Chemically Modified CRISPR crRNA for Advanced Gene Editing Applications

GENE THERAPY, ONCOLOGY

McGill

2 Provisional patents
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TRL 3

Pre-clinical stage
In vitro editing efficacy completed

Business Opportunity:
Licensing or Co-development

Market Opportunity:
Global market: gRNA market ~3.17 billion USD by 2034
CAGR: 18% for gRNA

TIMELINE

Chemically modified crRNAs synthesized	In vitro cell-based validation of gene-editing capability	Strong mechanistic rationale and differentiation vs unmodified crRNAs	Optimal patterns for full modification are close but not yet selected. No in vivo efficacy, PK, or safety data	Head-to-head benchmarking vs industry-standard crRNAs

THE PROBLEM

CRISPR-based gene editing faces persistent limitations related to RNA instability, off-target effects, immune activation, and toxicity, which restrict therapeutic applicability.

CRISPR-Cas9 and CRISPR-Cas12a systems, both of which we have made significant progress in, offer unique features for development but their guide RNAs remain highly susceptible to nuclease degradation, limiting in vivo performance. There is a strong unmet need for next-generation crRNA chemistries that improve stability and performance of Cas9 and Cas12a systems while maintaining editing efficiency and low toxicity.

OUR SOLUTION

The developped technologies are chemically modified CRISPR-Cas12a and CRISPR-Cas9 crRNAs designed to enhance RNA stability, reduce toxicity, and preserve high gene-editing efficiency.

The invention combines specific chemical modifications at defined positions of the crRNA backbone, including:
2'-AraOH, 2'-5'-RNA, 2'F-ANA, 2'-OMe, 4'-OMe, 4'-F-RNA, 2'-Fluoro, and phosphorothioate (PS)

These modifications are used in combinatorial and position-specific patterns to:

- Protect crRNA from exonuclease degradation
- Maintain or enhance editing efficiency
- Reduce off-target effects and cellular toxicity

Newly synthesized modified crRNAs have been validated in vitro for gene-editing performance.

MARKET

Target users include:
- CRISPR reagent manufacturers
- Gene-editing biotechnology companies
- Pharmaceutical companies developing CRISPR-based therapeutics

Multiple commercialization pathways:
- Sale of modified crRNA products
- Licensing of proprietary crRNA chemistry
- Integration into CRISPR platform companies

- The gRNA market includes products and services and should reach 3.17 billion USD by 2034, accelerating through a high CAGR (18%) for the period.
- North America represents 54% of this market, reaching 323 million USD in 2024.
- The research-use segment dominates the gRNA market and the "custom gRNA synthesis services" segment is expected to grow significantly.
- The GMP-grade segment, required for CRISPR-based therapeutics or clinical trials, is also expected to grow significantly.
- The gRNA market is highly influenced by the CRISPR market, which is expected to increase at 15.6% growth rate (CAGR) between 2023 and 2028.
- In 2028, the segment of the services should reach 1.6 billion USD and represent 23% of the CRISPR market.

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