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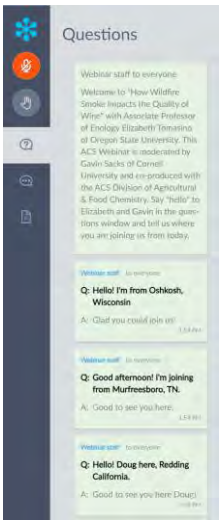

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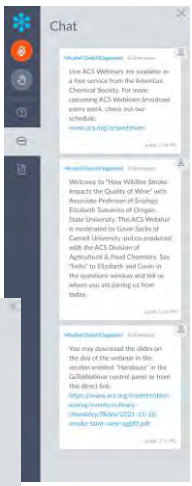

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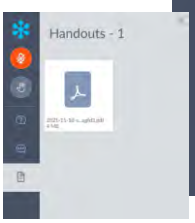

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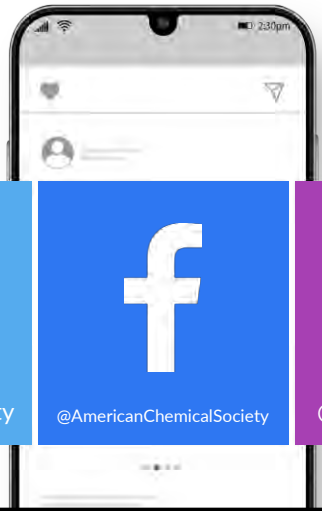

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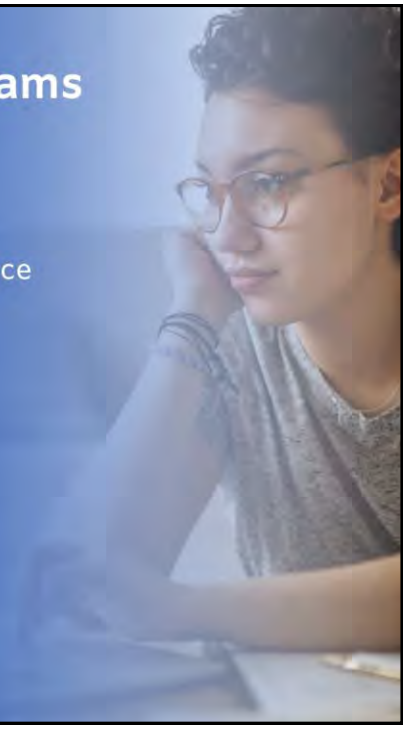

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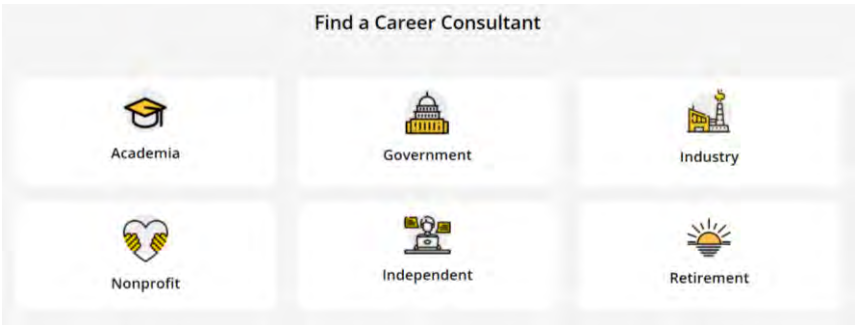

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RNA Therapeutics: The Evolving Landscape of Research, Challenges, and Opportunities

BARBARA AMBROSE
Senior Information Scientist, CAS

JOHN P. COOKE
Medical Director, Center for RNA Therapeutics, and Chief Translational Science Officer, Houston Methodist Research Institute

ROBERT DELONG
Associate Professor, Nanotechnology Innovation Center Kansas State, Kansas State University

RAMANA DOPPALAPUDI
Vice President, Avidity Biosciences

GILLES GEORGES
Vice President & Chief Scientific Officer, CAS, a division of ACS

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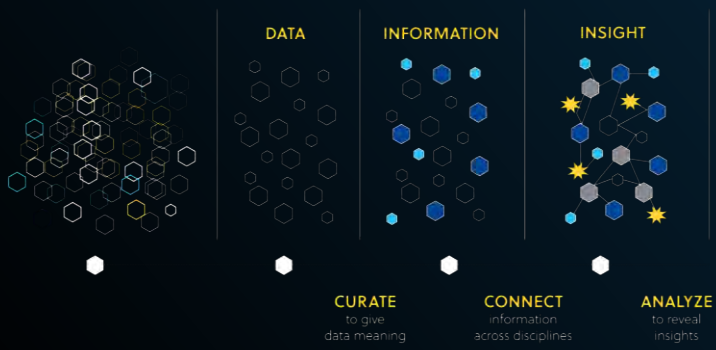

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CAS Biosequence database

Over 72 M sequences and 550 M sequence-patent relationships

Many databases treat chemistry and biologic sequences separately

Searching biologics with the chemical differences (partial alignments, modifications and patterns)

Connecting them reveals deeper insights

Maximizes discovery and patentability

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Expert Panelists & Moderator

Barbara Ambrose
Information Scientist, CAS

Robert DeLong
Assoc Prof., Kansas State University

Ramana Doppalapudi
VP, Avidity Biosciences

John Cooke MD
Chief Translational Science Officer, Houston Methodist

Gilles Georges (Moderator)
Chief Scientific Officer, CAS

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The Next Revolution

Starts with curing the uncurable

85%

Proteins undruggable by conventional medicines*

RNA

- Programmable
- Versatile
- Targeted

*https://www.drugdiscoverytrends.com/what-makes-something-undruggable/

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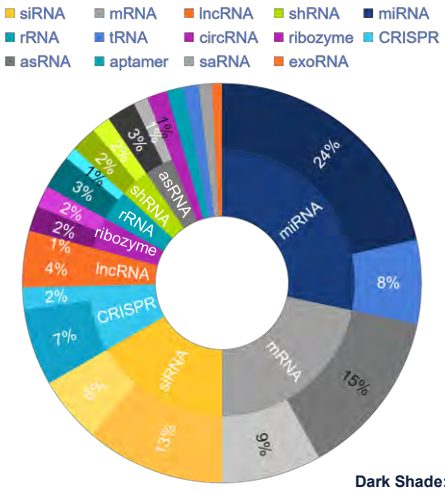
Beyond COVID mRNA vaccines

Many types of RNA are potential therapeutics



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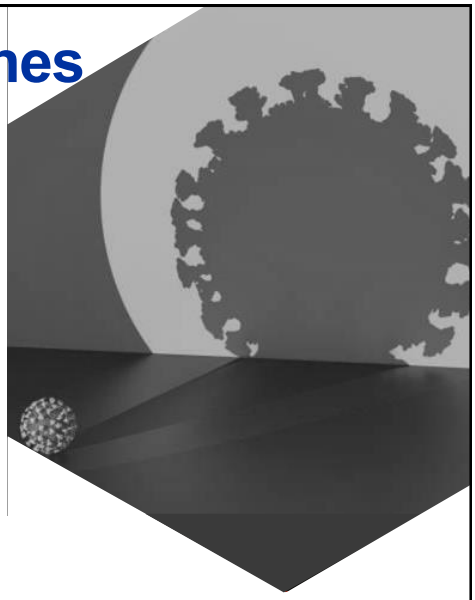

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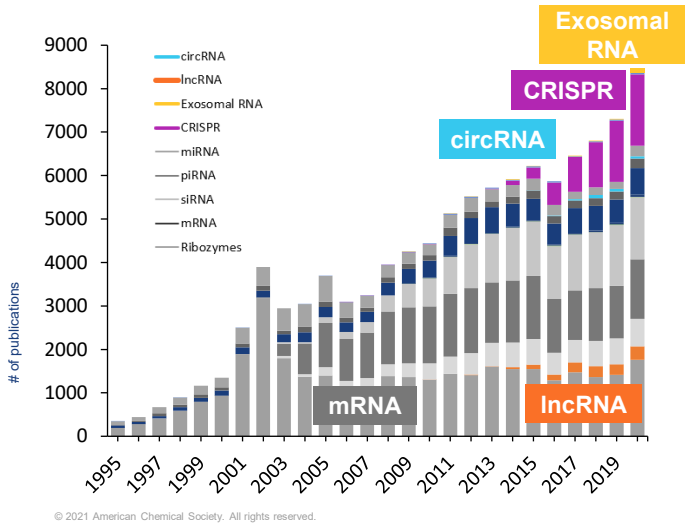
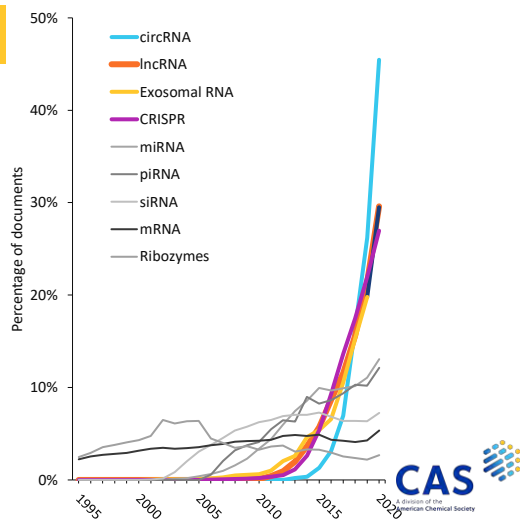
RNA research trends reveal key insights

Diversification of RNA technology and rapid growth in 4 emerging areas





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CRISPR, AOC opportunities ahead while aptamer and miRNA are active in the clinic

With a robust pipeline in key therapeutic areas like blood disease and cancer

	Preclinical	Active	Completed
CRISPR	100%	0%	0%
AOC	86%	14%	0%
saRNA	50%	50%	0%
mRNA	38%	37%	21%
siRNA	38%	32%	24%
ASO	16%	48%	21%
Aptamer (RNA)	5%	26%	63%
miRNA	0%	22%	67%

	Preclinical	Active	Completed
Respiratory Disease	58%	33%	8%
Autoimmune Disease	50%	33%	17%
Blood Disease	50%	31%	13%
Liver Disease	46%	38%	8%
Infectious Disease	43%	25%	29%
Neurological and Neuromuscular	38%	38%	5%
Metabolic Disease	33%	24%	24%
Other*	33%	0%	67%
Wound healing	33%	0%	67%
Cardiovascular Disease	28%	34%	31%
Cancer	26%	48%	23%
Eye Disease	20%	35%	35%
Kidney Disease	14%	36%	36%
Transplantation	0%	0%	100%

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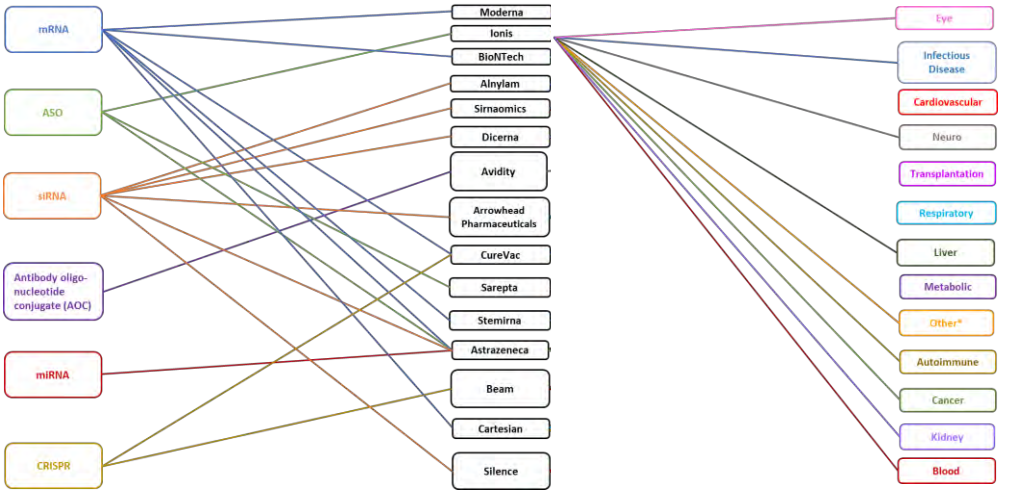
The versatility and targetability of RNA is diverse across therapeutic areas

siRNA and mRNA are most utilized



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Critical challenges

That are slowing the growth of RNA therapeutics

Specificity

Delivery & Stability

Tolerability

Carrier Systems

Modifications

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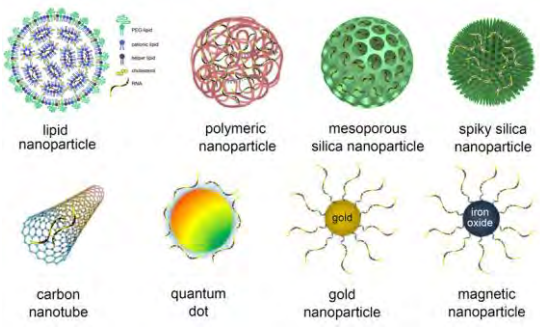
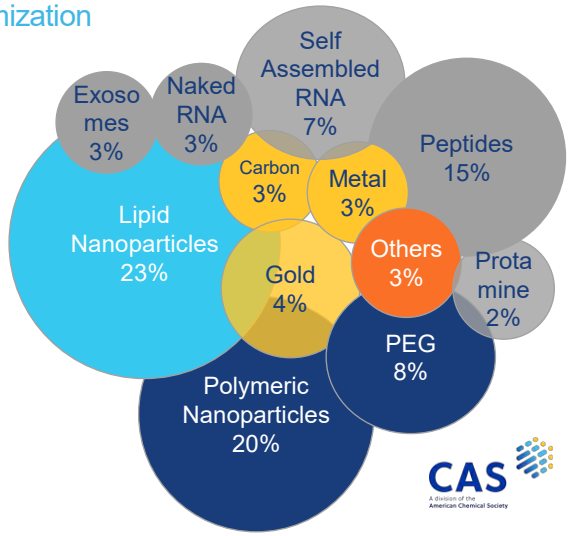
Carrier systems are optimized for deliverability and tolerability challenges

Nanoparticle technology is the key area for optimization





https://pubs.acs.org/doi/10.1021/acsnano.1c04996

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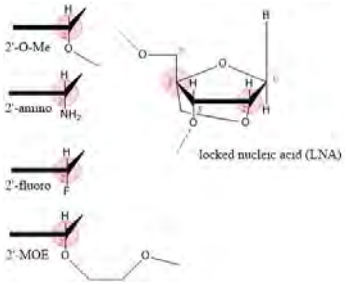
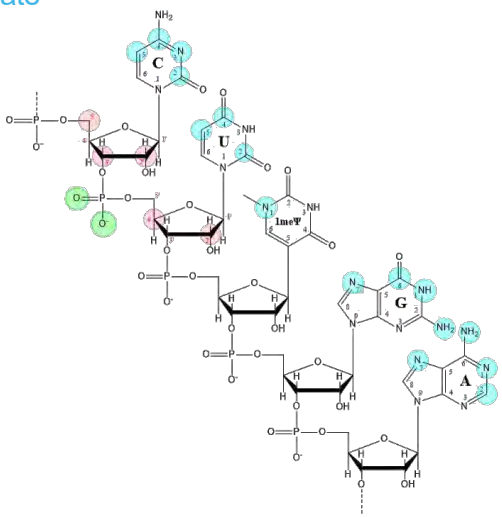
Locations of modifications on RNA

Nucleic acid base, ribose and phosphate

$CU_{1me}\Psi GA$





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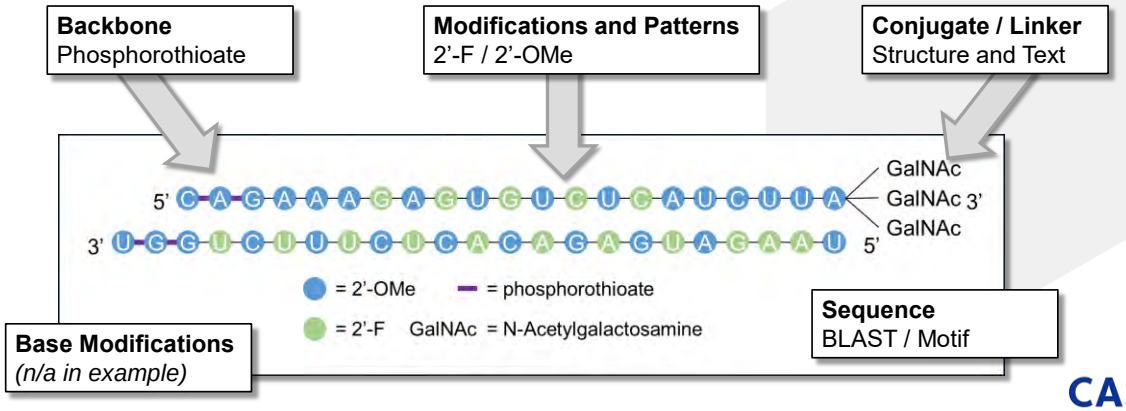
CAS indexes sequence modifications and patterns

Ex: Givlaari™ (givosiran – Alnylam)



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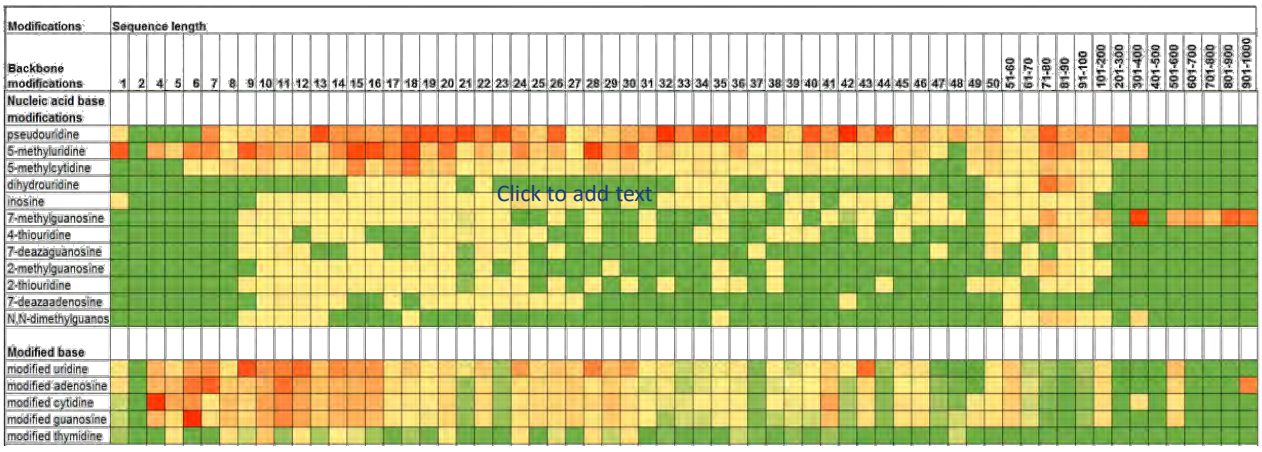
Modified nucleic acid bases at different sequence lengths



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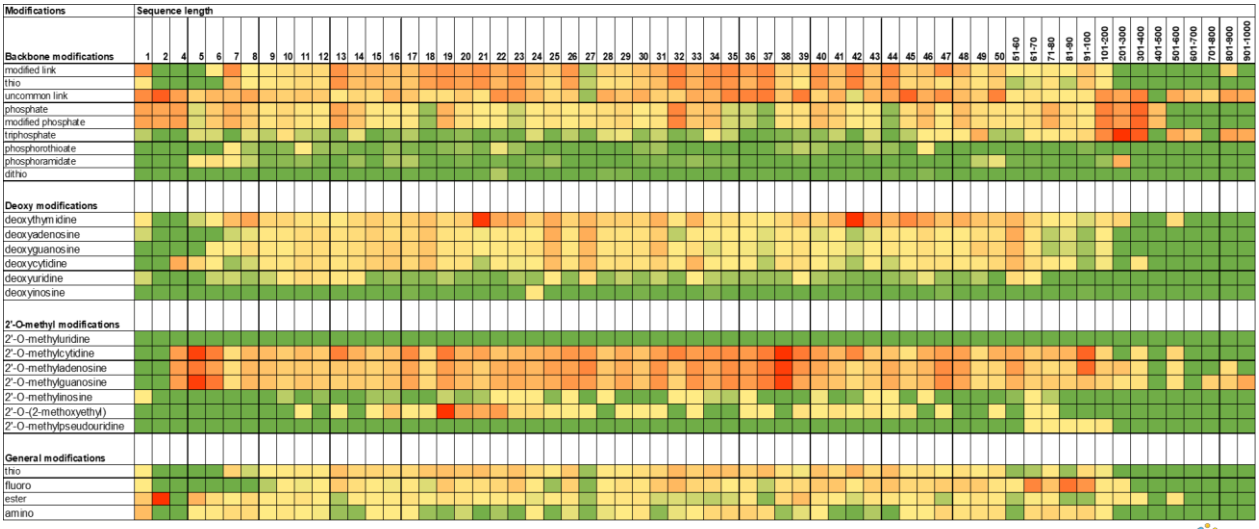
Modified nucleotides at different sequence lengths



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Opportunities Ahead

To accelerate RNA development and success

- RNA sequence representation
- Data Delivery Mechanisms
- Modification and carrier description

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Characterizing RNA nanoscale interactions and the potential of zinc nanoparticle (ZNP) composites

R. K. DeLong, M.S., PhD
Department of Anatomy and Physiology,
Nanotechnology Innovation Center,
College of Veterinary Medicine,
Kansas State University,
Manhattan, KS 66506

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Career timeline

Johns University PhD 1988-1994 — NIH National Research Service Award

UNC-CH School of Medicine 1994-1997 — NIH F-31 Post-doctoral Award

1999-2001 — GeneMed Scientist/ Senior Sci

2001-2004 — PowderJect Vaccines Group leader/ manager

GSK Bio-Analytical award

2004-2006 — Teaching Chemistry And Biology

2007-08 — HHMI Summer Research Fellow

NIH R15 — NSF MRI — NIH R15

2009-2014 — MSU Biomedical Sciences

US Patent 8,349,364

Tenure

US Patent 9,977,016

CF Foundation Pilot Award

K-State 2014-**current…**

NIH R15 — NIH R03 — NSF COVID-19 RAPID

First in vivo ASO paper: Comparative pharmacokinetics, tissue distribution, and tumor accumulation of phosphorothioate, phosphorodithioate, and methylphosphonate oligonucleotides in nude mice. DeLong RK, Nolting A, Fisher M, Chen Q, Wickstrom E, Kligshteyn M, Demirdji S, Caruthers M, Juliano RL. ***Antisense Nucleic Acid Drug Dev.*** 1997 Apr;7(2):71-7. doi: 10.1089/oli.1.1997.7.71.PMID: 9149842

First in vivo lipid nanoparticle paper: pDNA bioparticles: comparative heterogeneity, surface, binding, and activity analyses. P Medberry, S Dennis, T Van Hecke, RK DeLong. ***Biochemical and Biophysical Research Communications*** 319 (2), 426-432 (2004)

First in vivo nucleic acid nanoparticle paper: R4 peptide-pDNA nanoparticle coated HepB vaccine microparticles: sedimentation, partitioning, and spray freeze dry bioprocesses. Knowle R, Werner A, **DeLong RK. J Nanosci Nanotechnol**. 2006 Sep-Oct;6(9-10):2783-9. doi: 10.1166/jnn.2006.427.PMID: 17048483

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Health disparity: mRNA vaccine instability

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University announces research collaboration to improve COVID-19 vaccine stability

Wednesday, Jan. 5, 2022

Robert DeLong, right, associate professor at the university's Nanotechnology Innovation Center of Kansas State, with second-year veterinary medicine student Hanah Huber. DeLong and other K-State researchers are part of a research collaboration with Tonix

Sources
Robert DeLong
785-532-6313
robertdelong@vet.k-state.edu
Waithaka Mwangi
785-532-5994
wmwangi@vet.k-state.edu

Websites
tonixpharma.com
nicks.ksu.edu

Written by
Erin Pennington
785-532-5110
erin120@k-state.edu

At a glance
Kansas State University has an exclusive license and option agreement and research collaboration with Tonix Pharmaceuticals. Through the partnership, researchers will develop zinc nanoparticle, or ZNP, mRNA vaccines that replace the lipid nanoparticle, or LNP, technology in current COVID-19 vaccines.

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Some opportunities and challenges

- n=14 RNA therapies clinically approved
- Many in the pipeline

Our lab

- mRNA, TFO and aptamer

Challenges:

- Formulation
- Characterization
- Stability
- In vivo delivery

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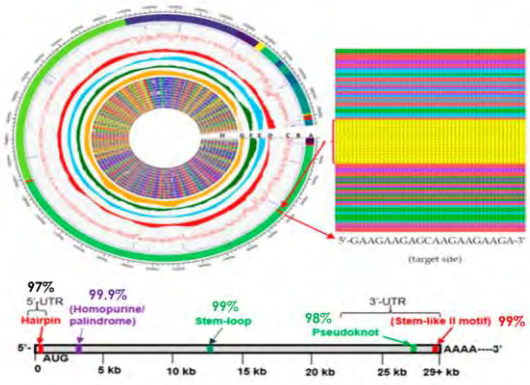
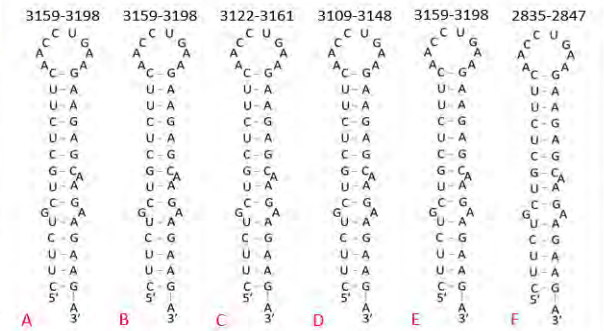
Targeting SARS-CoV-2 RNA





A – Wuhan reference, B – B.1.1.7/alpha UK variant, C – B.1.351/beta South Africa variant D – B.1.617.2/delta India variant, E – P.1/gamma Brazil variant, F – B.1.1.529/omicron Kenya variant

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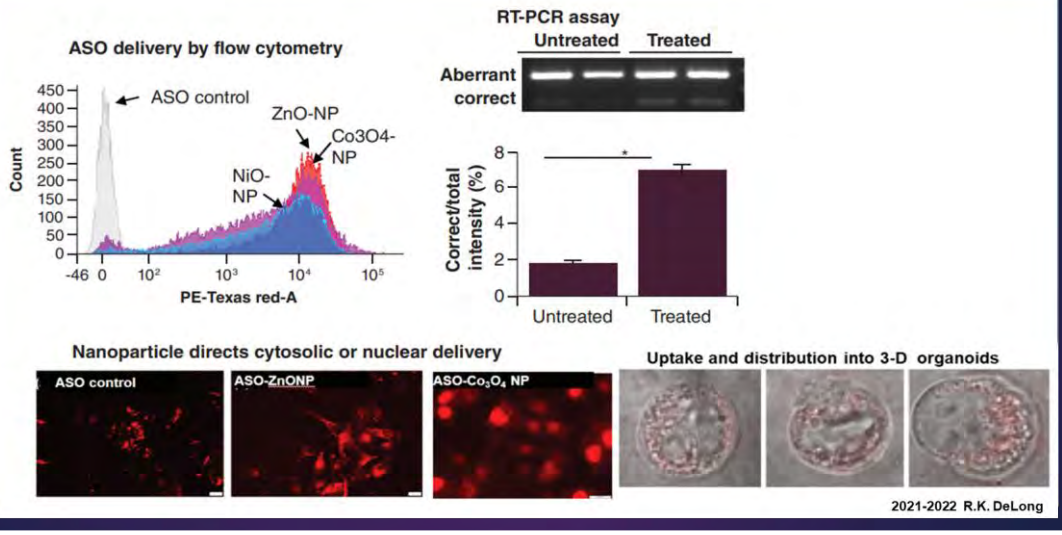
ASO delivery



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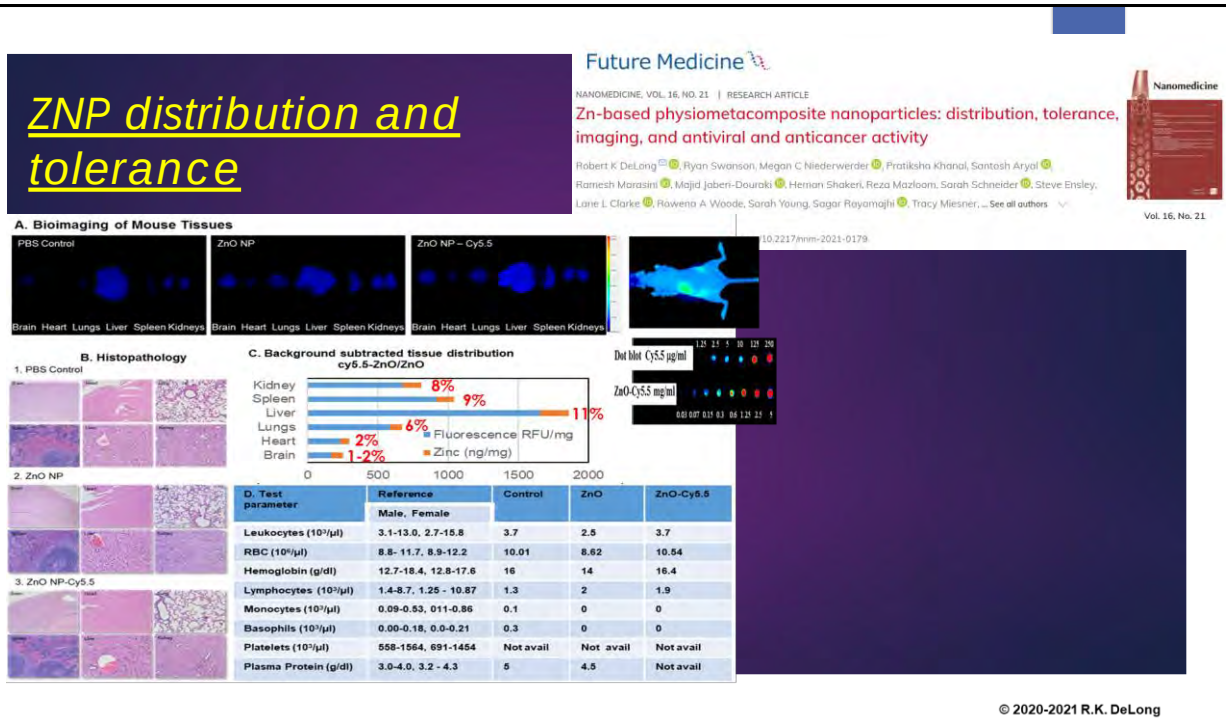
ZNP distribution and tolerance



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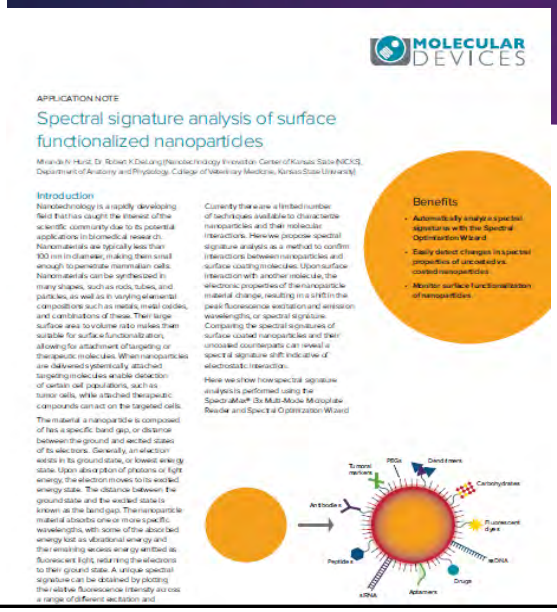
Quantifying nanoscale interactions


MOLECULAR DEVICES

APPLICATION NOTE

Spectral signature analysis of surface functionalized nanoparticles

Miranda N. Hurst, Dr. Robert K DeLong (Nanotechnology Innovation Center of Kansas State (NICKS), Department of Anatomy and Physiology, College of Veterinary Medicine, Kansas State University)

Introduction

Nanotechnology is a rapidly developing field that has caught the interest of the scientific community due to its potential applications in biomedical research. Nanomaterials are typically less than 100 nm in diameter, making them small enough to penetrate mammalian cells. Nanomaterials can be synthesized in many shapes, such as rods, tubes, and particles, as well as in varying elemental compositions such as metals, metal oxides, and combinations of these. Their large surface area to volume ratio makes them suitable for surface functionalization, allowing for attachment of targeting or therapeutic molecules. When nanoparticles are delivered systemically, attached targeting molecules enable detection of certain cell populations, such as tumor cells, while attached therapeutic compounds can act on the targeted cells.

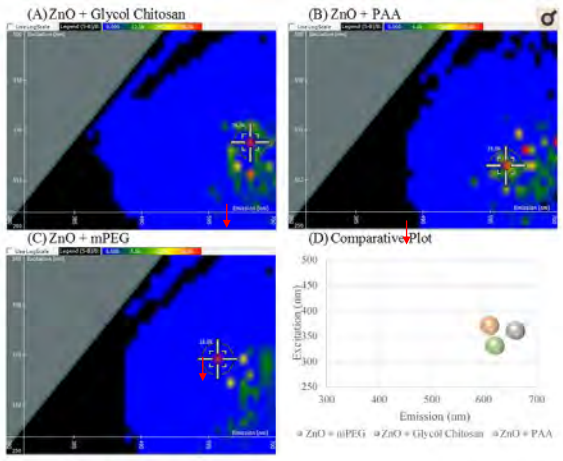
The material a nanoparticle is composed of has a specific band gap, or distance between the ground and excited states of its electrons. Generally, an electron exists in its ground state, or lowest energy state. Upon absorption of photons or light energy, the electron moves to its excited energy state. The distance between the ground state and the excited state is known as the band gap. The nanoparticle material absorbs one or more specific wavelengths, with some of the absorbed energy lost as vibrational energy and the remaining excess energy emitted as fluorescent light, returning the electrons to their ground state. A unique spectral signature can be obtained by plotting the relative fluorescence intensity across a range of different excitation and

Currently there are a limited number of techniques available to characterize nanoparticles and their molecular interactions. Here we propose spectral signature analysis as a method to confirm interactions between nanoparticles and surface coating molecules. Upon surface interaction with another molecule, the electronic properties of the nanoparticle material change, resulting in a shift in the peak fluorescence excitation and emission wavelengths, or spectral signature. Comparing the spectral signatures of surface coated nanoparticles and their uncoated counterparts can reveal a spectral signature shift indicative of electrostatic interaction.

Here we show how spectral signature analysis is performed using the SpectraMax® i3x Multi-Mode Microplate Reader and Spectral Optimization Wizard.

Benefits

- Automatically analyze spectral signatures with the Spectral Optimization Wizard
- Easily detect changes in spectral properties of uncoated vs. coated nanoparticles
- Monitor surface functionalization of nanoparticles



Spectral Signature Shift upon Surface Functionalization of zinc oxide (ZnO) nanoparticle as a result of polymer association.

U.S., Patent No. 9,977,016. Issued on May 22, 2018. Inventors: **Robert K. DeLong**, and Miranda Hurst. Two Dimensional Fluorescence Difference Spectroscopy Characterization of Nanoparticles and their Interactions. Manhattan, KS (US), Assignee: Kansas State University Research Foundation, Manhattan, KS (US).

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Collaborators

@K-State

Jürgen A. Richt
Regents Distinguished Professor & KBA Eminent Scholar
Director
Center of Excellence for Emerging and Zoonotic Animal Diseases (CEEZAD), www.ceezad.org
Director
NIH COBRE Center on Emerging and Zoonotic Infectious Diseases (CEZID)
www.k-state.edu/cezid

Majid Jaberi-Douraki
Associate Professor of Mathematics and Data Science
Co-Lead
1Data

Dr. Kartik C. Ghosh
Physics, Astronomy, and Materials Science
Distinguished Professor
The University of Texas at TYLER

Santosh Aryal

Dr. Mwangi
Diagnostic Medicine/Pathobiology

Natasha N Gaudreault

Megan C. Niederwerder

Biomedical Sciences
University of Missouri
College of Veterinary Medicine
University of Missouri
Lane L. Clarke, DVM, PhD

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Acknowledgments

Pictured (right to left):
Ryan Swanson (VRSP),
Elza N. Mathew (PhD)
Me,
Tej Shrestha (lab manager)
Sarah Wilson (M.S.)
Sunyoung Yoon (DVM)
Sarah Devader (Undergrad)

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Funding

NIH National Institutes of Health Turning Discovery Into Health — NIH P20GM130448, 1R03EB025566-01/REB025566A, 7R15CA139390-03.

NSF National Science Foundation WHERE DISCOVERIES BEGIN — NSF 2029579

CYSTIC FIBROSIS FOUNDATION
- CF Foundation C00066519-1

- Johnson Cancer Research Center ZF0042-GVAP900042, A00-1185-001 (ZF0038), A00-0682-001 (ZF0022)

Other early support:
hhmi Howard Hughes Medical Institute
aps American Physiological Society

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Dr. Ramana Doppalapudi
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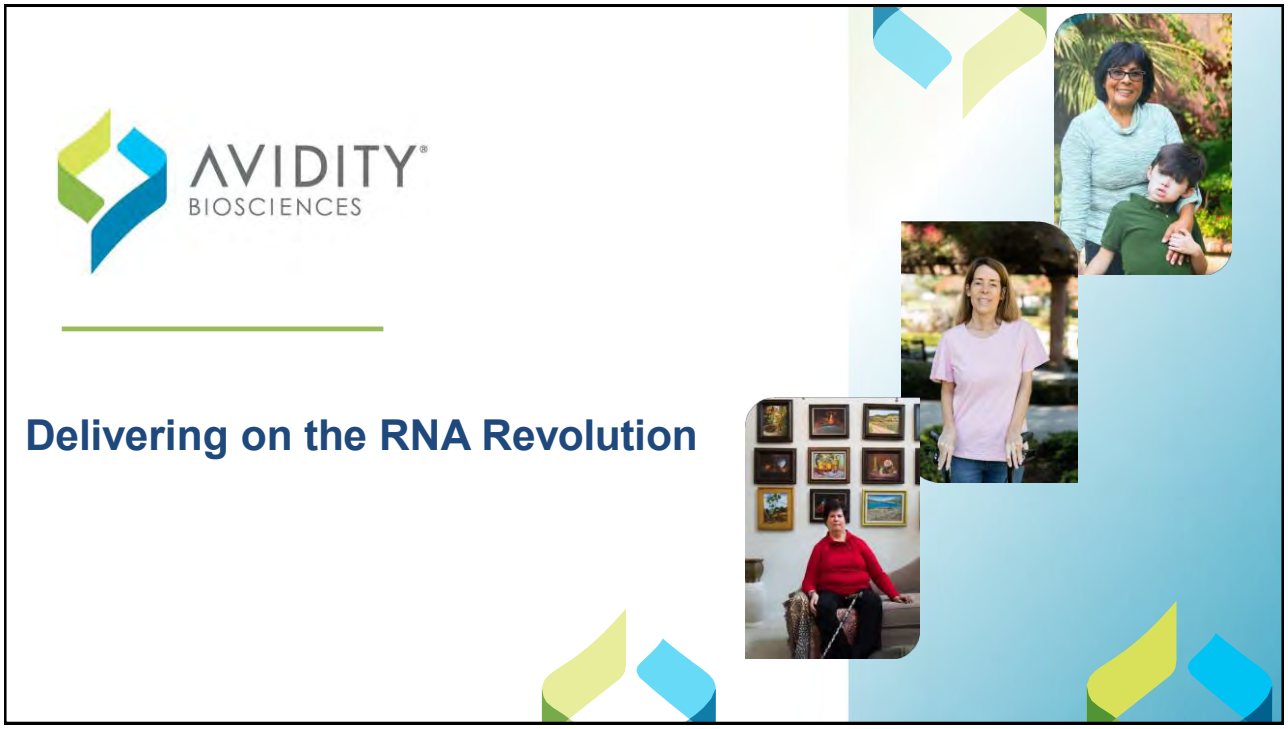
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Delivering on the RNA Revolution

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Forward Looking Statements

We caution the reader that this presentation contains forward-looking statements that involve substantial risks and uncertainties. All statements other than statements of historical facts contained in this presentation, including statements regarding our future results of operations and financial position, business strategy, the anticipated timing, costs, design and conduct of our ongoing and planned preclinical studies and planned clinical trials, research and development plans, timing and likelihood of success, prospective products, product approvals, plans and objectives of management for future operations, and future results of anticipated product development efforts, are forward-looking statements. In some cases, the reader can identify forward-looking statements by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target," "project," "contemplates," "believes," "estimates," "predicts," "potential" or "continue" or the negative of these terms or other similar expressions. The inclusion of forward-looking statements should not be regarded as a representation by Avidity that any of our plans will be achieved. Actual results may differ from those set forth in this presentation due to the risks and uncertainties inherent in our business, including, without limitation: we are early in our development efforts and many of our development programs are in the preclinical or discovery stage; our approach to the discovery and development of product candidates based on our AOC platform is unproven, and we do not know whether we will be able to develop any products of commercial value; the success of our preclinical studies and clinical trials for our product candidates; the results of preclinical studies and early clinical trials are not necessarily predictive of future results; potential delays in the commencement, enrollment and completion of clinical trials; our dependence on third parties in connection with preclinical testing and product manufacturing; disruption to our operations from the COVID-19 pandemic; unexpected adverse side effects or inadequate efficacy of our product candidates that may limit their development, regulatory approval and/or commercialization, or may result in recalls or product liability claims; regulatory developments in the United States and foreign countries, including acceptance of INDs and similar foreign regulatory submissions and our proposed design of future clinical trials; our ability to obtain and maintain intellectual property protection for our product candidates and proprietary technologies; we may use our capital resources sooner than we expect; and other risks described in our filings with the SEC, including under the heading "Risk Factors" in our Form 10-K for the year ending on December 31, 2020, filed with the SEC on March 15, 2021, and any subsequent filings with the SEC. The reader is cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof, and except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise. All forward-looking statements are qualified in their entirety by this cautionary statement, which is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995.

This presentation also contains estimates and other statistical data made by independent parties and by us relating to market size and growth and other data about our industry. This data involves a number of assumptions and limitations, and the reader is cautioned not to give undue weight to such estimates. In addition, projections, assumptions, and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk. These and other factors could cause results to differ materially from those expressed in the estimates made by the independent parties and by us.

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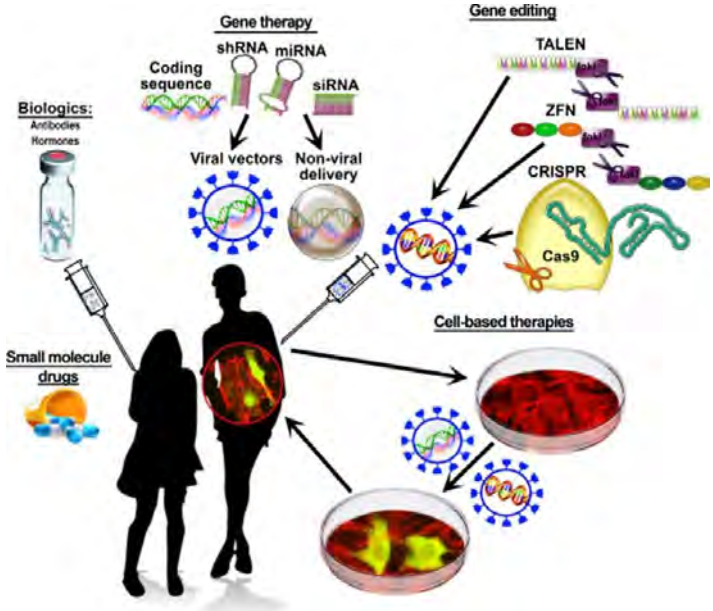
Modern Therapeutic Tools & Approaches



British Journal of Pharmacology, Volume: 172, Issue: 13, Pages: 3229-3241, First published: 09 January 2015, DOI: (10.1111/bph.13066)

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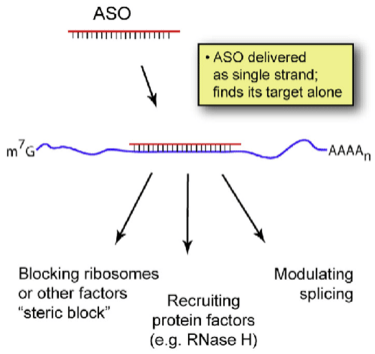
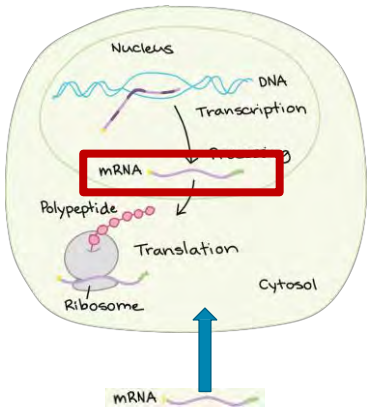
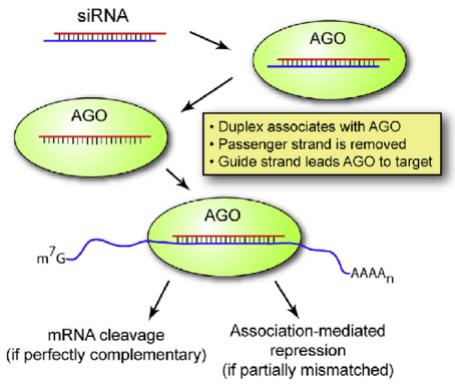
RNA Therapeutics Modulate Protein Production



Approved drugs
Steric Blocker: Fomivirsen
Degrader (RNase H): Mipomersen, Inotersen, Volanesorsen
Splice modulator: Eteplirsen, Nusinersen, Golodirsen, Viltolarsen, Casimersen



Exogenous mRNA
Approved Drugs:
Pfizer-BioNTech COVID-19,
Moderna COVID-19



Approved drugs
Degrader (RNAi): Patisiran, Givosiran

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Delivery is Key for RNA Therapeutics

- RNA therapeutics are rapidly degraded or cleared and show poor pharmacokinetics and biodistribution
  - Chemical modification of oligonucleotides greatly improved stability in circulation
- Limited cellular uptake due to their hydrophilic nature and size (~5 to 15 kDa)
- Hepatic delivery of RNA therapeutics is improved by lipid nanoparticles and N-acetylgalactosamine (GalNAc) conjugates
- Delivery to non-hepatic tissues is a major problem to be solved

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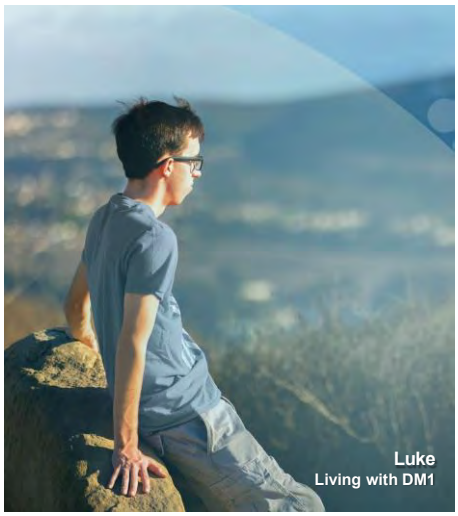
Our Vision



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To profoundly improve people's lives by revolutionizing the delivery of RNA therapeutics

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Avidity's AOC™ Platform

A Potential New Class of RNA Therapeutics

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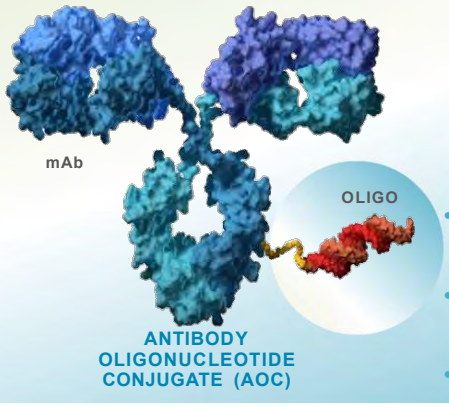
AOCs - A Powerful Potential New Class of Drugs

Utilizing decades of proven science in an effort to deliver the power of oligonucleotides



- Designed to combine the proven and safe technologies of approved monoclonal antibodies and oligonucleotides
  - ✓ Specificity of targeting with mAbs
  - ✓ Potency & precision of oligonucleotides
  - ✓ Targets tissues with durable agents
- Designed to deliver to tissues previously untreatable with RNA therapeutics
- Focused first on muscle, broadening to other tissues (i.e. cardiac) and cell types (i.e. B Cells)
- Readily scalable with many experienced manufacturers

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Advancing our Muscle Disease Franchise of AOCs

PROGRAM / INDICATION	TARGET	DISCOVERY / LEAD OPTIMIZATION	IND ENABLING	PHASE 1/2
MUSCLE DISORDERS				
AOC 1001: Myotonic Dystrophy Type 1 (DM1)	DMPK			
AOC 1020: Facioscapulohumeral Muscular Dystrophy (FSHD)	DUX4		Clinical trial initiations planned for 2022	
AOC 1044: Duchenne Muscular Dystrophy (DMD)	Exon 44 Dystrophin		Clinical trial initiations planned for 2022	
Next AOC DMD Programs	Exon 51 Dystrophin			
	Exon 45 Dystrophin			
AOC Muscle Atrophy: Muscle Atrophy*	MuRF1			
AOC Pompe Disease: Pompe Disease	GYS1			

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* Opportunity for a rare disease indication

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Myotonic Dystrophy Type 1 (DM1): Disease Overview

>40,000
PEOPLE WITH DM1 IN THE US

0
APPROVED THERAPIES

- DM1 is a complex disease with symptoms that present with high variability from patient to patient
- Monogenic, autosomal dominant, progressive disease that primarily affects muscle: skeletal, cardiac & smooth
- Increases in severity from generation to generation
- Significant impact on quality of life
- Shortened life-expectancy

"Some days I don't have the energy to take another step."
Karin, Living with DM1

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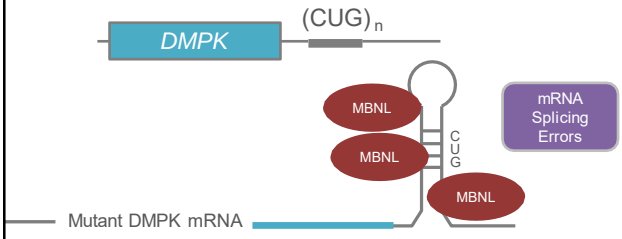
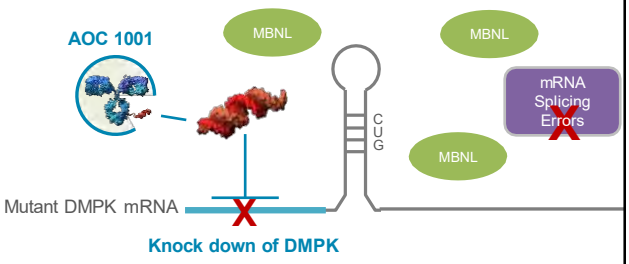
DM1, Caused by a Toxic Gain-of-Function mRNA, is Well Suited to an siRNA Approach

MECHANISM OF DISEASE



THERAPEUTIC APPROACH



- Trinucleotide expansion in DMPK mRNA sequesters an RNA splicing protein MBNL (Muscleblind like) in nuclear foci.
- Sequestration of MBNL leads to RNA splicing errors in multiple muscle-related RNAs and induces DM1 disease manifestations.
- Allows MBNL to be released to perform its natural function to aid in splicing key mRNAs in muscle
- Improves the splice patterns and muscle function. Splice patterns can serve as biomarkers.

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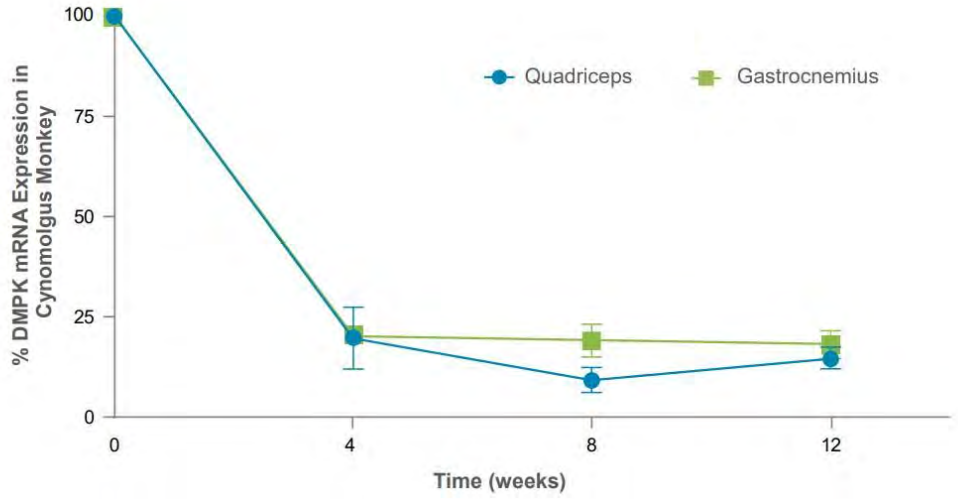
Durable ~75% Reduction of DMPK mRNA Observed in Monkey Skeletal Muscles After a Single Dose of 2mg/kg of siDMPK.19



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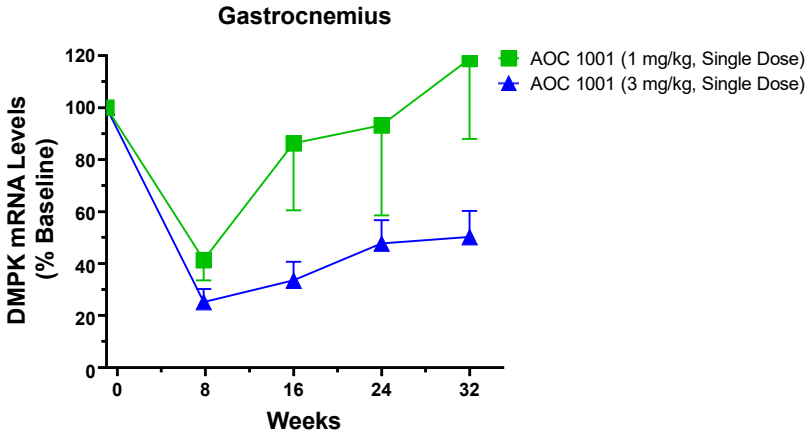
Durable Reductions in DMPK mRNA in Monkey Skeletal Muscle were Dose Dependent



Dose expressed as mg/kg siRNA

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AOC 1001 Has Been Engineered to Optimize Potential Therapeutic Profile

- ✓ Delivered RNA product candidate to skeletal muscle *in vivo*
- ✓ Reduced target mRNA in a broad range of muscles in a dose dependent manner *in vivo*
- ✓ EC50s in muscle biopsies were in the nM range
- ✓ Activity after a single dose continued for months *in vivo*
- ✓ Favorable toxicology results that support clinical development plans
- ✓ US Patent No. 10,881,743 for AOC 1001 issued in January 2021

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DELIVERING ON DM1

PHASE 1/2 MARINA TRIAL ONGOING

MARINA preliminary assessment planned for Q4 2022

FDA & EMA granted Orphan Designation

FDA granted Fast Track Designation

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Dr. John P. Cooke

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DEMOCRATIZING RNA THERAPEUTICS

John P. Cooke MD PhD
Medical Director, Center for RNA Therapeutics
Professor and Chair, Dept of Cardiovascular Sciences
Houston Methodist Research Institute

Mar 3, 2022

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TEXAS MEDICAL CENTER
AN EPICENTER FOR RNA THERAPEUTICS?

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- TMC is largest medical center in the world
- > 50 research and medical entities with >100,000 employees
- Rich and diverse scientific community with dense network of collaboration
- Growing strength in RNA Biology.

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HOSPITAL-BASED RNA THERAPEUTICS

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cGMP RNA synthesis
Plasmid generation
Research constructs

Plasmid Vector Design of Novel Construct

Proprietary chemistry for IVT RNA synthesis

RNA Purification

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DEMOCRATIZING RNA THERAPEUTICS

WE WILL HELP YOU DEVELOP, MANUFACTURE, DELIVER AND TEST NOVEL RNA THERAPIES

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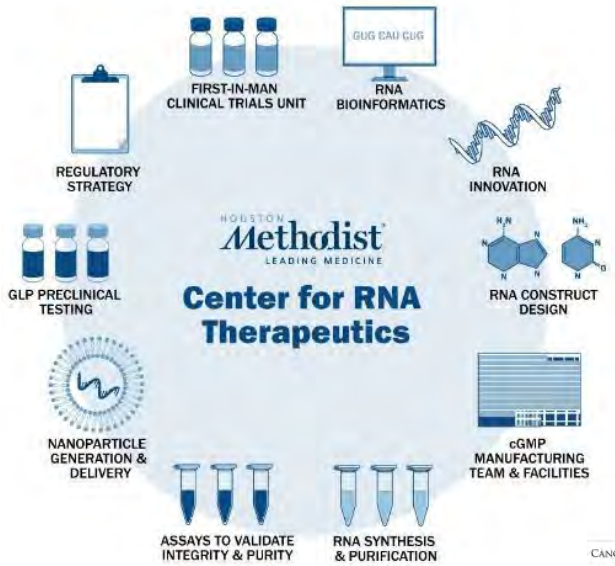

CANCER PREVENTION & RESEARCH INSTITUTE OF TEXAS

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RNA THERAPEUTICS

SUPPORTING CANCER INVESTIGATORS IN TEXAS AND BEYOND

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Within past 5 years:

- Manufactured for 8 small biotech companies
- Supported clinical trials with analytical services for 3 biotech startups
- Consulted for 10 companies on RNA design, manufacturing, assessment, and delivery
- Designed and developed over 100 unique RNA constructs
- Fulfilled over 300 orders from more than 40 different clients
- Provided over 80 analytical services
- Published 26 manuscripts

CPRIT CANCER PREVENTION & RESEARCH INSTITUTE OF TEXAS

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DEMOCRATIZING RNA THERAPEUTICS

ROADMAP TO THE CLINIC

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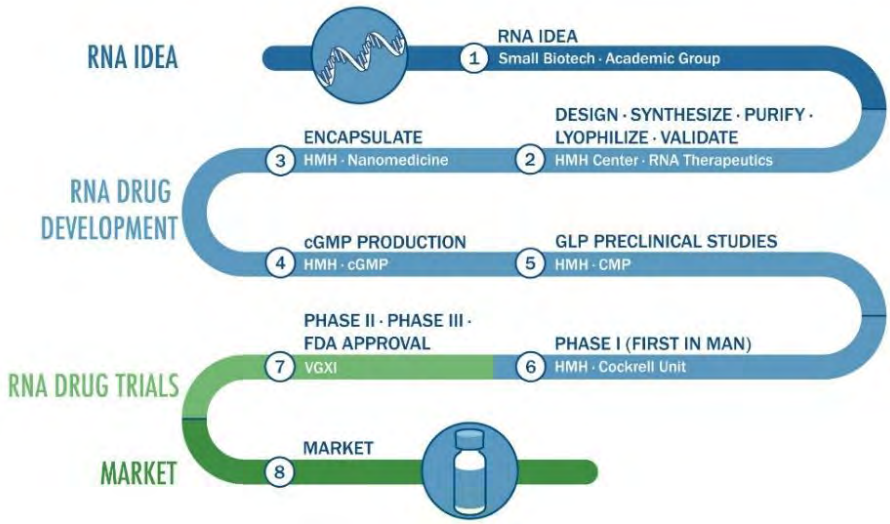

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PARTNER IN RNA MANUFACTURING

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Partner in RNA manufacturing
HMH: Innovation, Development
VGXI: Large batch manufacturing

HMH will generate RNA for Phase 1-2a Clinical Trials

VGXI will generate large batches for Phase 2b-3 and commercialization

VGXI breaks ground on 44 acre site in Conroe TX, Deison Technology Park, 11-9-20

L-R: CEO Young Park; Dr. John Cooke; VP Operations Dorothy Pederson

ENHANCING RNA STABILITY AND TRANSLATION

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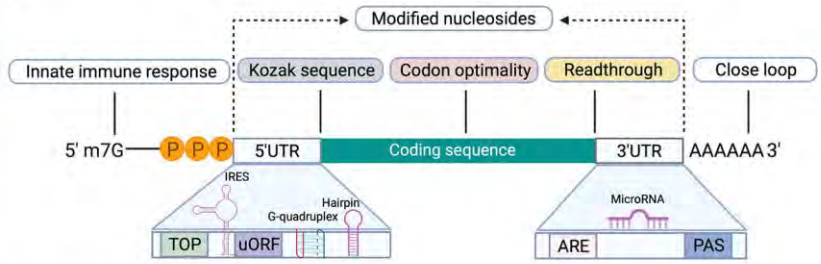
A Key elements of synthetic mRNA



B Optimization

Cap	5'UTR	ORF	3'UTR	Poly(A) tail
1. Higher capping efficiency using enzymatic capping 2. Natural Cap1 to avoid innate immune response	1. Including Kozak sequence 2. Avoid secondary structure or uORF 3. > 20 nt	1. Optimized codon that increase mRNA half-life and translation efficiency	1. Tandem stops to prevent stop codon readthrough 2. Avoid ARE or microRNA binding site 3. Short length	1.100-120 nt 2. Unmasked or mixed poly(A) that impede mRNA degradation
	Modified nucleosides to avoid innate immune activation			

Jia and Qian, *Acc. Chem. Res.* 2021

INNOVATION IN RNA THERAPEUTICS

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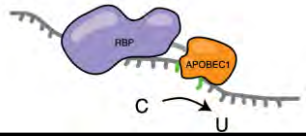
Characterizing RNA binding proteins(RBPs)

- STAMP(Surveying Targets by ApolipoproteinB MRNA Editing Enzyme Catalytic Subunit1-Mediated Profiling)
- Efficiently detects RBP-RNA interactions.
- Paired with long-read sequencing yields RBP target sites
- Ribo-STAMP leverages small ribosomal subunits to measure transcriptome-wide ribosome association in single cells

Kristopher Brannan PhD,
Assistant Professor,
Center for RNA Therapeutics
NIH K22; RR220017 CPRIT
Recruitment of First-Time Tenure
Track Faculty grant



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INNOVATION IN RNA THERAPEUTICS

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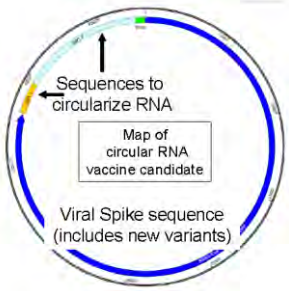
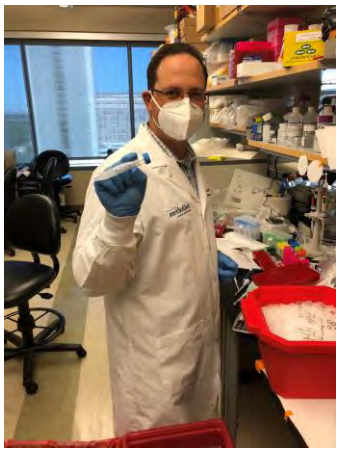
Circular mRNA

- mRNA degraded from ends
- Circular RNAs don't have ends and are harder to destroy
- Longer RNA lifespan = increased chance of effectiveness





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DELIVERY OF RNA THERAPEUTICS
NANOMEDICINE AT HMH

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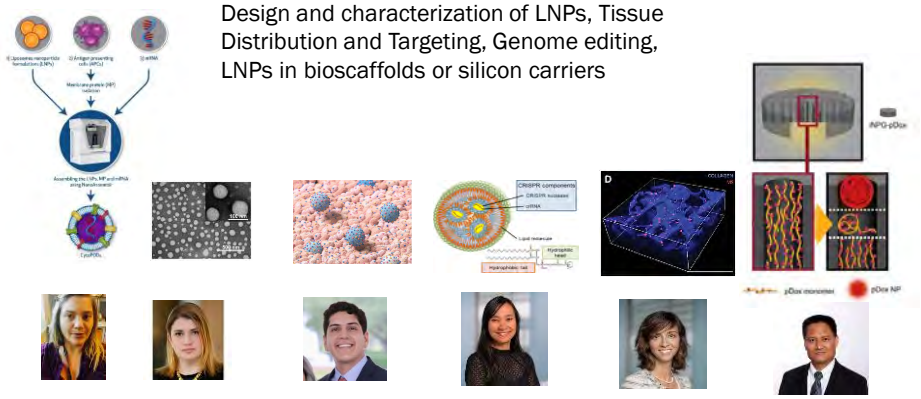
Design and characterization of LNPs, Tissue Distribution and Targeting, Genome editing, LNPs in bioscaffolds or silicon carriers



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LEUKOSOMES TARGET INFLAMED TISSUE

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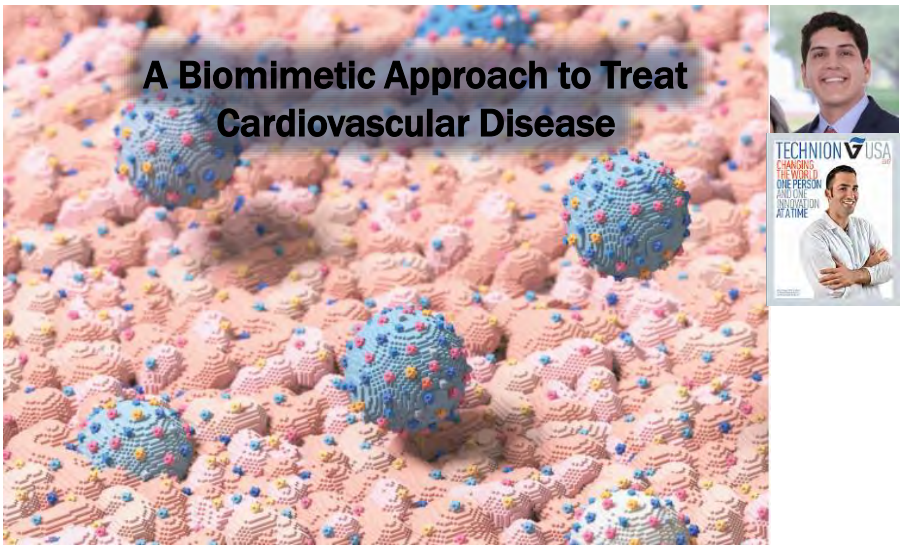

Boada C, Zinger A, et al, Circulation Research 2020

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SOME EXAMPLES OF PRODUCTS WE HAVE GENERATED

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What follows is a few of the >100 RNA drugs that we have generated internally or for other academic groups and companies

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MELANOMA VACCINE

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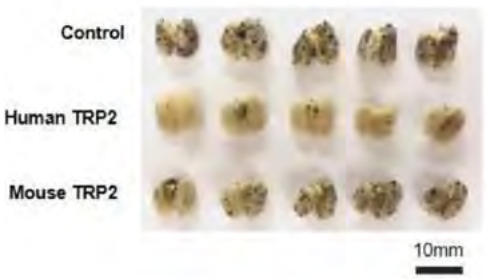

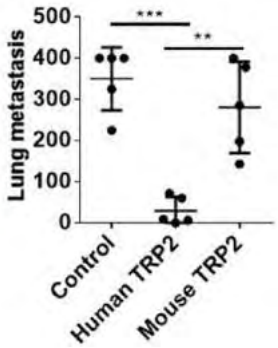

Courtesy, Rong Fu Wang, HMH

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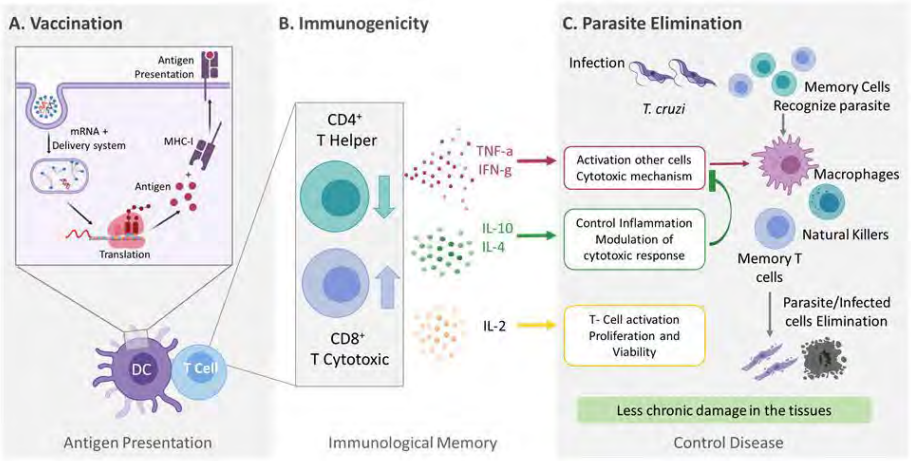
CHAGAS' DISEASE VACCINE



Chagas' Disease Vaccine: Jeroen Pollet, Peter Hotez, Roman Sukhovershin

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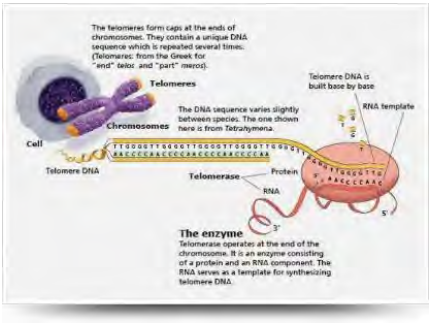
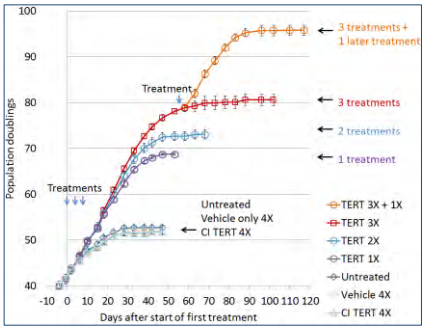
mRNA REGENERATIVE THERAPY

mRNA hTERT Restores Telomere Length, Replicative Capacity and Cell Functions





- We have extended telomeres of human adult cells
- Increased telomere length = increased replicative capacity
- Cells with longer telomeres function like young cells

Ramunas et al, FASEB 2015

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hTERT ENHANCED SKIN PRODUCT

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avita medical
transforming lives

Corporate partner working with us to improve their disaggregated skin product for burn patients

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RNA THERAPEUTICS AT HMH

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Roman Sukhovershin
Dan Kiss
Yi-lan Weng
Malgorzata Kloc
Kristopher Brannan
Nhat Tu Le
Longhou Fang
Guangyu Wang
Francisco Altimarano
Biana Godin
Francesca Taraballi
Bruna Coradetti
Keith Youker

City of Hope — Zhen Chen, Yingjun Luo

BCM Baylor College of Medicine — Malcolm Brenner, Peter Hotez, Jeroen Pollet

UTHealth The University of Texas Health Science Center at Houston — Louise McCullough

NIH R01HL148016; R01HL132155; R01HL149303; R01HL133254; Progeria Research Foundation, CPRIT RP150611

COME JOIN US!
Faculty positions in RNA biology and chemistry
jpcooke@houstonmethodist.org

Rod Pettigrew
Abhishek Jain
Michael McShane

Helen Blau
Wing Wong
Phillip Yang
Nick Leeper
Ngan Huang

BU Boston University — John Conner

The University of Texas MD Anderson Cancer Center, Making Cancer History® — Junichi Abe

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Continue exploring
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Articles
Case Studies
Journal of Medicinal Chemistry
Emerging Opportunities in RNA Therapeutics
accepted & publishing soon!
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THANK YOU

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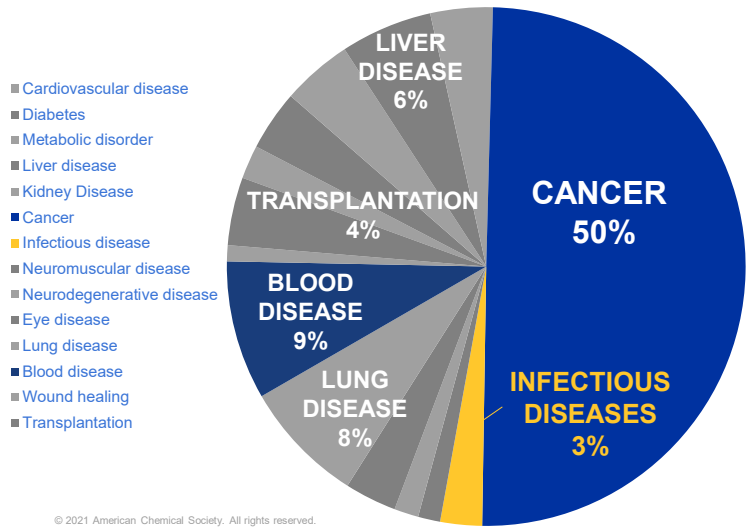
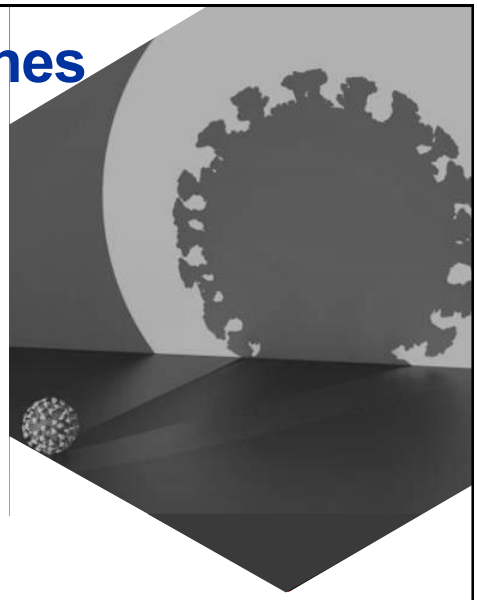
Beyond COVID mRNA vaccines

Many types of RNA are potential therapeutics





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Opportunities Ahead

To accelerate RNA development and success

- Better Data Transferability (?)
- Improved Delivery Mechanisms
- New Chemical Modifications

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