• **Adenoassociated viral (AAV) vectors** are very promising tools for therapeutic gene delivery, since they are safe and they induce an efficient and long-term transduction.
• In collaboration with uniQure a gene therapy product for the treatment of Acute Intermittent Porphyria has been developed and is currently being evaluated in **phase I clinical trial**.
• A new **AAV expression system** has been developed and an **AAV production system** has been optimized.
  - AAV tetracycline-inducible system
  - Liver and brain specific expression.

### AAV vectors
- Safe, with no or only limited toxicity.
- Induce an efficient and long-term transduction in quiescent cells, a very important point to be effective in adult tissues.
- Genes carried by rAAV vectors have been efficiently transduced in skeletal muscle, heart, brain, joints, eyes and liver leading to stable expression at therapeutic levels.

### AAV-PBGD: gene therapy product for Acute Intermittent Porphyria (licensed to UniQure)
- In collaboration with the Dutch company uniQure, a gene therapy product for the treatment of Acute Intermittent Porphyria has been developed.
- Orphan designation approved.
- Grant from the EU’s FP7 program (AIPGENE consortium) to bring this product forward to completion of a Phase I/II study in humans.
- The product is currently being evaluated in **phase I clinical trial** at the Clínica Universidad de Navarra and Hospital 12 de Octubre.
- Phase II clinical trial will be started in the near future.
- **References:**

### AAV Platform
- A new AAV tetracycline-inducible system for liver and brain specific expression has been developed.
- Recombinant AAV viruses to express cytokines, shRNAs, antibodies and many other different genes have been constructed and produced for the treatment of infectious diseases, malignancies or hereditary metabolic disorders.
- Gene therapy products for rare diseases, hyperoxaluria and Wilson disease are under development.
- AAV vectors for different research groups at CIMA, Universidad de la Laguna, Universidad Complutense de Madrid, Centre Esther Koplovitz (CEK), San Raffaele Scientific Institute have been designed and produced.
- **Competitive Advantage:** Developed tools that allow designing therapeutic vectors according to the characteristics of the disease, such as tissue specificity or controlled expression.

### Intellectual Property
- PCT/NL2009/050584. Porphobilinogen deaminase gene therapy. *(Shared ownership Proyecto Biomedicina CIMA SL and UniQure).*

### References

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