AN ADVANCED GENE THERAPY CLINICAL PLATFORM FOR ORPHAN GENETIC RETINAL DYSTROPHIES

HORAMA
THERAPIES FOR RETINOPATHIES

COMPANY PROFILE

HORAMA SA is a biotechnology company that was created in March 2014 as a spin-off from two Inserm units. its objective is to develop gene therapy products based on recombinant adeno-associated virus (rAAV) vectors for the treatment of inherited retinal dystrophies. Nonclinical and clinical studies have demonstrated that rAAVs are safe, well-tolerated, and efficacious. Horama has several products in development, one of which, HORA-RPE65, has shown long-lasting effects in patients in a completed phase I/II clinical study. Horama uses robust, efficient, and reproducible production processes. Horama combines its expertise in the vector development process with knowledge acquired from international experts in vectorology, genetics, and ophthalmology.

RECESSIVE RETINAL DYSTROPHIES (RRDs)

Recessive retinal dystrophies (rRD) include maculopathies and pigmentary retinopathies such as retinitis pigmentosa (RP). This orphan disease involves the progressive degeneration of retinal cells (mainly rod and cone photoreceptors, with or without the loss of retinal pigment epithelium cells) leading to gradual visual deterioration. RP is a collection of genetic diseases for which no approved treatment is available, and is the leading cause of hereditary blindness in developed countries.

HORAMA'S PIPELINE

INDICATION	US+EU5	PRODUCT	PROOF-OF-CONCEPT	NONCLINICAL	PHASE I/II	PHASE III	LAUNCH
PDE6B RP	3,600-5,400	HORA-PDE6B					
RLBP1 RD	900-1,200	HORA-RLBP1					
Undisclosed		HORA-XX					
RPE65 RP	2,750-3,300	HORA-RPE65					

HORAMA specialises in the development of non-pathogenic, serotype-specific recombinant adeno-associated viruses (rAAV) associated with a specific promoter, a combination that confers unique properties to our products.

- HORA-RPE65: HORA-RPE65 is a rAAV 2/4 encapsidating the human RPE65 gene under the control of the native human RPE65 promoter. In 2015, the team at Nantes University Hospital completed a monocentric, open-label, non-randomised, uncontrolled Phase I/II clinical trial evaluating the safety and efficacy of HORA-RPE65 in patients with retinal dystrophy caused by defects in RPE65.
- HORA-PDE6B is a rAAV2/5 vector containing the therapeutic human PDE6B transgene under the control of a specific promoter. In 2017, a Phase I/II clinical trial began in retinitis pigmentosa patients with PDE6B gene mutations and consequent defects in PDE6ß expression. No competing product has been described to date.
- HORA-RLBP1, another candidate therapeutic product, is a rAAV2/5 carrying a copy of the human RLBP1 gene and the CAG promoter, and is designed for the treatment of RLBP1 retinal dystrophy. A Phase I/II clinical trial is scheduled to end of 2018.
- Other candidate products remain confidential at this time.

SCIENTIFIC TEAM

- Internationally recognised experts working on gene therapy for retinopathies since 1997: Prof. M. Weber, Prof. C. Hamel†, and Prof. P. Moullier.
- Together, they formed the first team in France to manufacture a GMP clinical batch of rAAV and to complete a clinical gene therapy trial for an ophthalmic disease.

MANAGEMENT TEAM

- Christine Placet, Chief Executive Officer, ex-CEO of Trophos
- Dr. Jean-Yves Deslandes, Chief Medical Officer, ex-Novartis, ex-Sanofi, and ex-Allergan
- Dr. Philippe Moullier, Chief Scientific Officer, ex-Director of INSERM UMR 1089, Nantes

GENE THERAPY

HORAMA specialises in the development of gene replacement therapy products, designed to provide target cells with a non-mutated copy of a gene to replace the defective gene, thereby facilitating functional protein expression and ultimately delaying or halting retinal degeneration and preserving or improving visual function.

INTELLECTUAL PROPERTY

HORAMA has signed exclusive licensing agreements (INSERM Transfert) for several patent applications covering proprietary production processes and non-clinical and clinical data generated for drug candidates, as well as exclusive access to technology-related know-how. In addition, HORAMA holds EU and US orphan drug designations for the 3 HORAMA drug candidates.

FINANCIAL SUMMARY

- Seed investment (2014–1015): €0.8 M
- Series A (June 2016): €4.3 M
- Series B (November 2017): €19 M

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