Common Fund Venture Program Update

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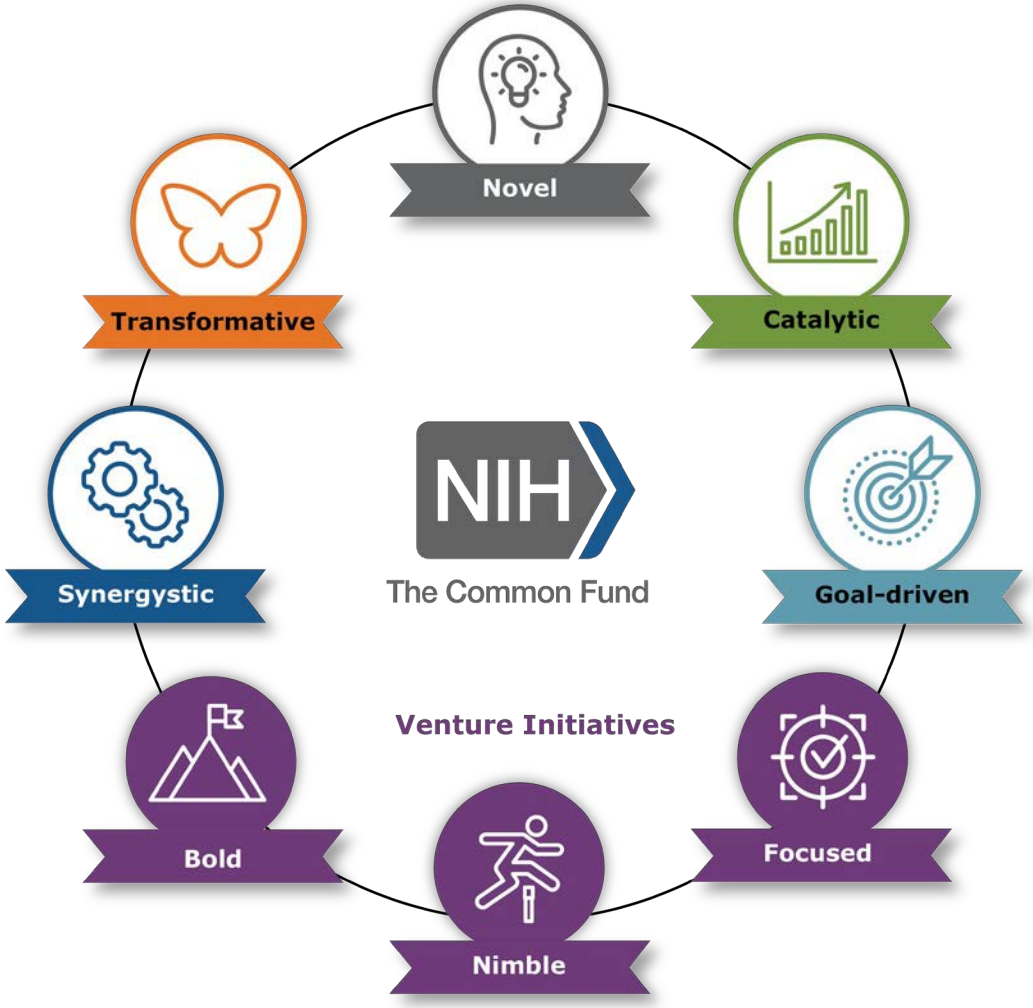
National Institutes of Health
Office of Strategic Coordination–The Common Fund

The Venture Program

The Venture Program enables Common Fund support of **bold, short-term** initiatives.

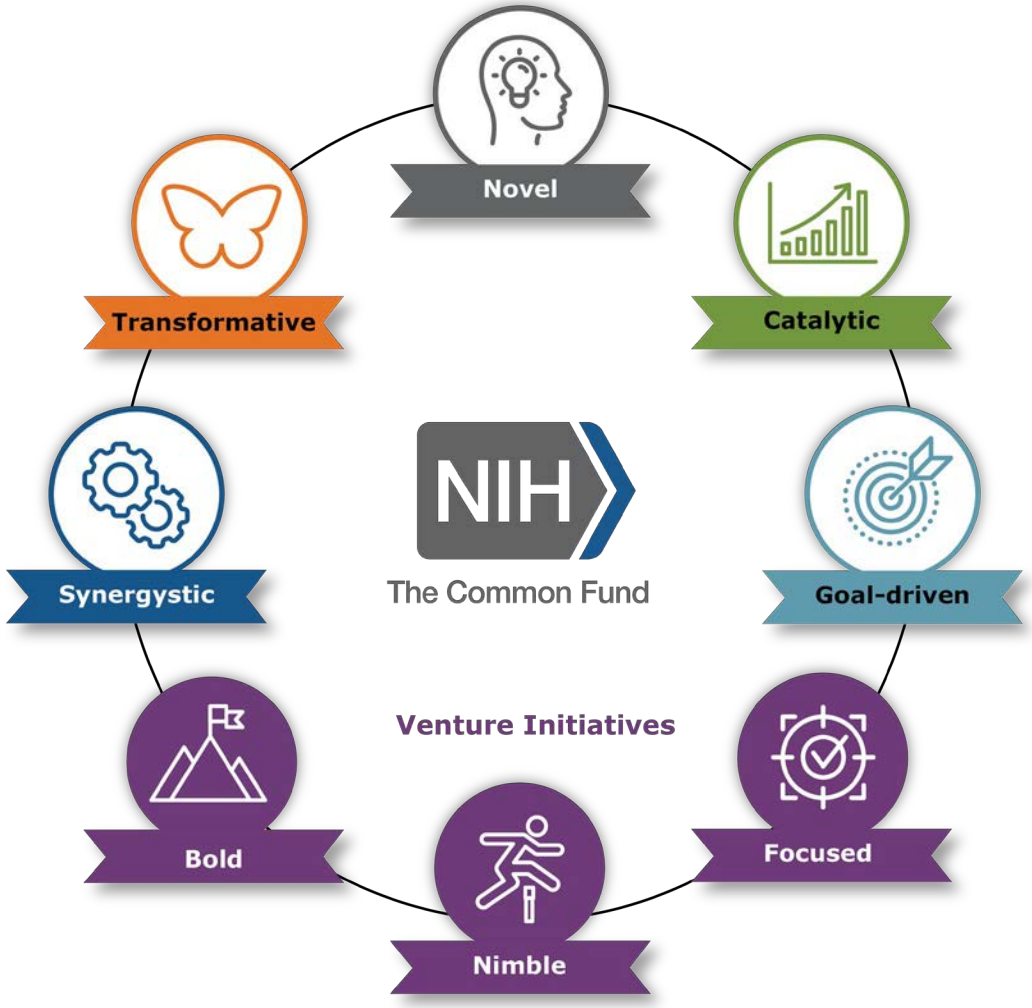
- Brief funding, no more than 3 years
- Clearly defined goals with go/no-go milestones
- Flexible approach to funding mechanisms and project timelines
- Nimble, responsive – fast implementation
- Smaller scale, higher-risk initiatives



The Venture Program Impact

Completion of these initiatives could lead to:

- Earlier disease detection and monitoring by non-invasive technologies
- Centralized data access to study multiple diseases through one portal
- Improved health outcomes for newborns
- Technologies to increase precision therapies to treat diseases



Update: ***Current*** *Venture initiative*

Development and Application of Imaging Technologies for Oculomics

FY24-FY26

Goal:

- Support the development and application of novel, noninvasive ocular imaging technologies, machine learning algorithms, and other tools
- Enable identification of highly sensitive and specific biomarkers for systemic diseases

Status: Three (3) awards issued

1. Cellular-level Vascular Oculomics (CVO) for monitoring systemic vascular health
2. Novel retinal higher-order capillary hemodynamics imaging for detecting cerebral small vessel disease
3. Achieving specificity in imaging neurodegeneration with visible light Optical Coherence Tomography

Update: ***Current*** *Venture initiative*

Systems Biology Data Platform (SysBio) Initiative

FY24-FY26

Goal:

- Create a centralized portal and workbench with tools to allow researchers to search for and use data across multiple Accelerating Medicines Partnership® (AMP®) programs.

Status:

- One (1) award issued to Verily Life Sciences
- Entering beta phase with a focus on:
  - Set up platform as a Centralized system for data management
  - Created a Common Data Model for data harmonization

Update: ***Current*** *Venture initiative*

Newborn Screening by Whole Genome Sequencing(NBSxWGS) Collaboratory Initiative

FY25-FY27

Goal:

- Provide NBS using WGS and genetic counseling to 5-10 partnered state public health laboratories
- Focuses on monogenic diseases that are actionable in the first year of life
    - Seeking significant community input on ethical, legal, and social implications (ELSI) and data retention and sharing issues

Status:

- Issued Research Opportunity Announcement in March 2025
- Applications received in May 2025
- Anticipate one (1) award by the end of September 2025

Update: ***Current*** *Venture initiative*

Advancing Non-Invasive Optical Imaging Approaches for Biological Systems (NIOI)

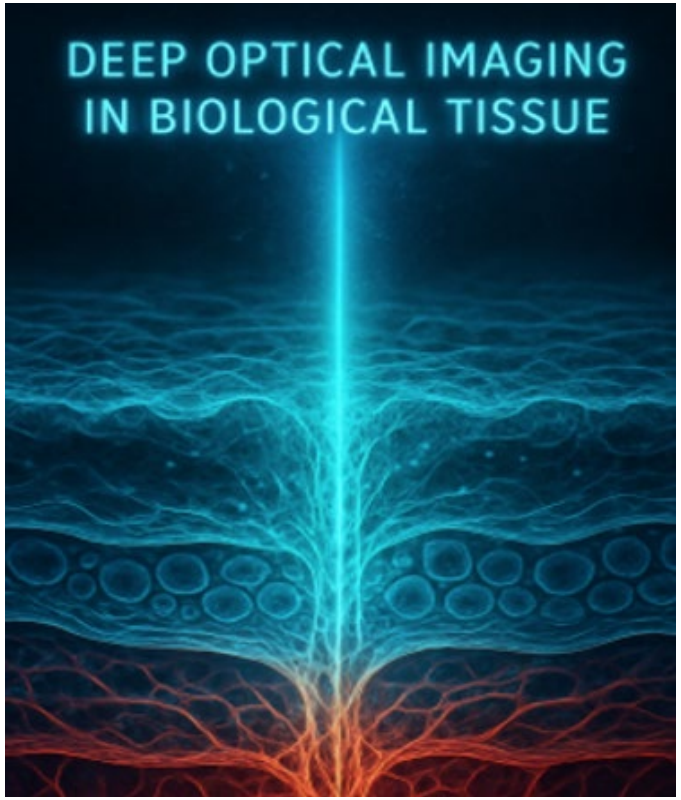
FY25-FY27

Goal:

- Develop next-generation non-invasive or minimally-invasive optical imaging techniques capable of significantly deeper imaging of biological systems with high spatial and temporal resolution

Status:

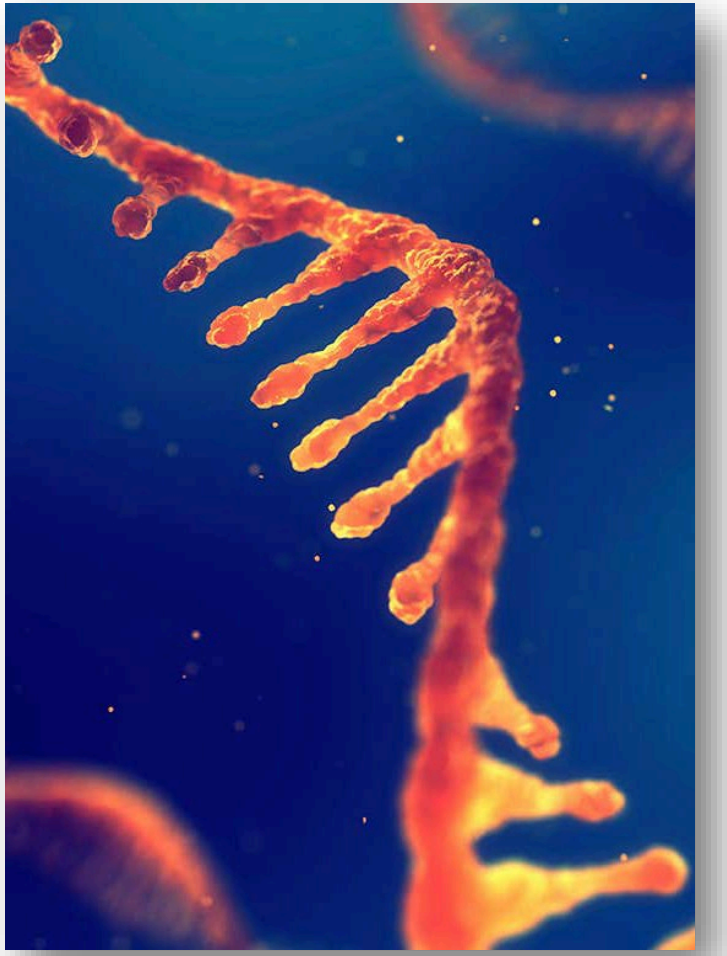
- Issued Notice of Funding Opportunity in December 2024
- Applications received by March 2025
- Anticipate ~ four (4) awards will be issued by end of September 2025



***New** Venture initiative*

Novel RNA Targeting Therapy Technologies

FY26-FY28



Objective: Develop, validate and disseminate a publicly available suite of novel RNA-targeting technologies for new therapeutic classes that can lead to the development of precision RNA-targeted therapies to treat diseases.

***New** Venture initiative*

Novel RNA Targeting Therapy Technologies

FY26-FY28 ***Not for the Development of RNA as a Therapeutic***

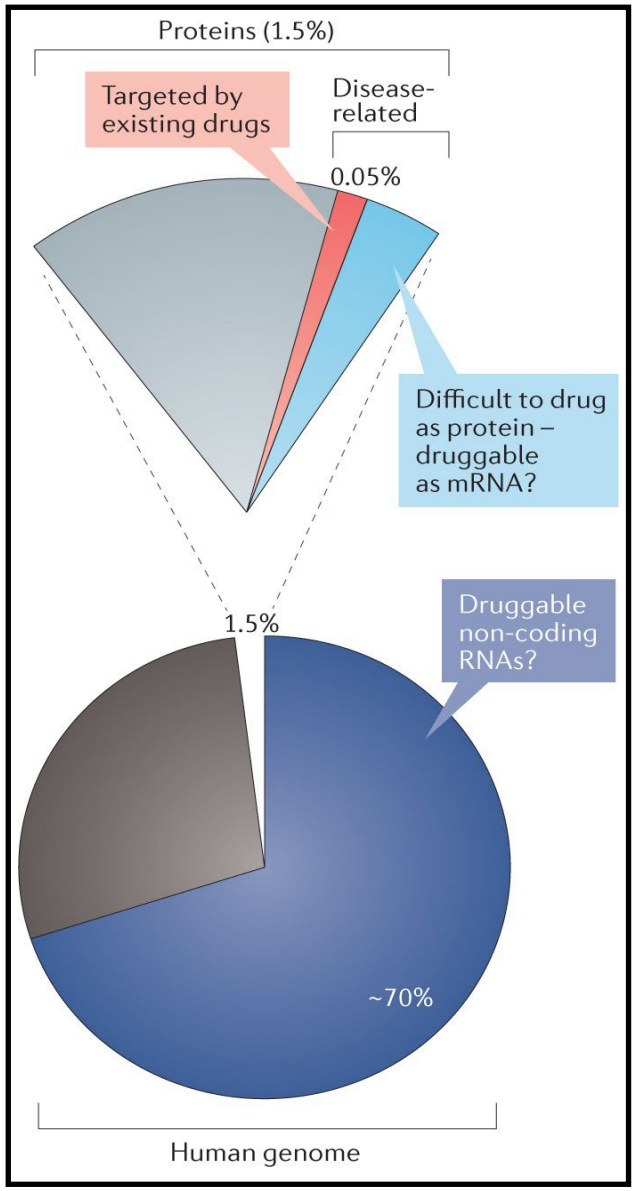
Significant past work has utilized RNA as a therapeutic:

- RNA Antisense Oligonucleotides
- mRNA Drugs/Vaccines
- RNAi-based Drugs
- RNA Aptamers
- Small Interfering RNA Drugs

The above methods all utilize RNA as the therapy. This is NOT the purpose of the following proposed initiative.

This Venture Fund initiative will encourage the development of technologies that will enable the discovery of RNA-TARGETING THERAPIES.

RNA as a Target for Treatment



Warner et al., 2018 Nature Reviews Drug Discovery

RNA

point mutations, insertions/deletions, microsatellite repeat expansions, etc.

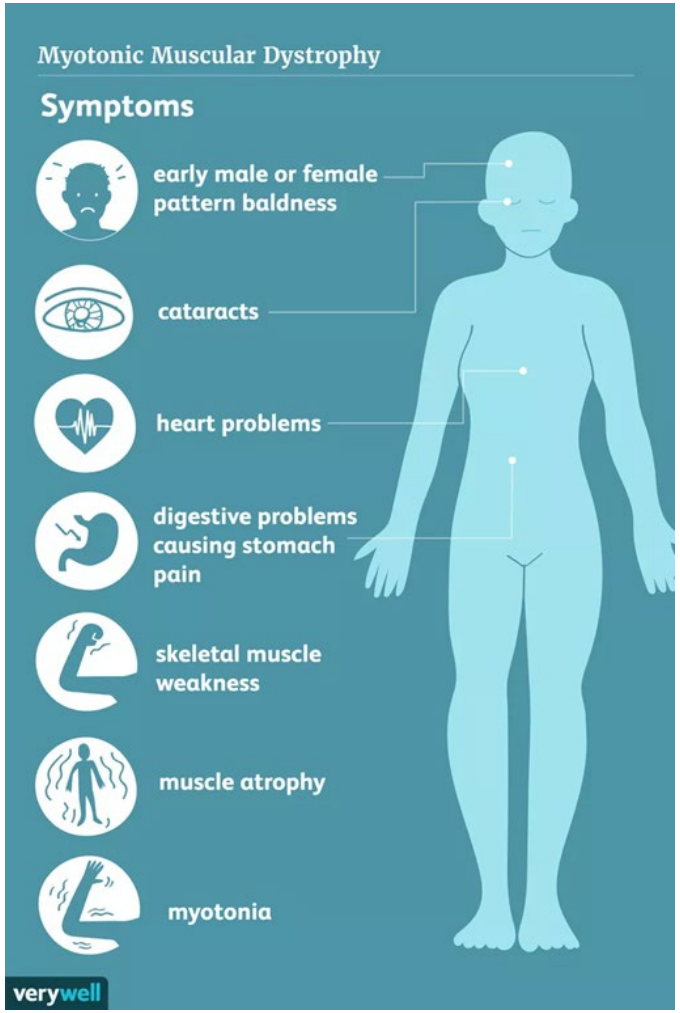
→

disease

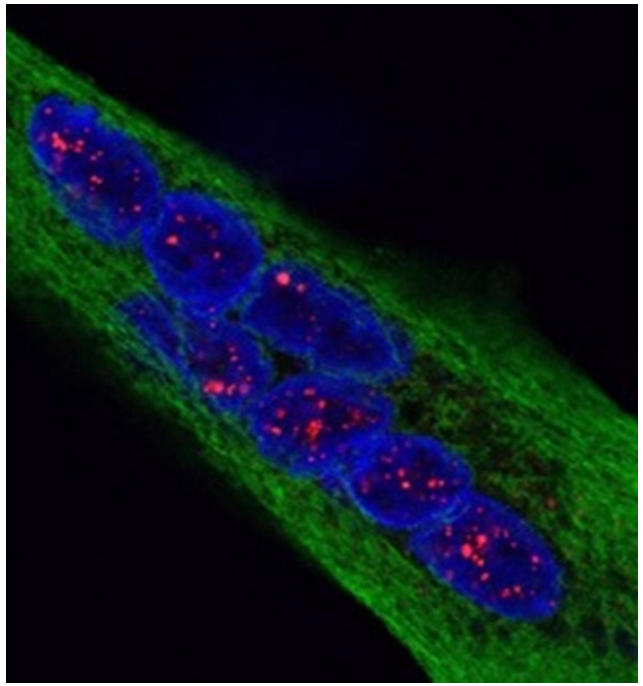
e.g. muscular dystrophies, parkinsonism, Hutchinson-Gilford progeria syndrome, repeat expansion disorders

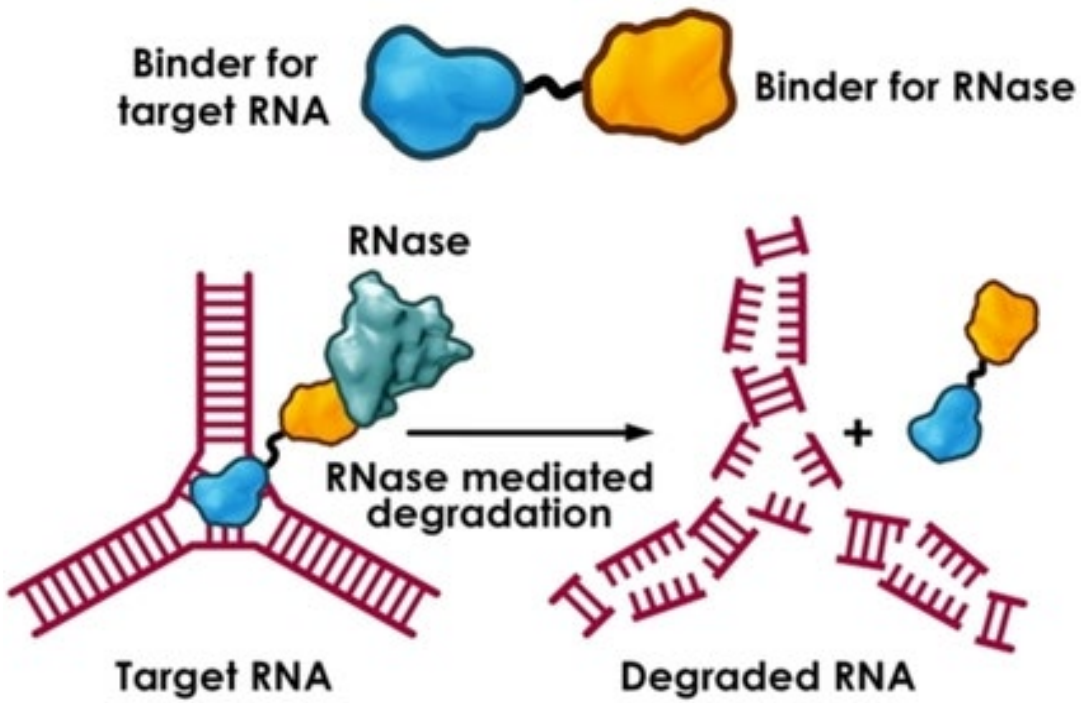
Adapted from Neueder, Andreas, 2019 Journal of Molecular Biology

Myotonic Dystrophy – an RNA Repeat Disease



Moawad, (2024) Verywell Health





Nalawansha 2024, ChemBioChem

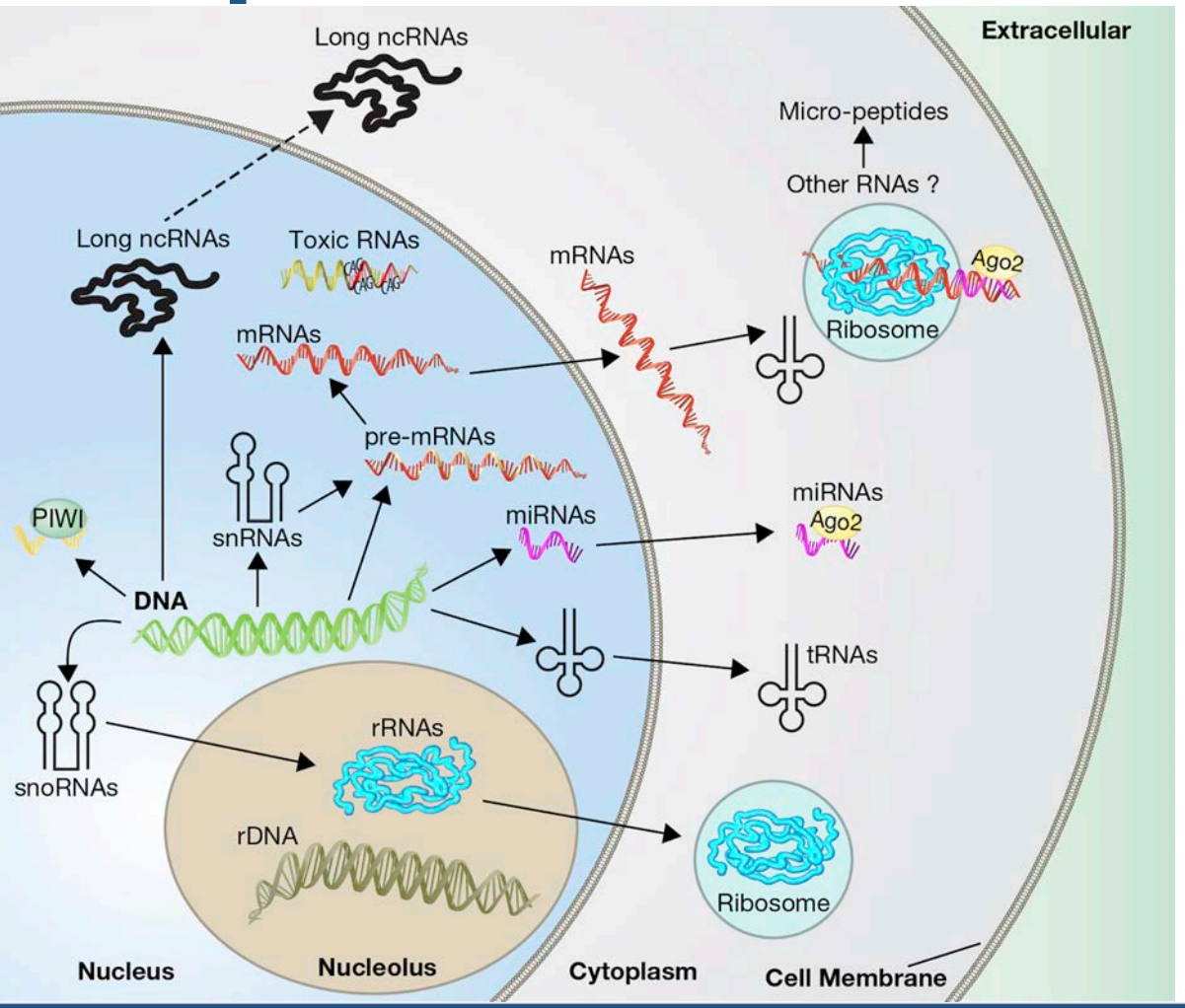
Novel RNA Targeting Therapy Technologies

FY26-FY28

Goal: Encourage and advance the discovery of novel RNA-targeting therapies for currently difficult-to-drug diseases by:

- Development and iteration of prototype RNA-targeting technologies
- Validation and testing of prototype RNA-targeting technologies
- Facilitating access to validated technologies by the scientific community

Major Work Products and Anticipated Impact



Impact

New therapeutics and novel treatments to address a sizable target class (RNA) that has been implicated in many difficult-to-drug diseases, such as Huntington's Disease, heart failure, muscular dystrophies and cancer.

- Technologies that enable development of novel, specific RNA-targeting therapies
- Protocols, tools and reagents available through public repositories, marketing, publication, etc.

Crooke et al, Cell Metabolism 2018

Initiative Leadership

Joni Rutter, Director, NCATS, Co-Chair
Nora Volkow, Director, NIDA, Co-Chair

Working Group Members:

Karlie Sharma (NCATS), Coordinator

Dobrila Rudnicki (NCATS)

Stefan Maas (NCI)

Sai Majji (NICHD)

Subramaniam Ananthan (NIDA)

Rebecca Roof (NINDS)

Jose Ruiz (NCATS), NCATS Challenge Manager

The OSC Team:

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Assistant Director for Re-engineering the Research Enterprise (RRE)

Kristina Faulk (OD), Advisor

Selyman Rastani (OD), Operations Manager

Vanessa Barnes (OD), SPEC Team Contact

Questions or comments?

commonfund.nih.gov

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