TaRGeT's primary mission is to develop gene therapy strategies based on AAV (adenovirus-associated virus) vectors. The laboratory covers the entire translational development chain for these innovative medicines, from basic research through to clinical trials. The laboratory's teams are implementing projects to generate new, more effective viral vectors through molecular and chemical engineering, to develop retinal or muscle organoid models for in vitro evaluation, and to develop analytical methods for characterising vectors, such as high-throughput sequencing covering the entire viral genome. The laboratory's studies aim to develop treatments for neuromuscular and ophthalmological diseases (including certain retinal pathologies) and to understand the immunology of viral vectors with a view to developing appropriate immunomodulation strategies for patients. The laboratory is capable of producing batches of viral vectors of up to 50 litres, a feat achieved by the CPV (Centre de Production des Vecteurs) platform, which has been awarded the bioproduction integrator label as part of the France 2030 national plan. This label is a continuation of the label of industrial integrator of the major national challenge in bioproduction, which the laboratory shared with only five other academic players between 2020 and 2022.

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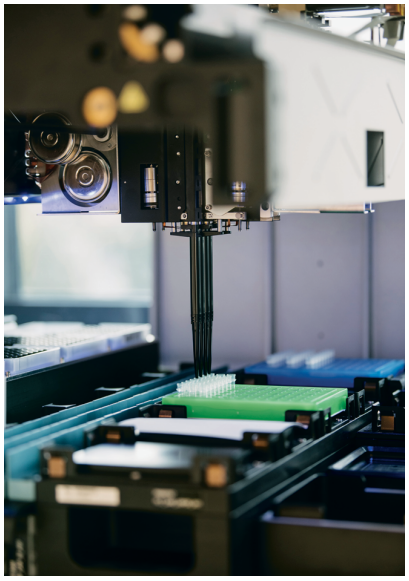

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The laboratory’s recognition through regional and national funding for excellence is enabling it to invest in cutting-edge equipment to develop the medicines of tomorrow. TaRGeT collaborates with other laboratories on the Nantes University

NExT site. This pooling of expertise is a welcome opportunity to develop treatments for as yet incurable non-genetic diseases such as osteoarthritis, to study the influence of gene therapy medicaments on the host immune system and to test new cellular systems for producing viral vectors, such as microalgae (a proof of concept is currently being developed with financial support from the ANR). Other collaborative projects involve robotics and process automation, the use of AI to improve the design of viral vectors and processes, etc.

Thanks to this multidisciplinary approach, TaRGeT is well placed to increase the effectiveness of viral vectors. This is essential if we are to reduce the doses injected and, consequently, inappropriate immune responses, not to mention the cost of certain treatments, in the absence of alternatives for rare and often incurable diseases such as spinal muscular atrophy. However, lower costs would make it possible to extend the field of application of gene therapies to more common diseases such as AMD. Exciting therapeutic prospects for patients!

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