15 September 2025
EMA/CAT/287112/2025
European Medicines Agency

CAT workshop on Gene Editing

With the support from the Alliance for Regenerative Medicines (ARM)

16 September 2025 (Virtual meeting)

Agenda

Morning Session (10.00 – 12.00)	**Chair: Ilona Reischl**	
Welcome and introduction	Steffen Thirstrup, Chief Medical Officer, EMA	10.00-10.05
	Ilona Reischl, CAT chair	10.05-10.10
	Tim Hunt; CEO, Alliance for Regenerative Medicine	10.10-10.15
1. Scope and aim of the workshop	Emmely de Vries	10.15–10.20
2. State of Art and common challenges		
Advancing Gene Editing Medicines from N=1 to N=many: the Unique Urgency and Opportunity of the Present Moment	Dr Fyodor Urnov, University of California, Berkeley, USA	10.20–10.45
3. Evaluation of safety concerns		
Uncovering and mitigating heterogeneity of genome editing outcome at ON and OFF target sites	Dr Luigi Naldini, San Raffaele Telethon Institute for Gene Therapy, Milan, Italy	10.45-11.10
Off-target assessment: how much is too much?	Dr Kiran Musunuru, Perelman School of Medicine at the University of Pennsylvania, USA	11.10-11.35
4. Question & Answer session		11.35-12.00

Lunch break (12.00-12.45)

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Afternoon session (Open part) (12.45-16.30)	**Chair: Marcos Timon**	
Platform-Based Genome Editing Strategy for Liver-Directed Integration of Therapeutic Transgenes via AAV-HITI and LNP-Cas9	Nicola Brunetti, Telethon Institute of Genetics and Medicine (TIGEM), Pozzuoli (NA), Italy	12.45-13.10
A platform genome editing medicinal product for inborn errors of metabolism	Kiran Musunuru and Rebecca Ahrens-Nicklas; Perelman School of Medicine at the University of Pennsylvania, and Children's Hospital of Philadelphia, Philadelphia, Pennsylvania, USA	13.10-13.35
Development of a Local non-viral CRISPR-Cas9 Therapy for Duchenne Muscular Dystrophy (DMD)	Niels Geijsen; LUMC, The Netherland	13.35-14.00
Risk-Appropriate Manufacturing for Personalized Gene Editing: Lessons from Baby KJ	Vanessa Almendro Navarro and Sadik Kassim, Danaher	14.00-14.25

Coffee Break (14.25-14.40)

Chair: Mike Lehmicke (ARM)		
ARM Contributions in the field of genome editing	Mike Lehmicke; SVP Scientific Affairs, Alliance for Regenerative Medicine	14.40-14.45
Challenges in Development of In Vivo Gene Editing Therapeutics	Kristy M Wood, Intellia	14.45-15.10
Base Editing Opportunities for Platform Development	Luis Barrera, Beam	15.10-15.35
Modular platform for in vivo LNP Formulated Prime Editors Targeting Rare Genetic Diseases of the Liver	Andrew Anzalone, Prime Medicine	15.35-16.00
Challenges in Gene Editing drug development	Pam Stetkiewicz, Arbor Biotechnologies	16.00-16.25
Closing Remarks	Ilona Reischl	16.25-16.30

End of the open part of the meeting

Afternoon session (Closed part[1])	**Chair: Ilona Reischl**	
Gene-Edited Cell-Based Medicinal Products -Key Insights and scientific challenges	Xiagoang Guo and Maria Rathmann Sørensen, Novo Nordisk	16.35-17.00

End of the closed part of the meeting

[1] The closed part is only open to experts and participants of Regulatory Authority