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Mtm1 deficient rats as a new preclinical model for myotubular myopathy gene therapy

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A la recherche des enhancers inconnus: identifying satellite cell-specific enhancers for targeted gene therapy

Matthew Borok, *Univ Paris Est Creteil, INSERM, IMRB, F-94010 Creteil, France*

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Preclinical evaluation of AAV-U7snRNA Exon Skipping Targeting exon 44 and exons 6--8 demonstrates dystrophin rescue and functional benefit in humanized DMD mouse models

Alice Rannou, *TaRGeT Lab, Translational Research for Gene Therapy, INSERM, UMR 1089, Nantes Université, CHU Nantes*

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AAV cellular response is mediated by CD8⁺ effector memory T cells in healthy donors

Manon Schmitt, *UMR1089 TaRGeT*

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CRISPR/Cas9 and AAV-mediated gene correction approach for B-hemoglobinopathies

Alexia Spinu, *GENETHON - INSERM U951*

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Sebastien Paillusson, *TaRGeT UMR1089*

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Héloïse Delépée, *Nantes université*

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Laure Carré, *UMR1089- Nantes Université*

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Pauline Finard, *Généthon_UMR 951*

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Marie-Anne Burlot, *Coave Therapeutics*

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Banchi Elena, *ICM*

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Rafael Alonso, *Genov Institut du cerveau*

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Jeremy Rouillon, *GENETHON*

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Maïna Giri, *Nantes Université, CHU Nantes, INSERM, TaRGeT (Translational Research in Gene Therapy - UMR 1089)*

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Ninon Carlet, *Nantes Université, CHU Nantes, INSERM, TaRGeT – Translational Research In Gene Therapy, UMR1089, F-44200 Nantes, France*

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Sylvie Jacquot, *GENOV - Institut du cerveau*

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Wassila Salim, *Commissariat à l'Energie Atomique (CEA)*

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Ludmila JURICEK, *Coave Therapeutics*

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Nicolas Wein, *TaRGeT UMR1089*

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Intensification of rAAV Purification at the Capture Step Using Multi-Column Chromatography with the Resolute® BioSMB PD System

Maïlys Pennors, *Nantes Université, CHU Nantes, INSERM, TARGET, F-44000 Nantes, France*

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Establishing a robust and open-access rAAV manufacturing process for gene therapy within a major European partnership (ERDERA)

Youssef Krimi Benchekroun, *Généthon_UMR 951*

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Conditioning strategies to enhance functional maturation of human EMT (Engineered Muscle Tissues)

Morgane Biette, *Nantes Université, CHU de Nantes, INSERM, TaRGeT - Translational Research in Gene Therapy, UMR 1089, F-44200 Nantes, France*

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Streamlined Production of 105 Barcoded rAAV Capsid Variants for Pooled Evaluation in Non-Human Primate Retina

Jihane Challita, *Revvity Gene Delivery*

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Development of a High-Yield AAV Production Platform Using DOE and Automated Bioreactors

Flici Hakima, *Sartorius*

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Advanced pipeline for CRISPR/Cas9 off-targets detection in Guide-seq and related integration-based assays

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