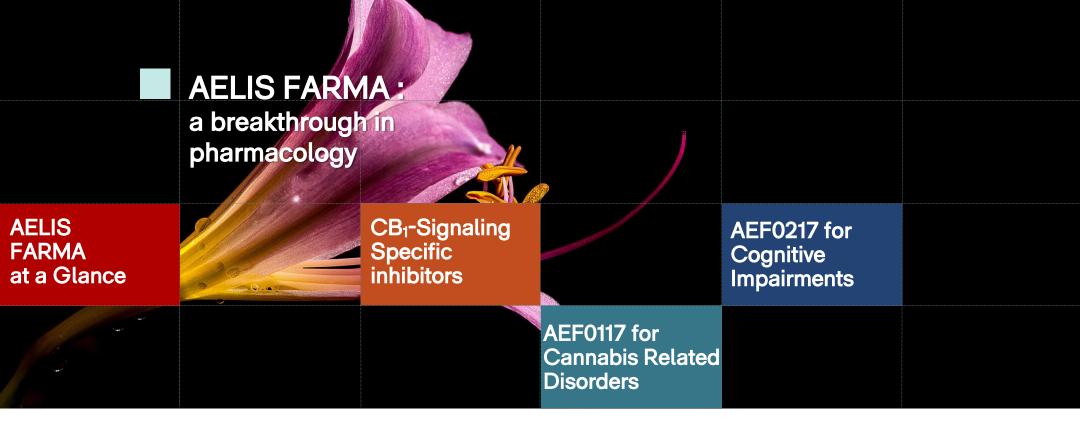


## Supported by the H2020 program of the EU





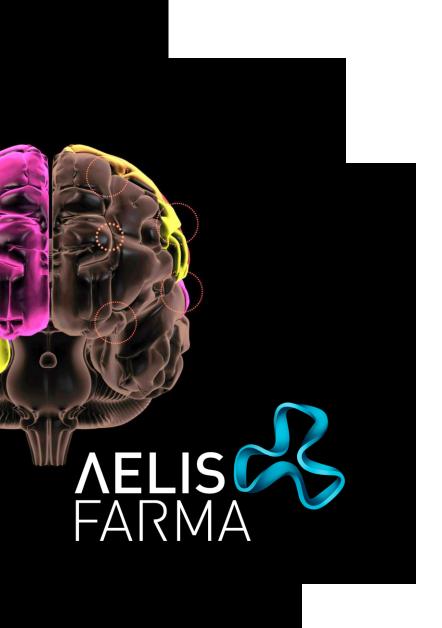






A paradigm shift in inhibitors pharmacology





# Thank you!

contact@aelisfarma.com