

6th International Prader-Willi Syndrome Caregivers' Conference



21-23 May 2024, Berlin, Germany

PBF-999: Potential New Therapeutic Option to Control Hyperphagia in PWS

Dr. Santiago Figueroa

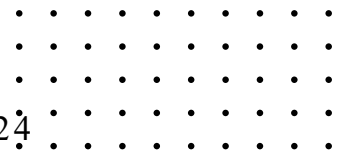
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#PWSCARE24



PBF-999: Potential New Therapeutic Option to Control Hyperphagia in PWS



PBF-999 is a potent and selective inhibitor of the enzyme Phosphodiesterase 10, which is thought to be involved in central appetite regulation.



Currently in clinical Phase 2
Orphan Drug Designation granted by FDA
and EMA



Phase 3 to start in 2025

Rationale of PDE10 inhibitors in PWS



PDE is a class of enzymes, located within a cell, that degrades cyclic nucleotides (cAMP and cGMP).



Inhibitors of PDEs increases the concentration of cyclic nucleotides inside the cell.



Cyclic nucleotides are second messengers that convey the signal from a neurotransmitter in the cell surface to the cell's interior, to elicit a cellular response.



PDE10 is localized in the brain, mainly in striatum inhibitory neurons

**PBF-999 increases
cAMP and cGMP in
striatum neurons**



**Modulate
DOPAMINE
Signaling**

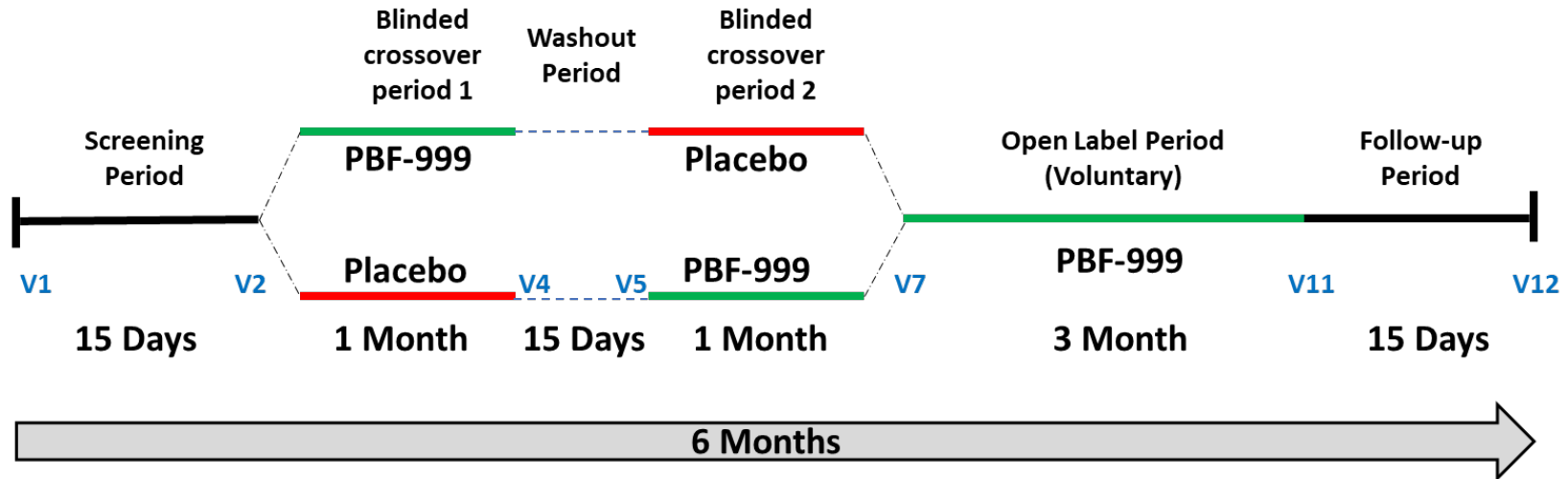


**Food reward
processing**



**Appetite
hormonal
regulation**

Phase 2 trial to investigate safety and efficacy of PBF-999 in patients with PWS



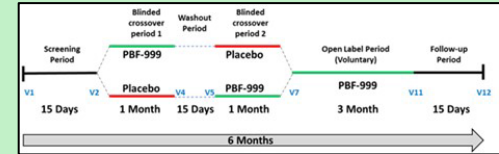
Phase 2 trial to investigate safety and efficacy of PBF-999 in patients with PWS

Objectives:

1. Investigate safety and tolerability of PBF-999 in patients with PWS
2. Explore the effect of PBF-999 in hyperphagia, behaviour, body weight and metabolic parameters

Measures

- Adverse Events
- HQ-CT Score
- CGIC (hyperphagia)
- Caregiver reported change in PWS specific behaviours
- Weight, Waist Circumference, etc.
- Clinical chemistry, etc



Phase 2 trial to investigate safety and efficacy of PBF-999 in patients with PWS

Site:

Parc Taulí University Hospital in Barcelona, Spain.

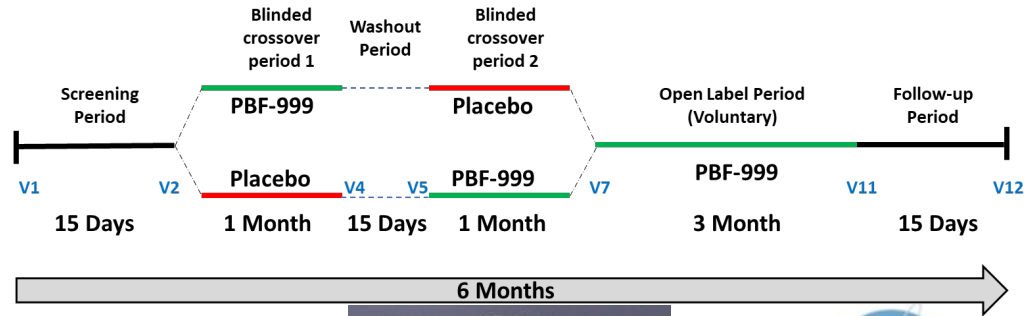
Principal Investigator:

Dr. Assumpta Caixàs,
Coordinator of PWS working group of Spanish Society of Obesity.

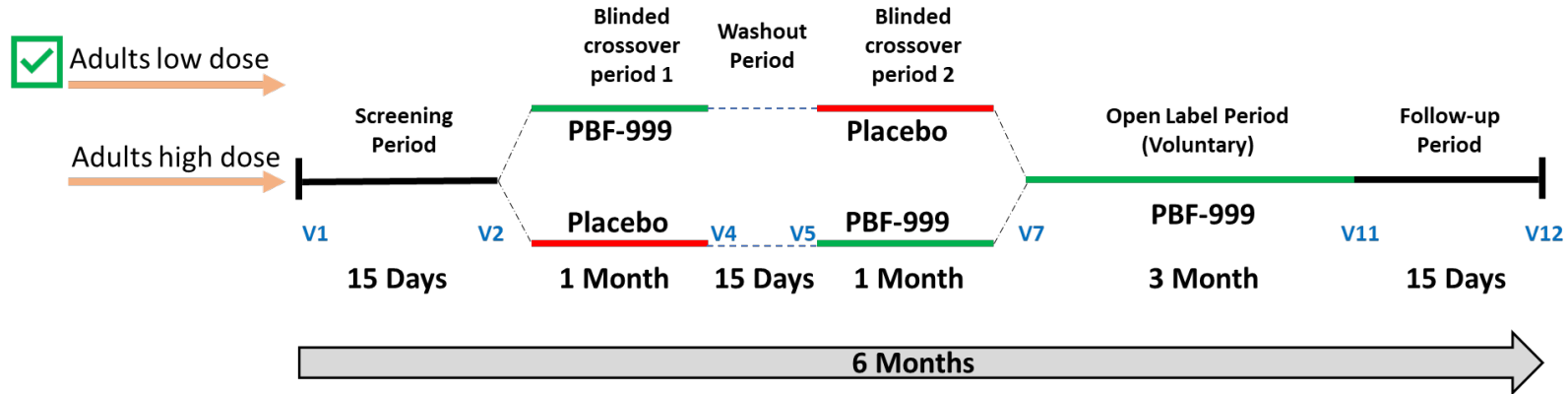
Sponsor: Palobiofarm S.L

Founding:

Palobiofarm and FPWR



Phase 2 trial to investigate safety and efficacy of PBF-999 in patients with PWS



2 sequential cohorts (10 patients each):

- Adult cohort PBF-999 low dose
- Adult cohort PBF-999 high dose

Future Plans PBF-999





Thank you

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