Alliance for Regenerative Medicine

Leveraging Data via Platform Principles in Gene Editing

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Scientific Exchange 2024

Exchanging Information on Gene Editing Platforms for Rare Diseases

Platform Objective

Streamlining therapeutic development for rare conditions that are **scientifically feasible but commercially non-viable** through **repeatable gene editing platforms** across multiple therapies.

Developers / CROs

- charles river
- Beam Therapeutics
- prime medicine
- arbor biotechnologies
- CRISPR Therapeutics
- verve Therapeutics

Academic

- Innovative Genomics Institute
- Penn
- NIH National Institute of Allergy and Infectious Diseases
- Harvard University

- FDA
- European Medicines Agency
- MHRA Regulating Medicines and Medical Devices

Outcomes

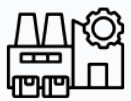
Peer-reviewed publications summarizing **streamlining opportunities**

- Consistent delivery vehicle
- Consistent manufacturing
- Benefit-risk appropriate GMP
- Innovative umbrella clinical trial design

- ✓ 5-fold material efficiency gains
- ✓ Decrease time required to dose patients: 6 months timeframe

Call by Agency leadership for a "proactive, not reactive, regulatory framework for gene editing."

Scientific Exchange – A Proactive Regulatory Framework for Gene Editing

Gene editing case study themes		Opportunities
 The delivery vehicle primarily drives biodistribution including germline, and toxicology	›	 Streamline animal studies - and retain focus on *in silica* on- and off-target editing studies
 Fixing manufacturing (same location, equipment, process, starting materials, etc.) yields consistent process characterization	›	 Consistent process characterization enables streamlining of process validation steps
 Enrolling patients in a single trial (same phenotypical disease but differing genotypes) will yield efficiencies…	›	 …in enrollment, clinical trial protocols, dosing, sites, and long-term follow up – and is patient-friendly for fragmented rare disease populations
 GMP requirements for guide RNA should be more precisely tailored for stage of development and benefit-risk of target population	›	 Limiting # of batches of gRNA required within and across patients would substantially increase drug development efficiency
 If validated, streamlining opportunities from gene editing platforms could translate into material efficiency gains (2x – 5x)		 These gains present opportunity to address additional rare disease therapeutic targets that are currently not viable

Regulatory Solutions

- Define criteria for a gene editing platform (e.g., fixed delivery system, validated editing enzyme, modular gRNAs)
- Enable cross-referencing of prior INDs for CMC, preclinical, and clinical modules across related therapies
- Develop evidentiary guidelines for expanding platforms across new indications or patient populations
- Allow master protocol approaches with shared clinical infrastructure and long-term follow-up