



The Accelerated Development of mRNA-Based Therapeutics

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January 25th, 2022

Contents



An overview of 2022 - the RNA landscape



Focus on mRNA-based drug and trial landscape



Looking beyond COVID-19 into the future

Beacon RNA

Beacon RNA includes assets comprising of oligonucleotide-based or RNA-based genetic material, and small molecules that directly bind to RNA.

Beacon RNA covers:

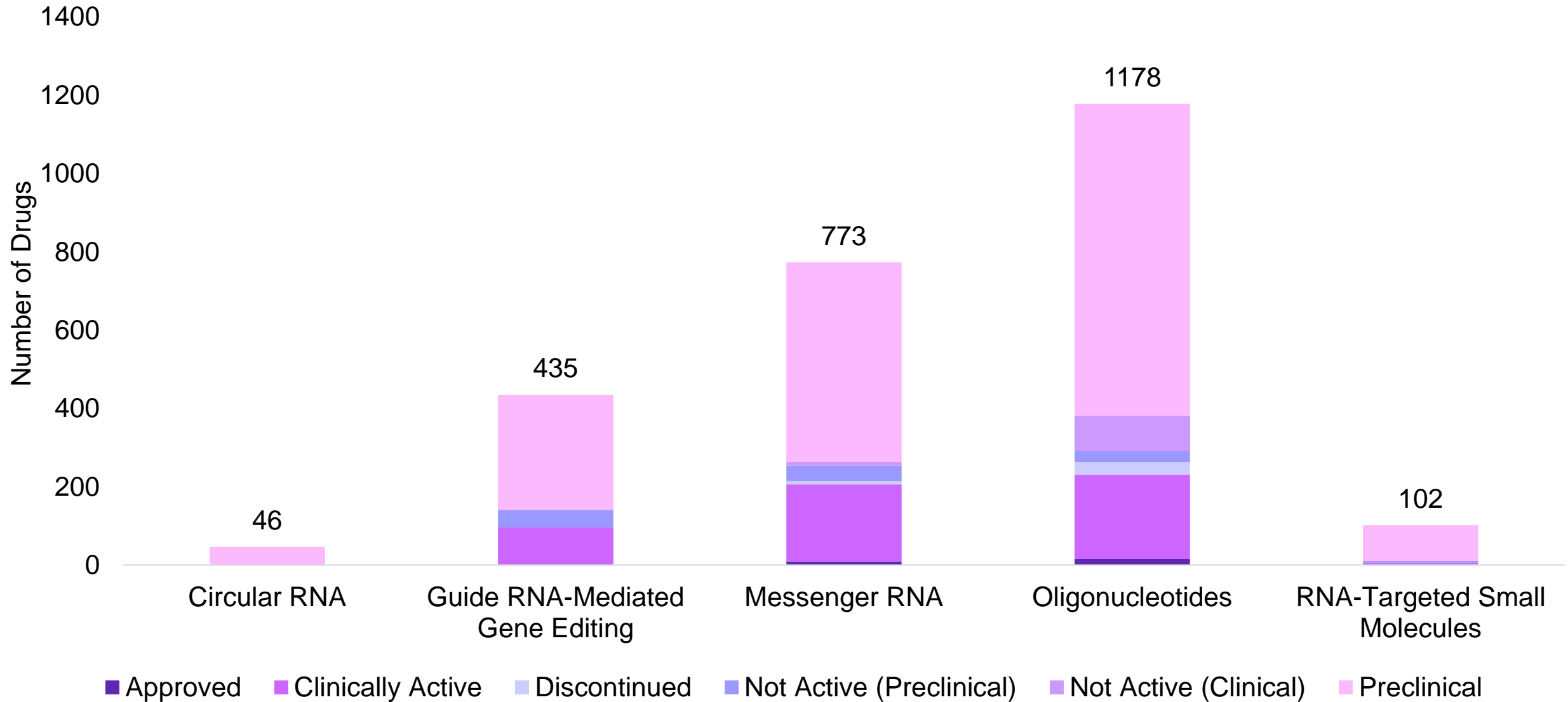
- All disease indications
- Any combination trial that includes any of the RNA modalities within the scope with other therapies
- All research/data since 2011

Technology	
Therapeutic Class	mRNA-Based Vaccine, Intramuscular Formulation
Drug Characteristics	
Mechanism of Action	Immune Stimulation
Editing Technology	Not Applicable
Delivery System	Lipid nanoparticle (LNP) (Non-Viral)
Genetic Material for Therapy	mRNA
Site of Editing/Delivery	Invivo
Gene/Mutation	Not Applicable
Nucleic Acid Modification	N1-Methylpseudouridine

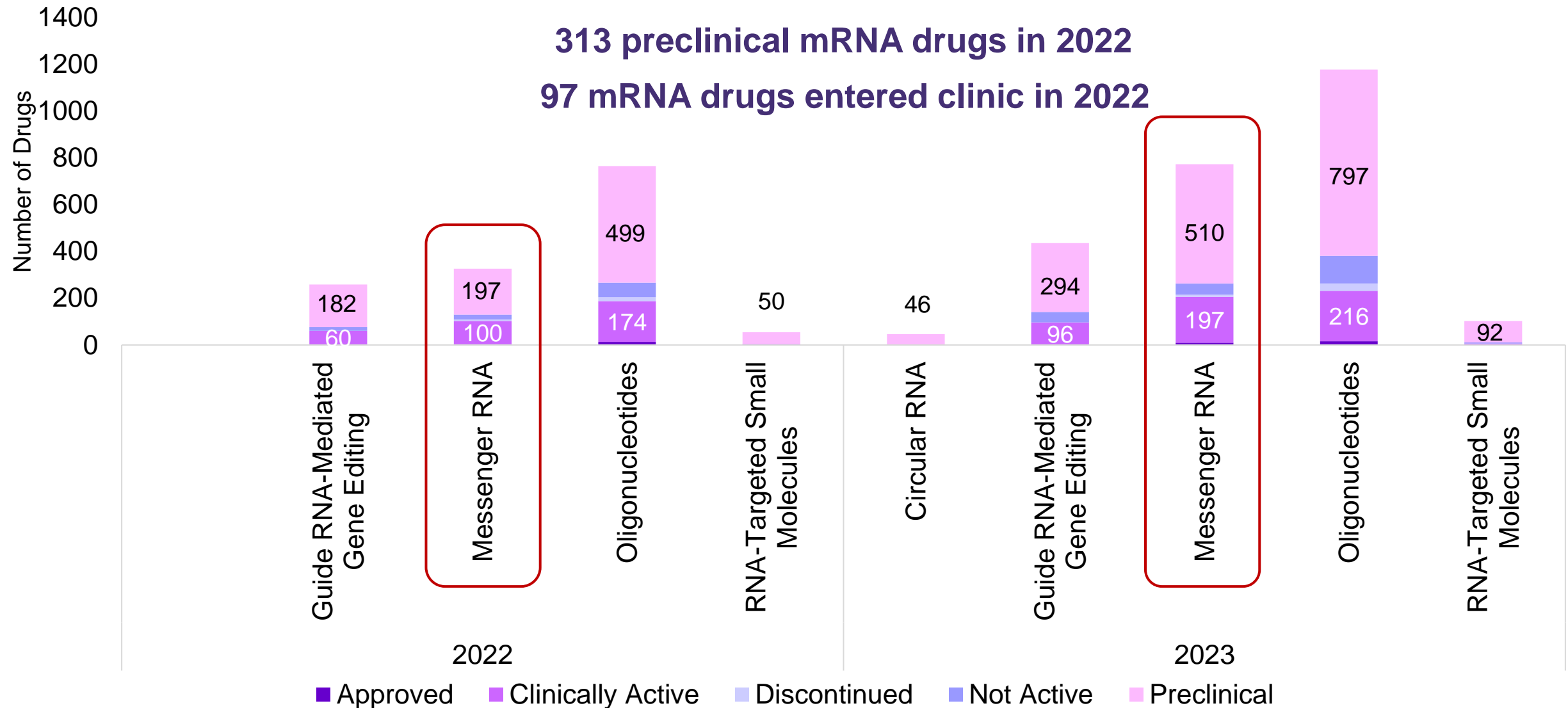
Review of 2022 - the RNA Landscape

*Data as of 04/01/2023

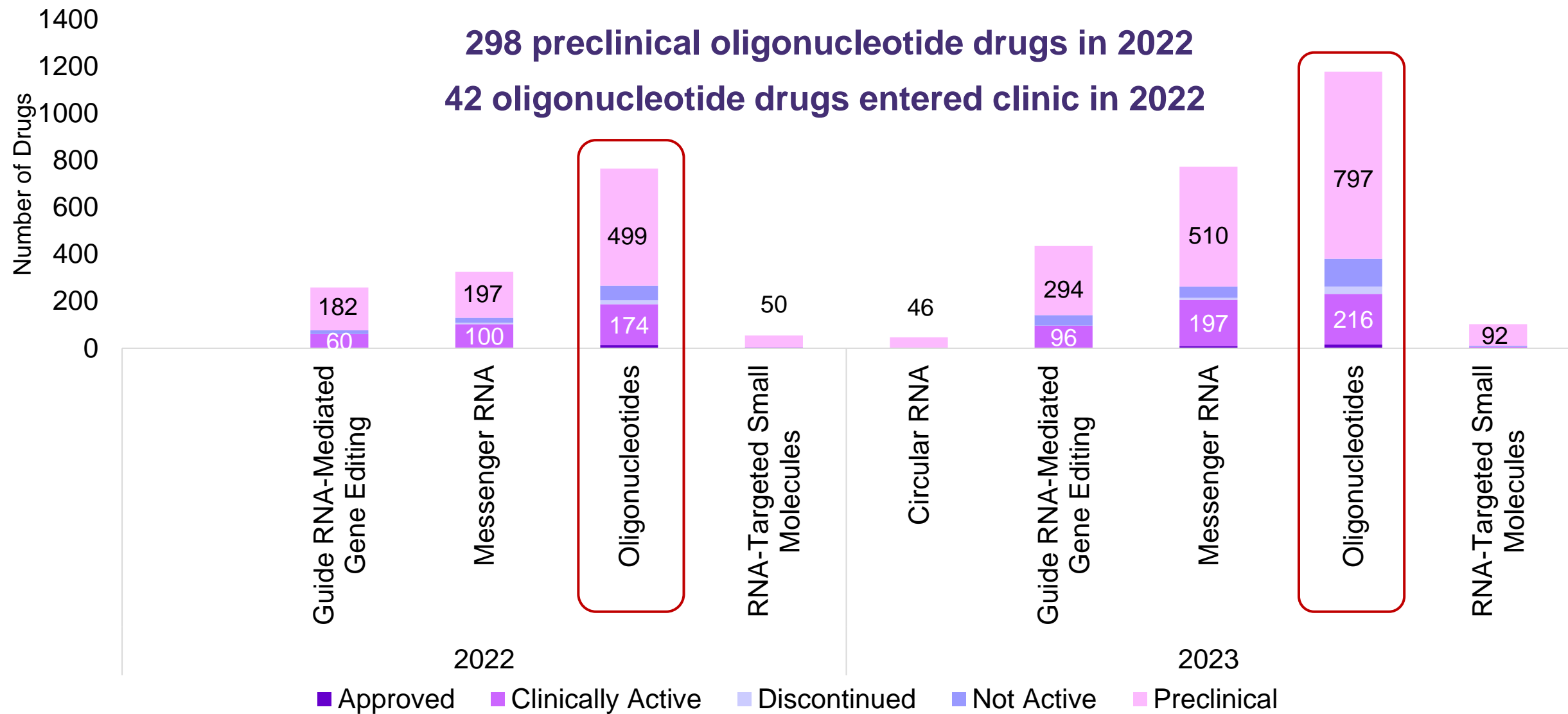
RNA Landscape by Therapeutic Class - 2023



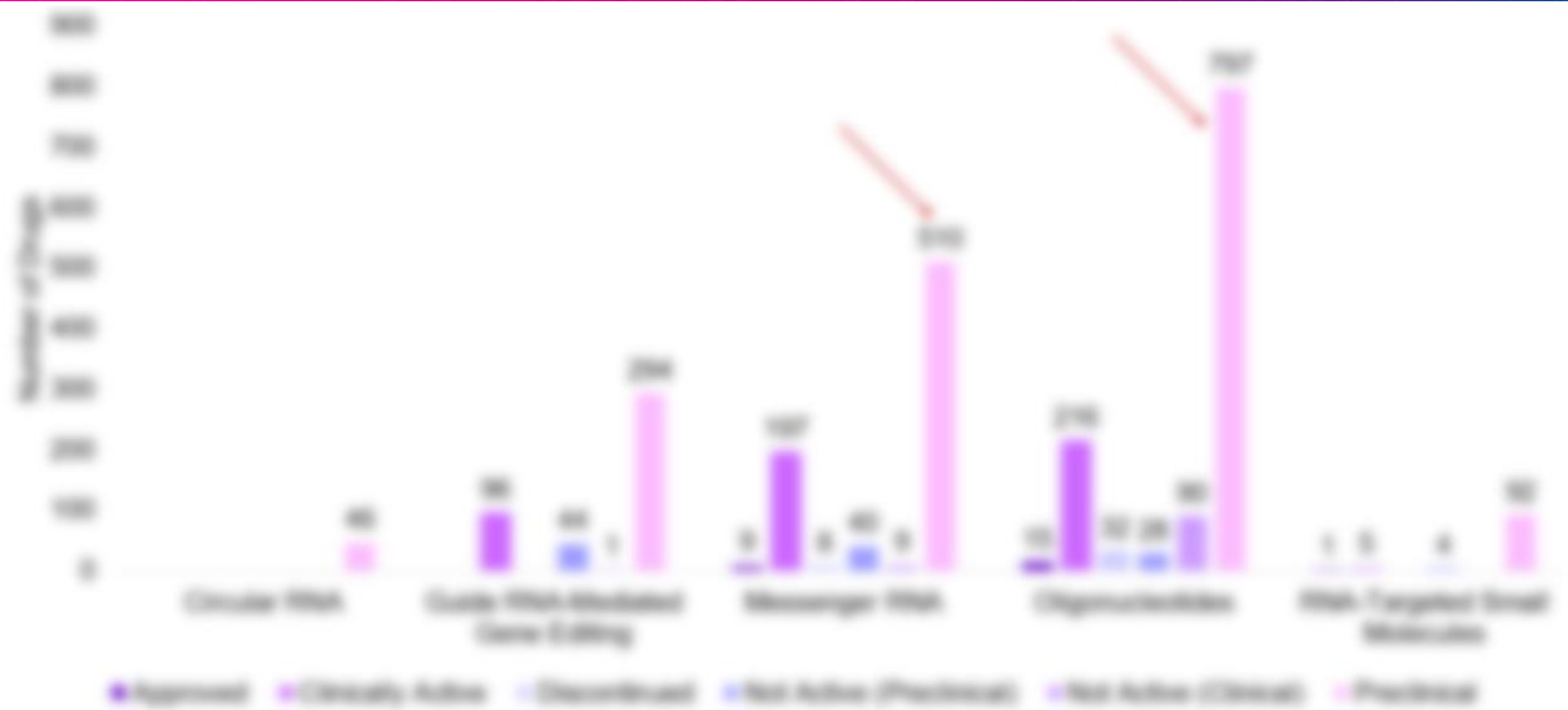
RNA Landscape by Therapeutic Class – 2022 vs 2023



RNA Landscape by Therapeutic Class – 2022 vs 2023



Status of RNA Therapies



Status of RNA Therapies



Trials Initiated in the RNA Space Over the Past 10 Years



mRNA Trials Initiated Over the Past 10 Years

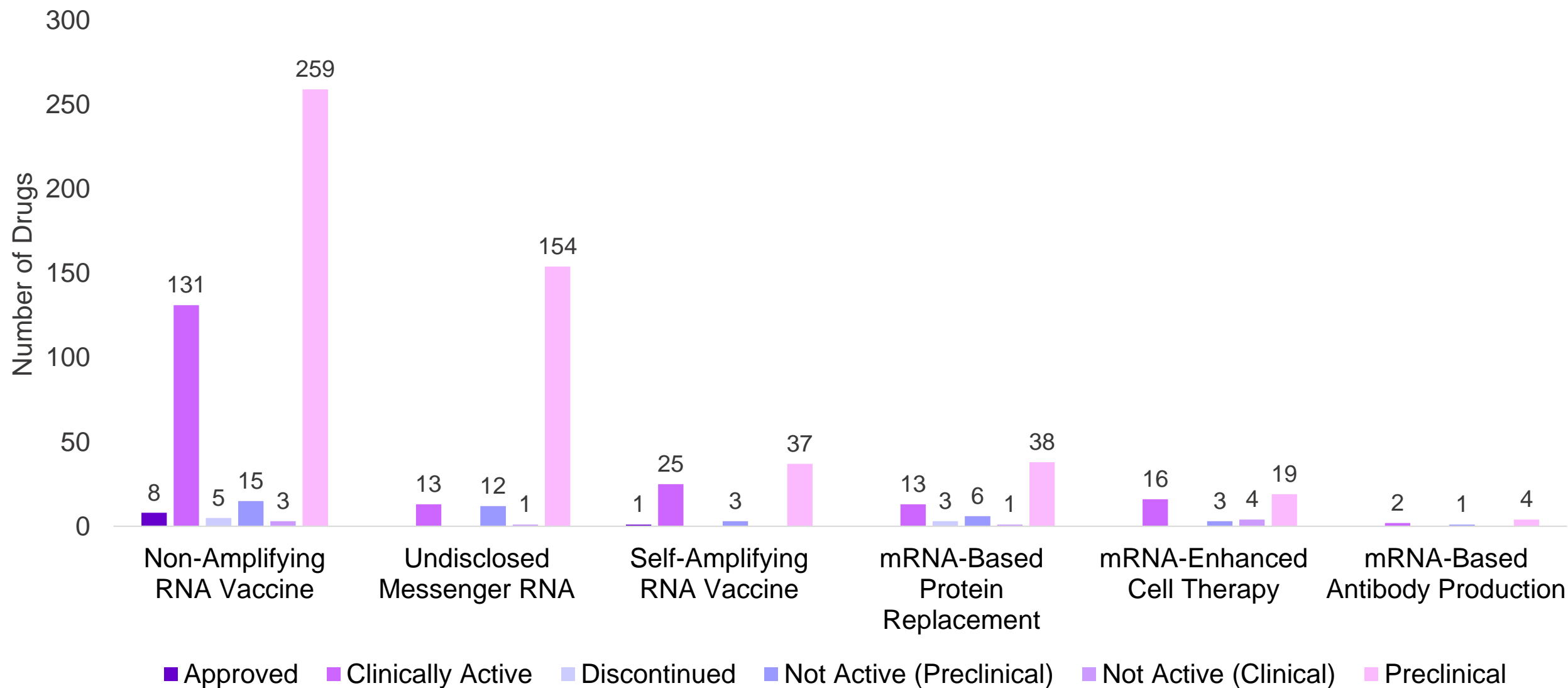


Drugs Entering the Clinic in 2022 vs Discontinued

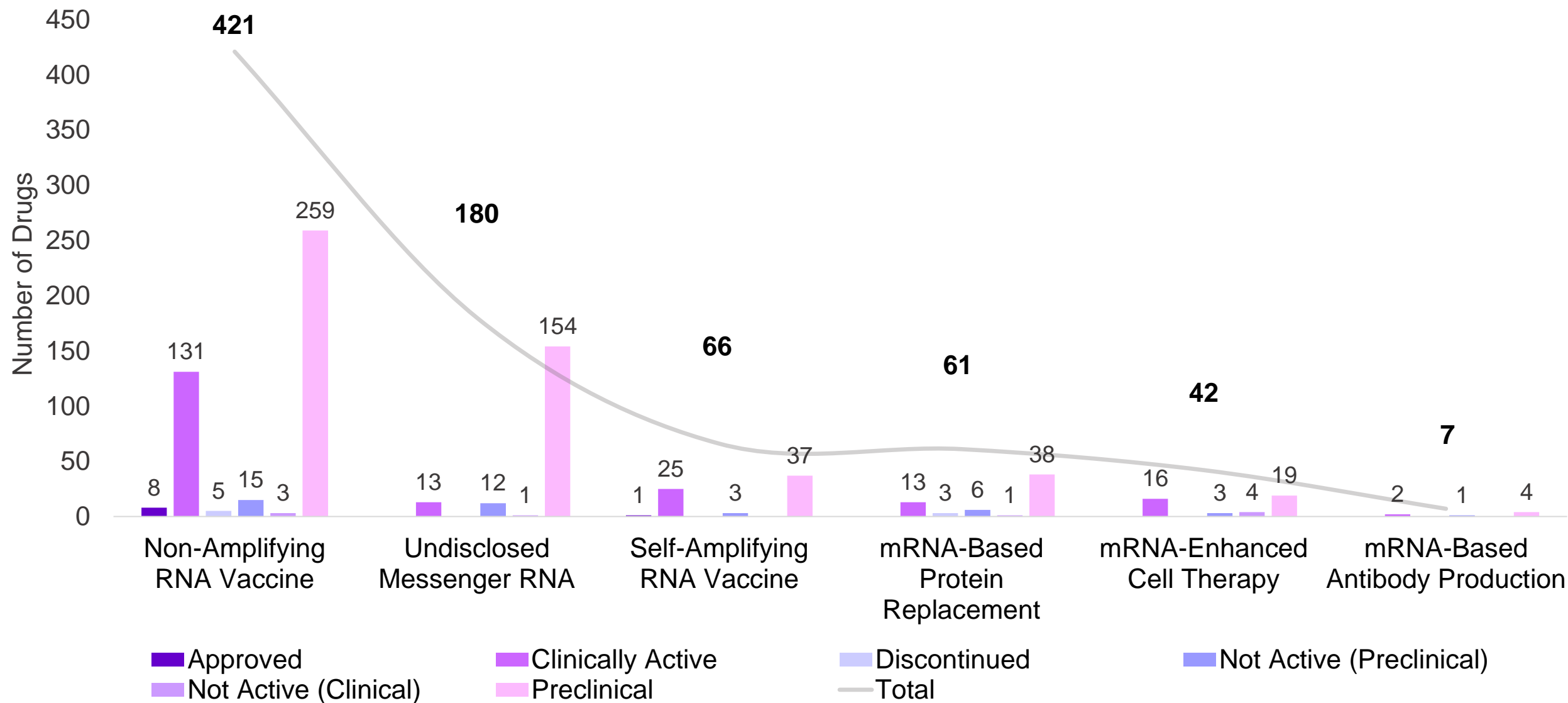


Messenger RNA Landscape Analysis

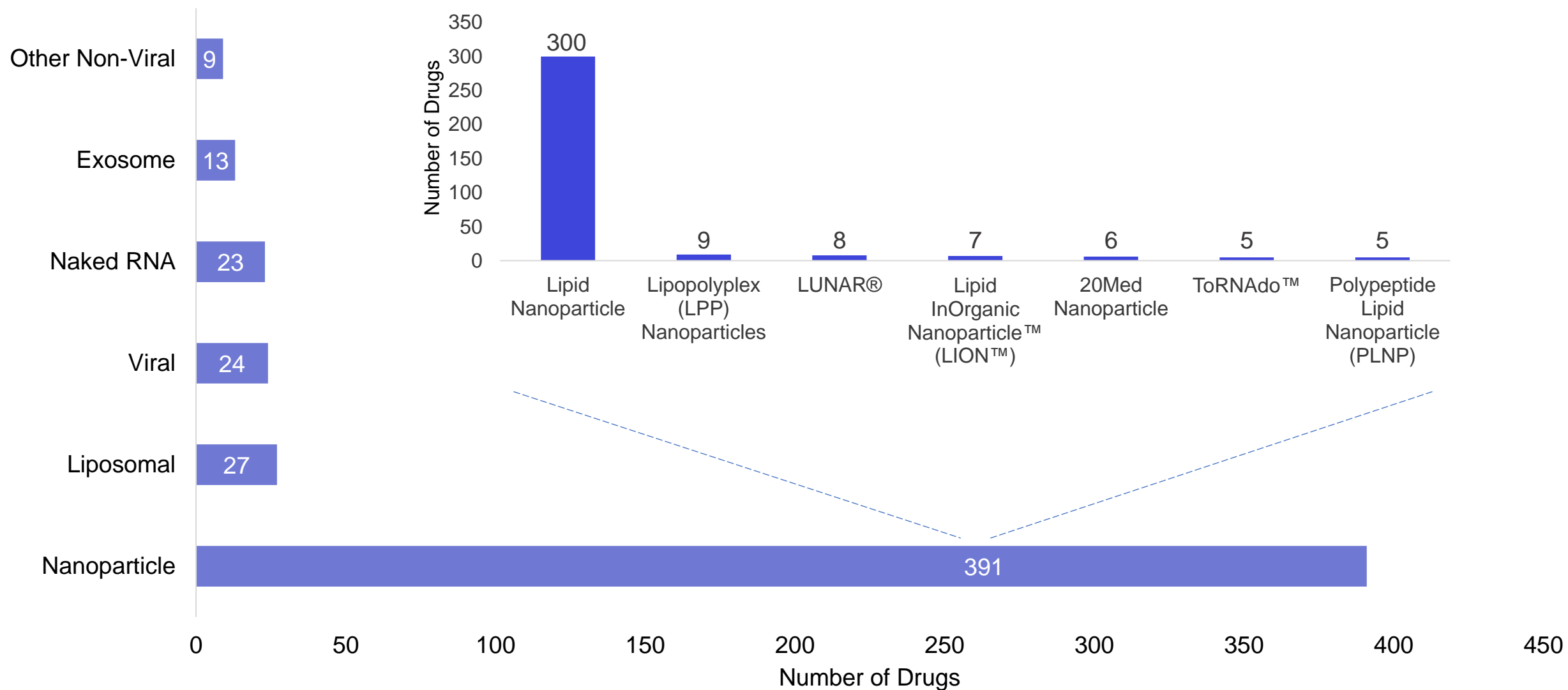
Drug Status of mRNA Drugs



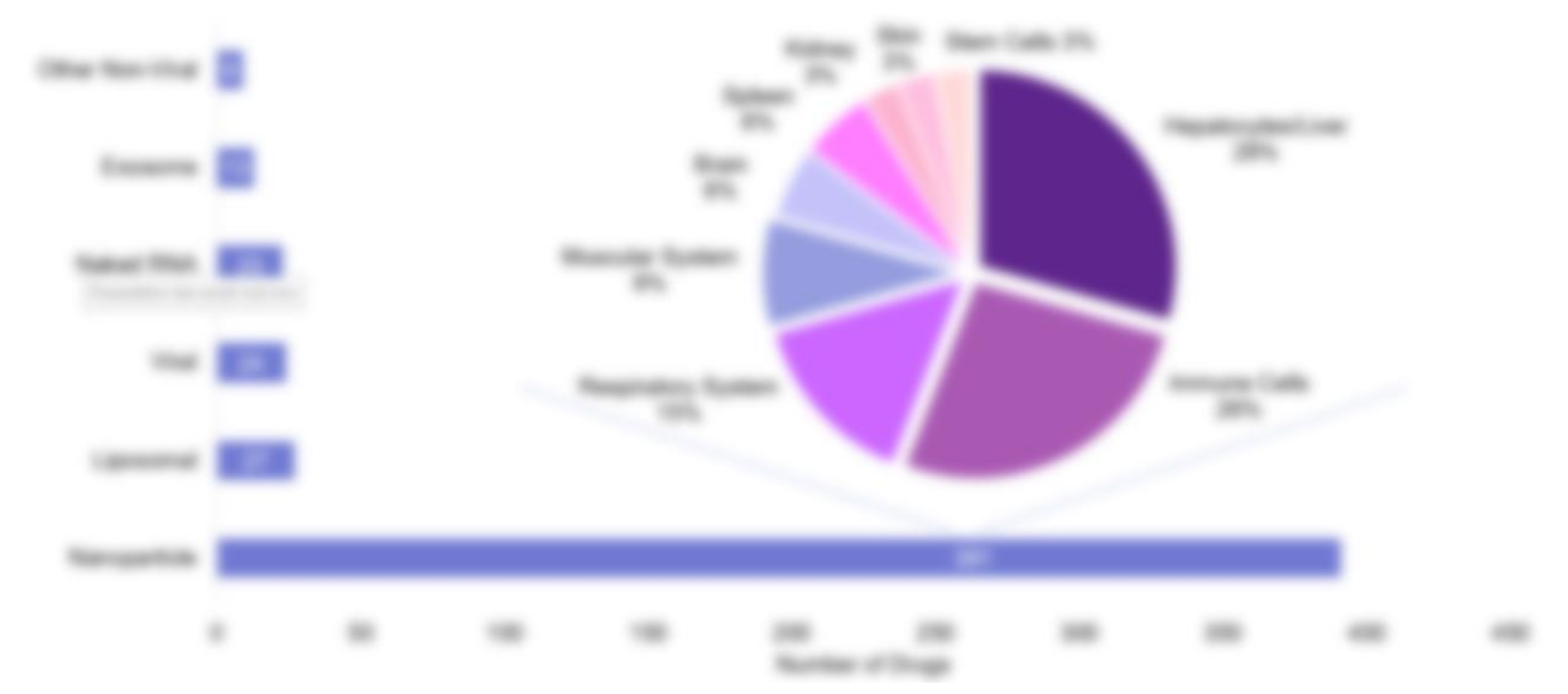
Drug Status of mRNA Drugs



Top Delivery Systems for mRNA



Target Organ Trends in Nanoparticle Delivery



Diseases Targeted by mRNA Therapies

*drugs have been counted per developer

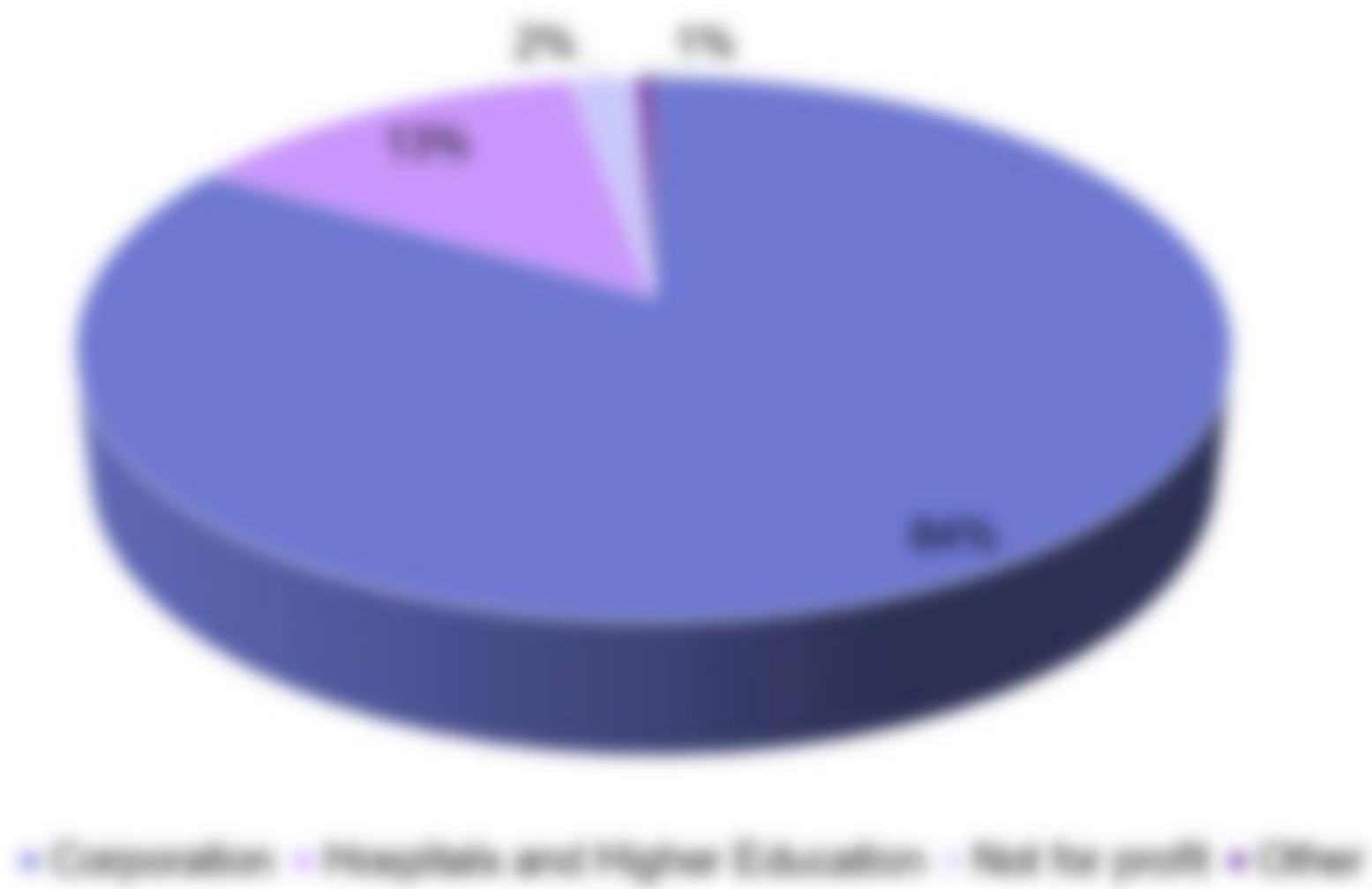


Developer Types and Locations



Developer Types and Locations

*drugs have been counted per developer



Country	Count
USA	10
Canada	1
UK	1
France	1
Germany	1
Italy	1
Spain	1
Sweden	1
Switzerland	1
Other	1

Phase Distribution of mRNA Trials



Phase Distribution of mRNA Trials Initiated in 2022

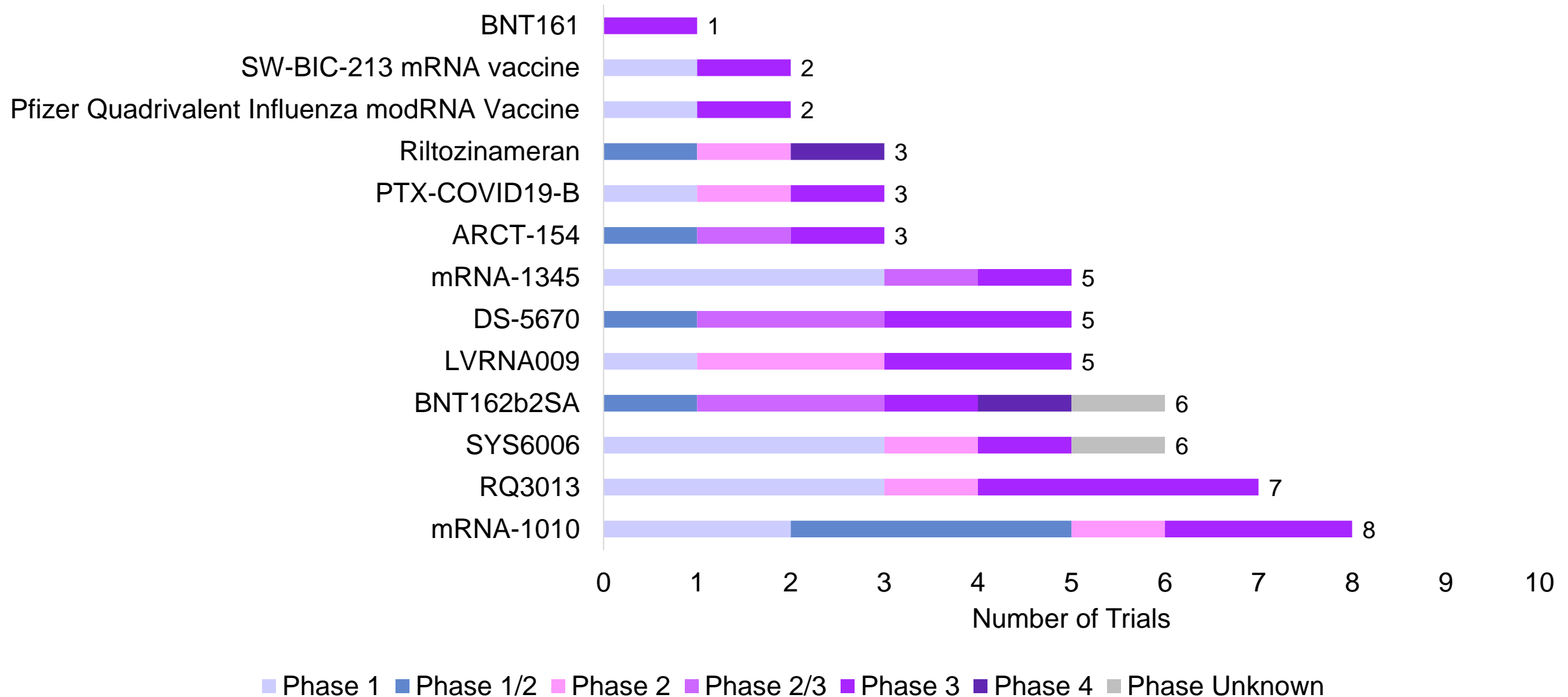


mRNA Approvals of 2022

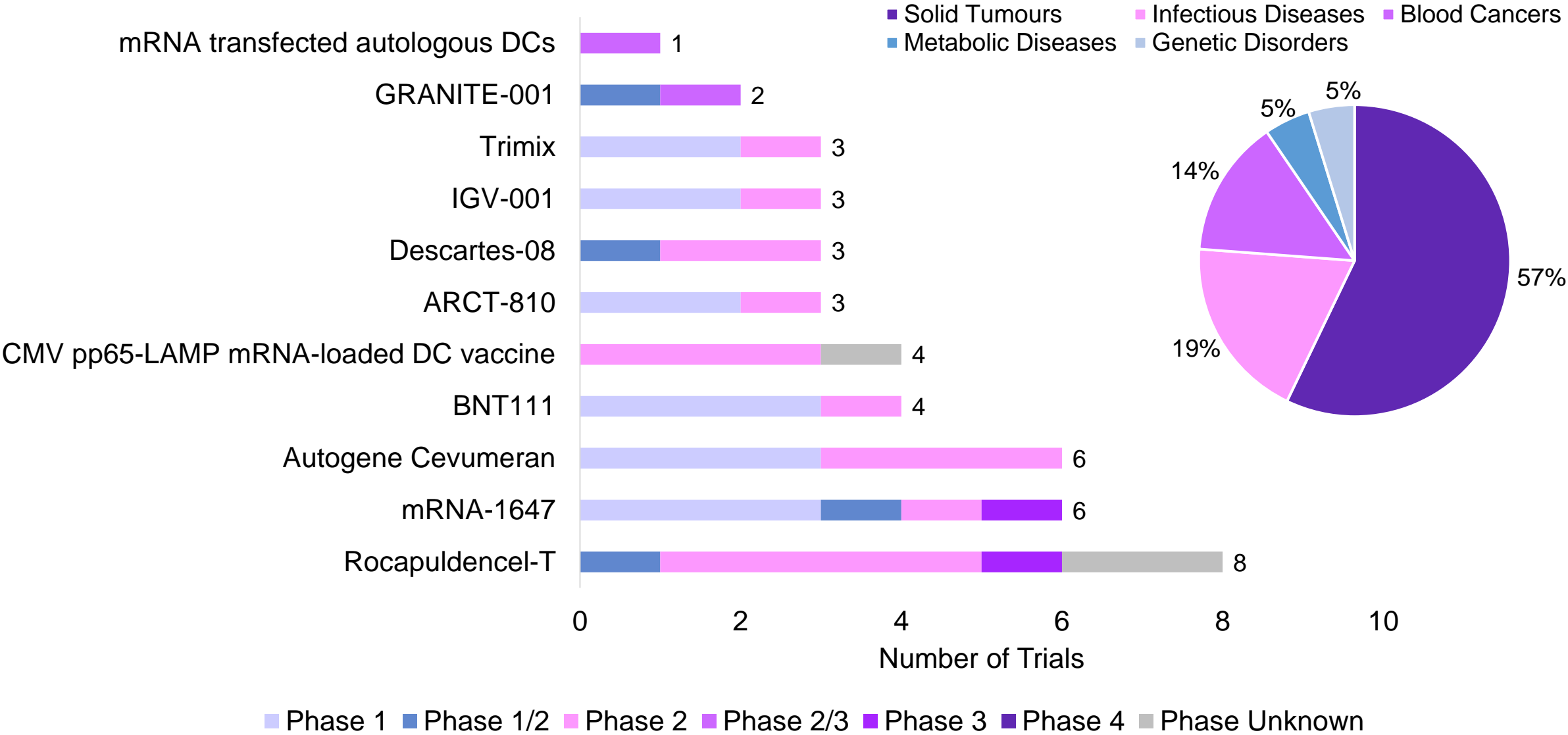


The Future of mRNA Therapies

Highest Phase of Development in the COVID-19 Space

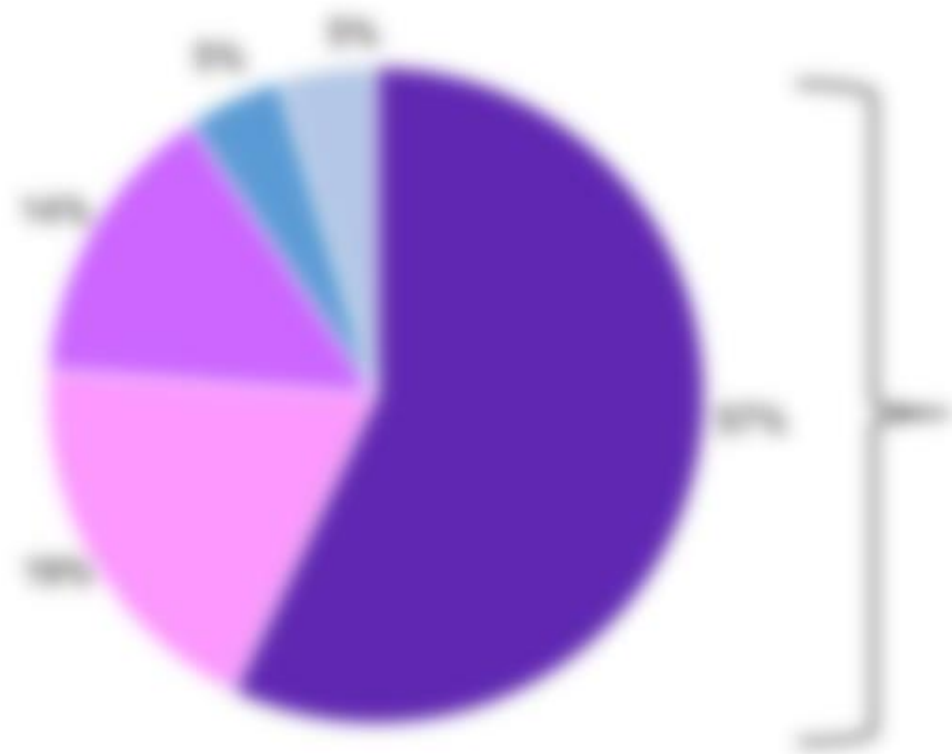


Highest Phase of Development Beyond COVID-19

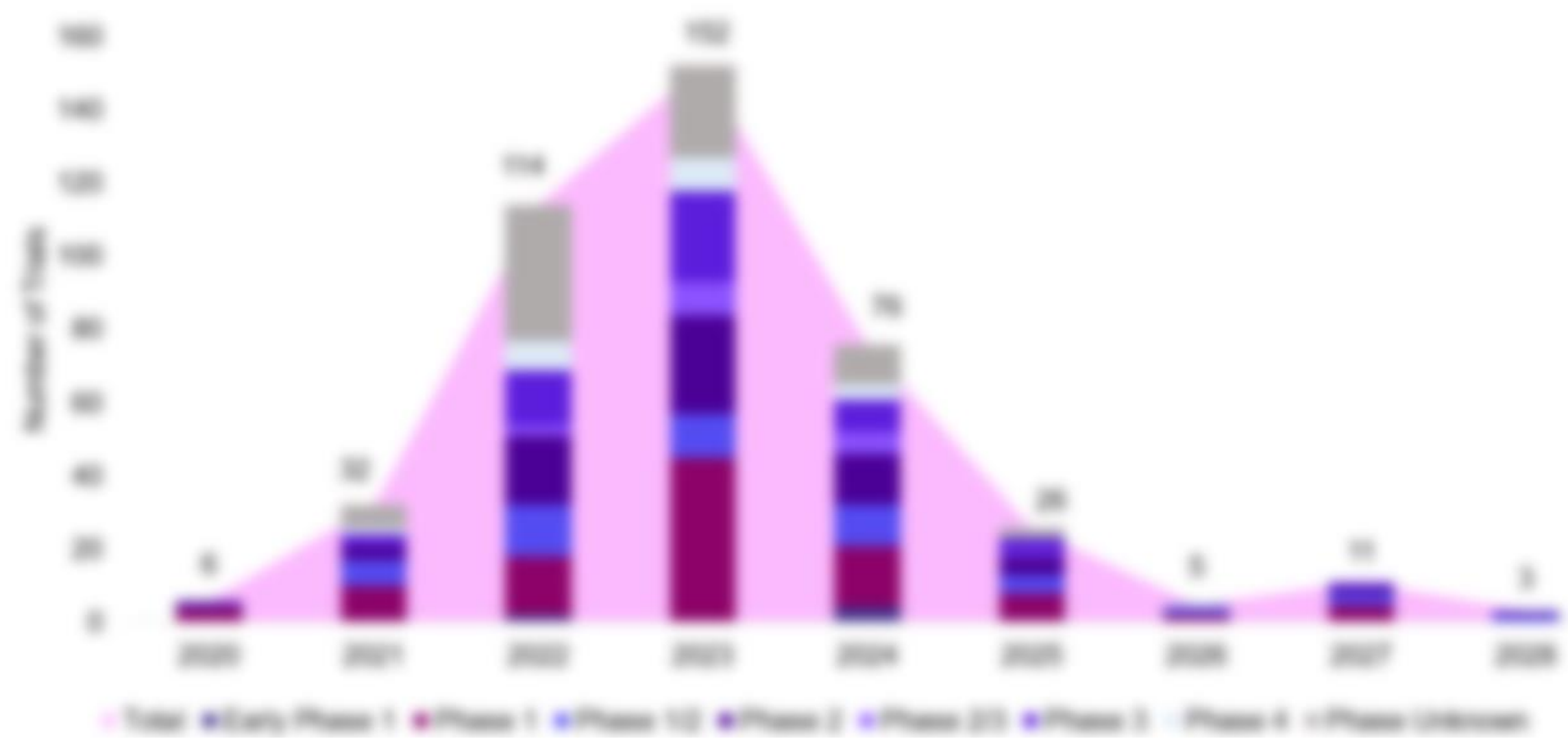


Disease Landscape – What's Next?

Global Diseases
Regional Diseases
Emerging Diseases
Re-emerging Diseases



Clinical mRNA Trials Ending by 2030



Summary



The RNA space is growing with an 86% average annual growth in initiation of RNA trials since 2013



The messenger RNA field is progressing like no other RNA-based therapy, with exciting milestones expected within the infectious diseases and cancer indications space



Messenger RNA therapies show great promise for 2023, with many late-stage trials set to end this year

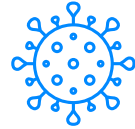
Beacon RNA

Beacon RNA Data Sourcing

WE MONITOR THOUSANDS OF SOURCES EVERY DAY AND UPDATE WITHIN 24-48 HOURS OF PUBLICATION & PIECE THEM TOGETHER IN ONE PLACE



CONFERENCE POSTERS
& ABSTRACTS



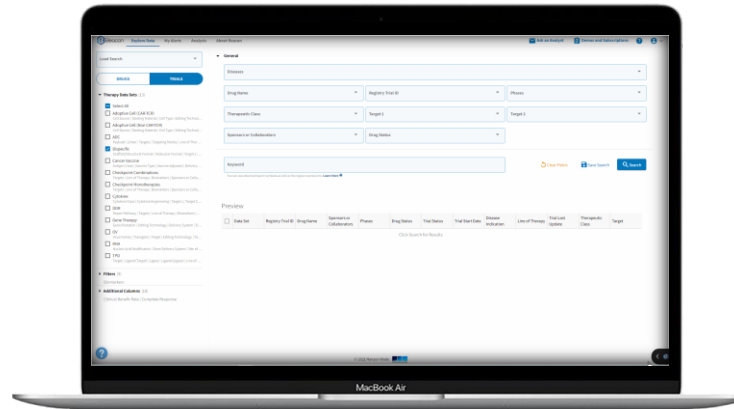
SCIENTIFIC JOURNALS
AND PUBLICATIONS



GOVERNMENT AND
REGULATORY ORGS



CLINICAL TRIAL
REGISTRIES



NEWSPAPERS AND
OTHER MEDIA SITES



PATENTS



PRESS
RELEASES



COMPANY REPORTS &
PRESENTATIONS

ALL DATA IN BEACON IS CLOSELY CITED WITH REFERENCE LINKS TO PUBLIC SOURCES

WE ALSO PRODUCE REGULAR LANDSCAPE & CONFERENCE REPORTS, INCLUDING BESPOKE ANALYSIS

Beacon RNA Platform

Load Search

DRUGS

TRIALS

► **Therapy Data Sets** (12)

RNA

► **Filters** (13)

Delivery System | Formulation | Genetic Material for Therapy | Nucleic Acid Modification | Site of Delivery | Target Organ/Cell

► **Additional Columns** (13)

Mechanism of Action | Preclinical Efficacy | Preclinical In Vitro | Preclinical PK | Preclinical References | Preclinical Toxicity/Safety

▼ **General Filters**

Disease Indication

Therapeutic Class

Drug > Targeted Therapy > RNA Therapy

Drug > Targeted Therapy > RNA Therapy > Circular RNA (circRNA)

Drug > Targeted Therapy > RNA Therapy > Guide RNA (gRNA)-Mediated Gene Editing

Drug > Targeted Therapy > RNA Therapy > Messenger RNA (mRNA)

Drug > Targeted Therapy > RNA Therapy > Messenger RNA (mRNA) > mRNA-Based Antibody Production

Drug > Targeted Therapy > RNA Therapy > Messenger RNA (mRNA) > mRNA-Based Protein Replacement

Drug > Targeted Therapy > RNA Therapy > Messenger RNA (mRNA) > mRNA-Based Vaccine

Drug > Targeted Therapy > RNA Therapy > Messenger RNA (mRNA) > mRNA-Based Vaccine > Non-Amplifying RNA Vaccine

Nucleic Acid Modification

Site of Delivery

Target Organ/Cell



Beacon RNA Platform



Dose Escalation Study of mRNA-2752 for Intratumoral Injection to Participants in Advanced Malignancies

Add to Watchlist

Print

Summary Study Inclusion/Exclusion Arms Drug Dosing Patients Toxicity Results References Commentary Update History

Identifier

NCT03739931

Other Trial IDs

mRNA-2752-P101 | 2022-001597-55

Sponsors

Moderna Therapeutics

Collaborators

AstraZeneca

Biomarkers

BRAF, PD-L1 (B7H1, PDCD1L1, PDCD1LG1, PDL1, CD274), OX40L, IL-6, IL-23, IL-36, IL-22

Trial Status

Clinical Trial Status Recruiting participants

Trial Start Date 27 Nov 2018

Trial End Date 31 Mar 2025

Pivotal Trial No

PHASE 1

mRNA-2752

Drug Name mRNA-2752, Acavameran

Therapeutic Class Immune Checkpoint Modulator, mRNA-Based Protein Replacement

Route of Administration Intratumor

Target OX-40L

Target IL-23

Target IL-36γ

Editing Technology Not Applicable

Site of Delivery Undisclosed

Durvalumab

Drug Name Imfinzi, MEDI4736, MEDI-4736, Durvalumab

Therapeutic Class Antineoplastic, PD-1/PD-L1 targeting checkpoint modulators

Route of Administration Undisclosed

Target PD-L1

Targeting Moiety Undisclosed

Disease Indication

Lymphoma 2nd Line or greater

Triple Negative Breast Cancer 2nd Line or greater

Non-Hodgkin Lymphoma 2nd Line or greater

Head and neck squamous cell carcinoma (HNSCC) 2nd Line or greater

Advanced Solid Tumors 2nd Line or greater

Metastatic Solid Tumors 2nd Line or greater





RNA
Beacon
Targeted Therapies

**MAKING DRUG DEVELOPMENT
DECISIONS, FASTER.**

THANK YOU



Ioana Panait
Lead Research Analyst - RNA

ioana.panait@hansonwade.com