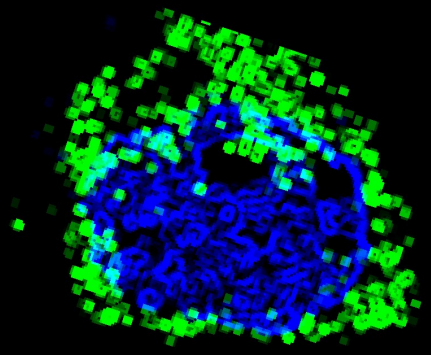
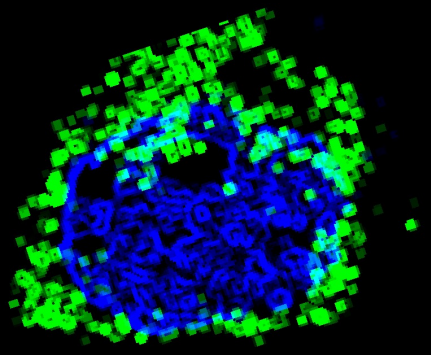




Insights of RNA biology

BIOPIECES XI

Kasun Buddika

Bioinformatics Scientist

2022-01-30

My story: From Thalawakelle to San Francisco

School: Sumana Central College, Thalawakelle
Biological Science

College: University of Colombo, Sri Lanka
B. Sc. in Biochemistry and Molecular Biology

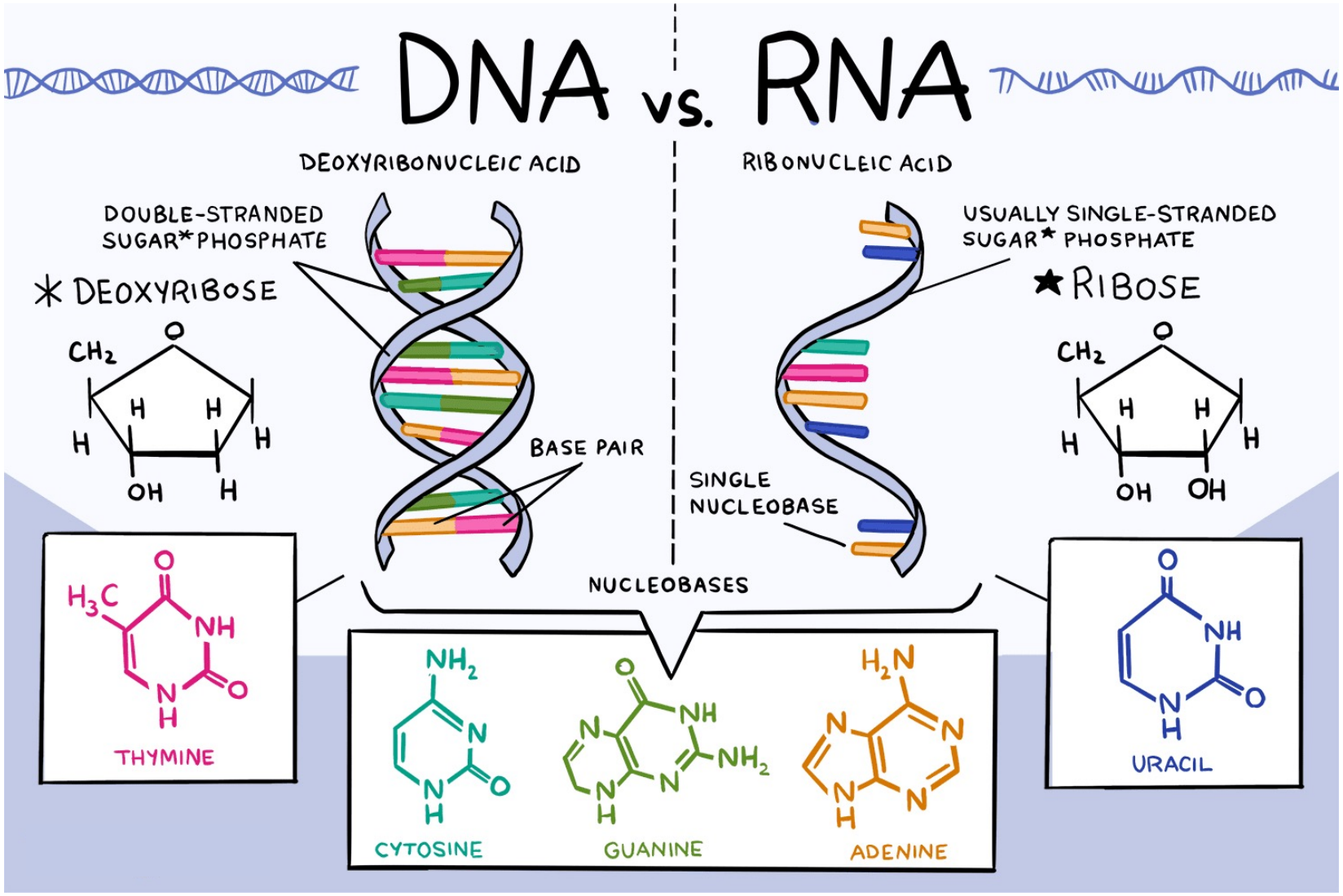
Graduate School: Indiana University, Bloomington, USA
Ph.D. in Genome, Cell and Developmental Biology

Current Employment: Advanced Cell Diagnostics, San Francisco
Bioinformatics Scientist

Next stop: Alamar Biosciences, San Francisco
Bioinformatics Engineer

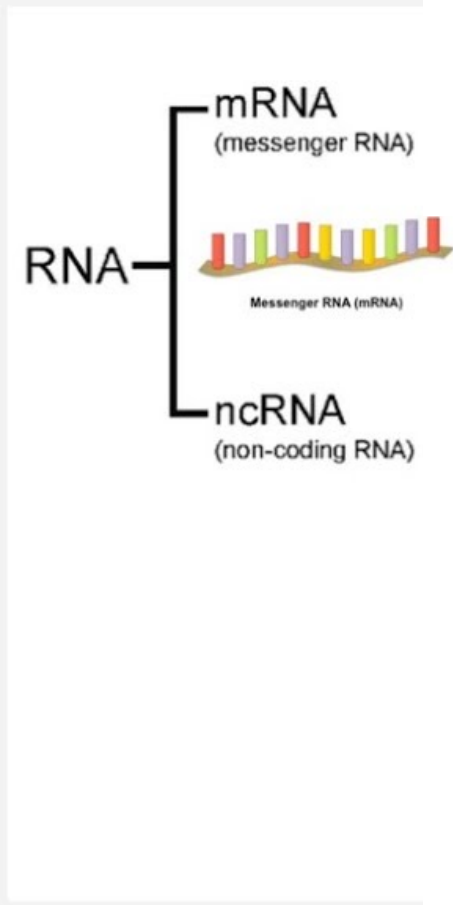
Learn more: **https://kasunbuddika.com/**

Nucleic acids: DNA vs RNA

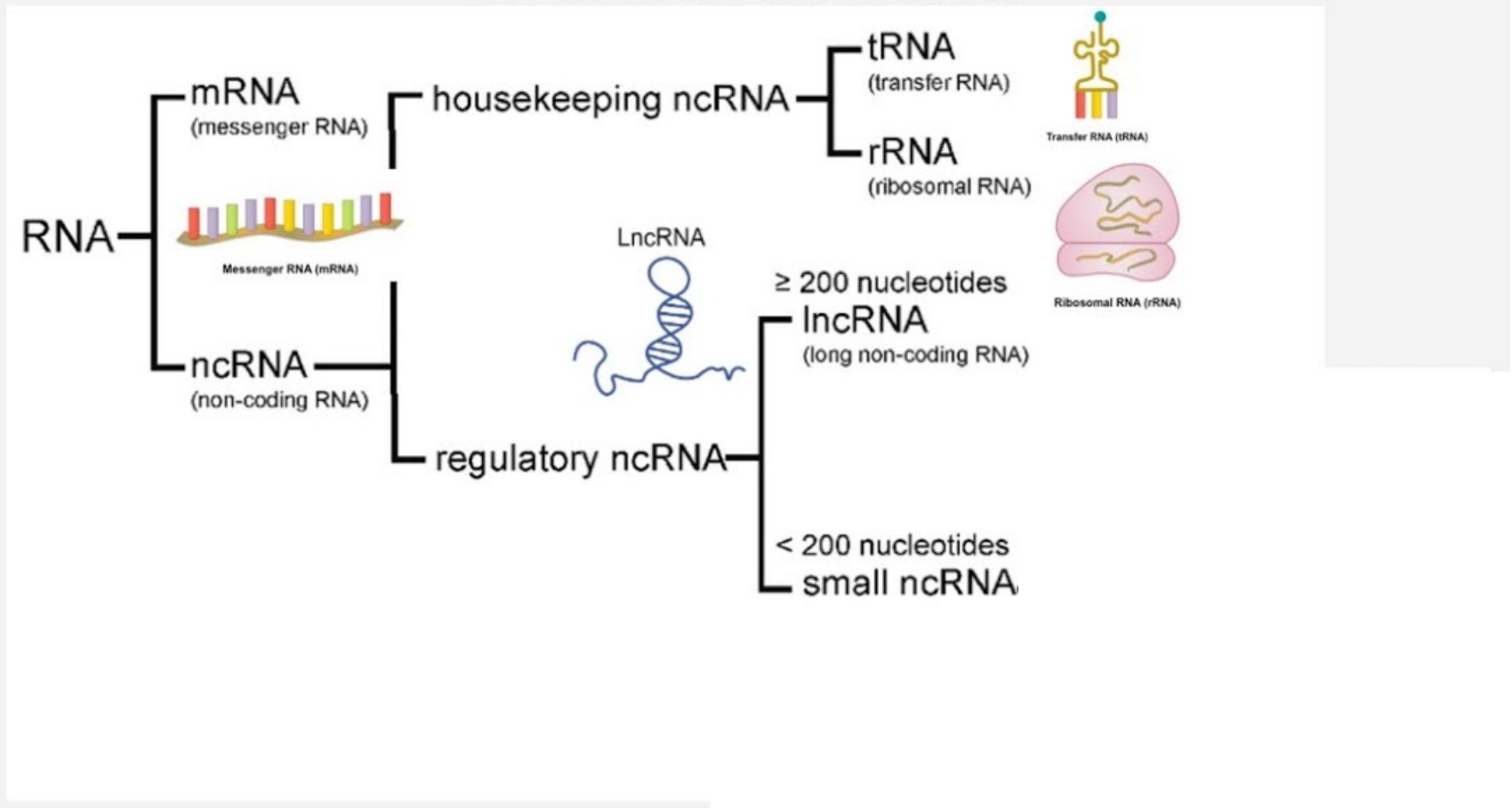


Source: www.thoughtco.com

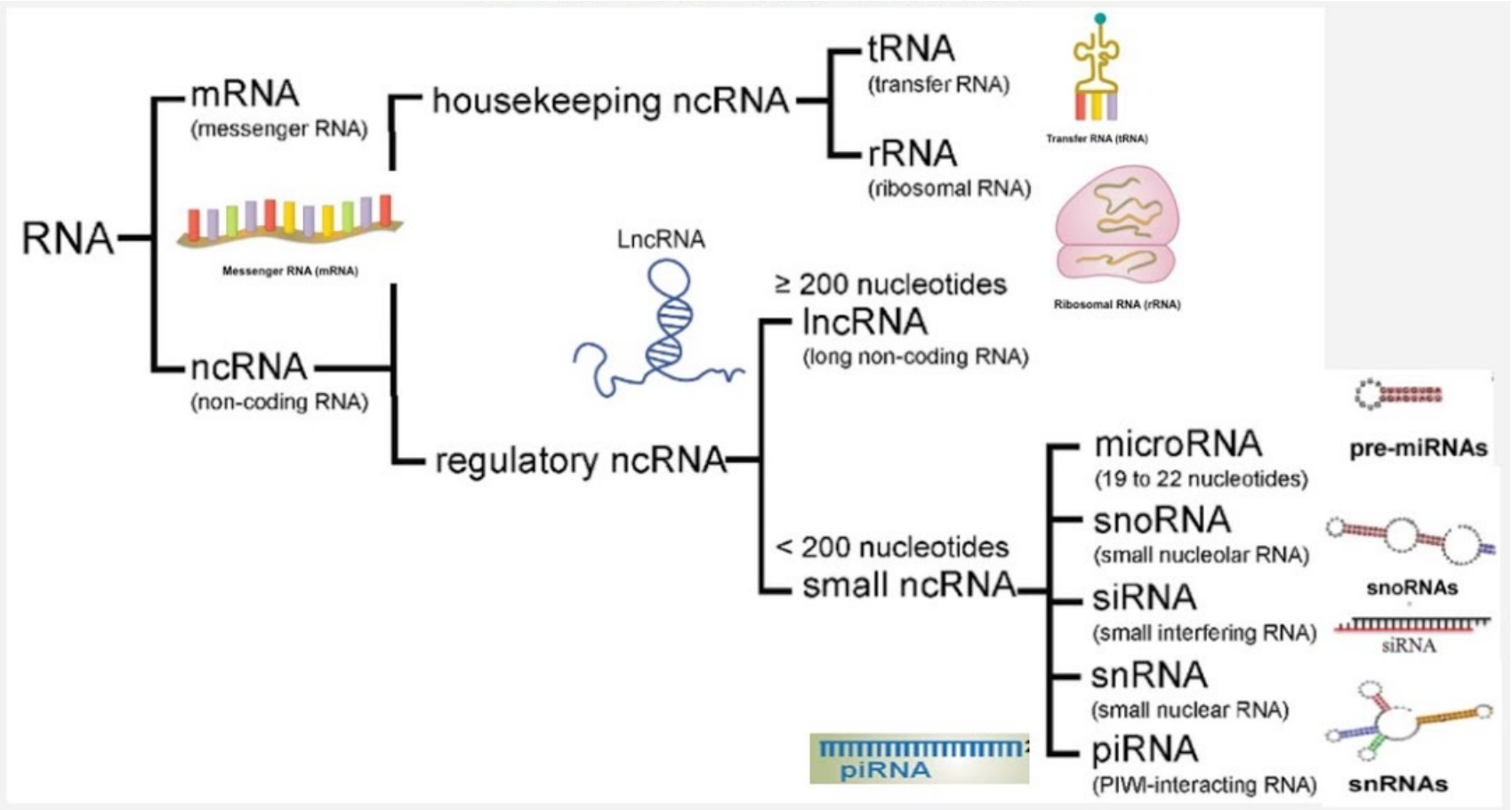
Types of eukaryotic RNAs



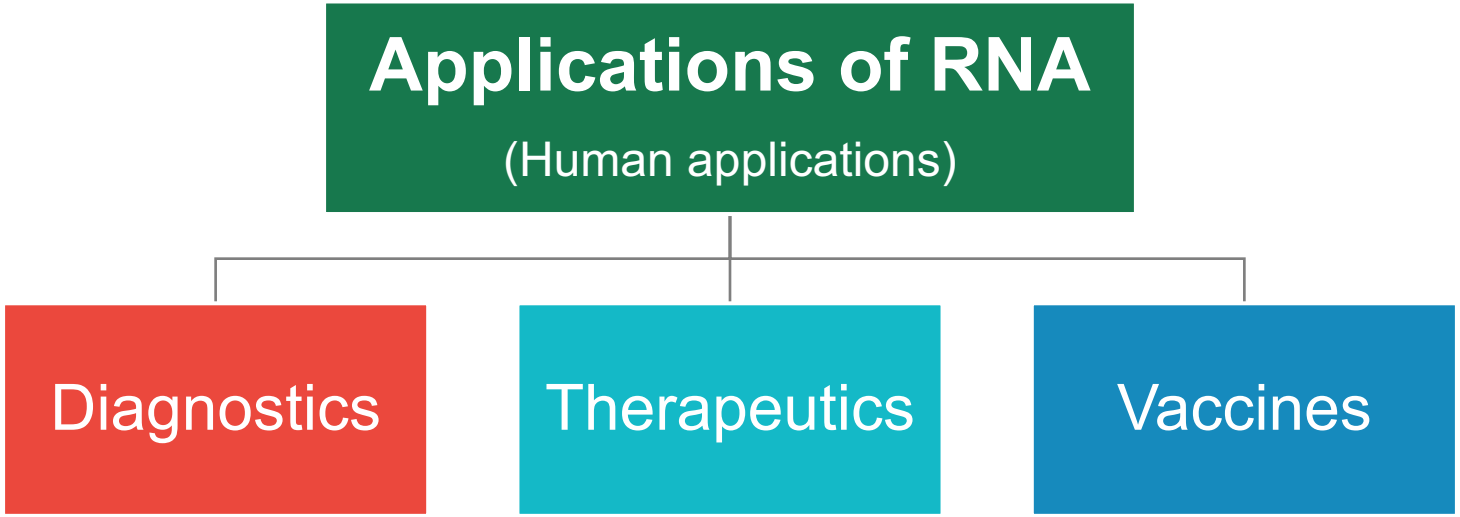
Types of eukaryotic RNAs



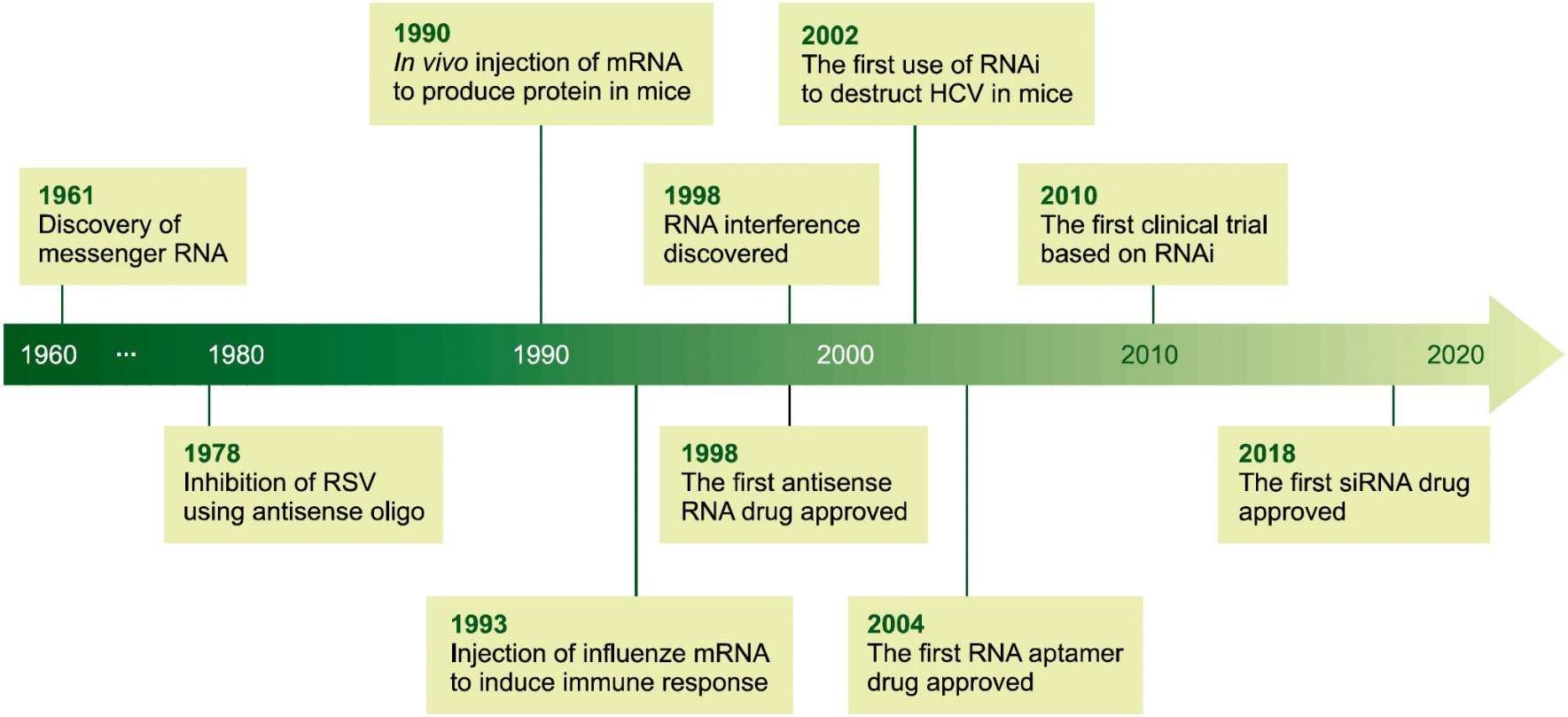
Types of eukaryotic RNAs



Applications of RNA: Overview



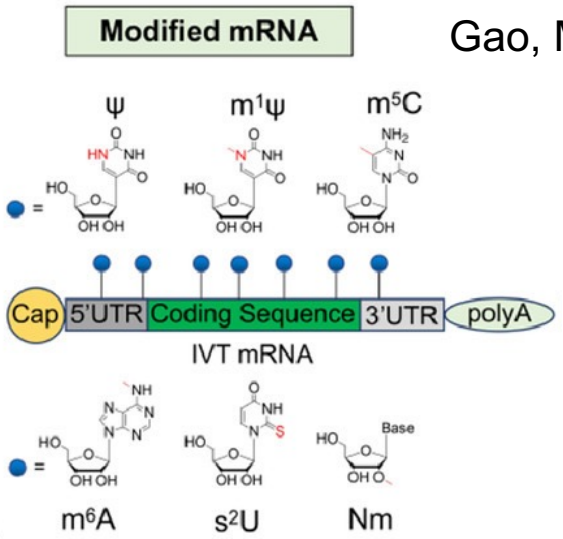
Timeline of key discoveries in RNA therapy

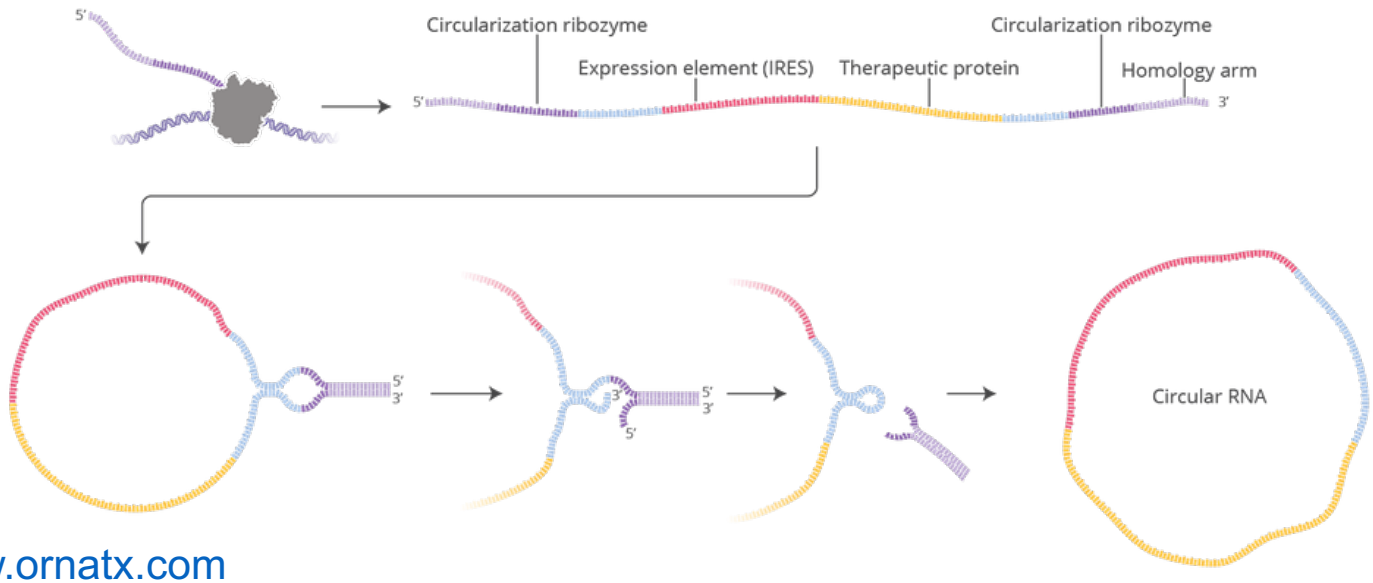


Kim, Y. K., *et al.* **Chonnam Med J.**, 2020

Modifying RNA for therapeutics

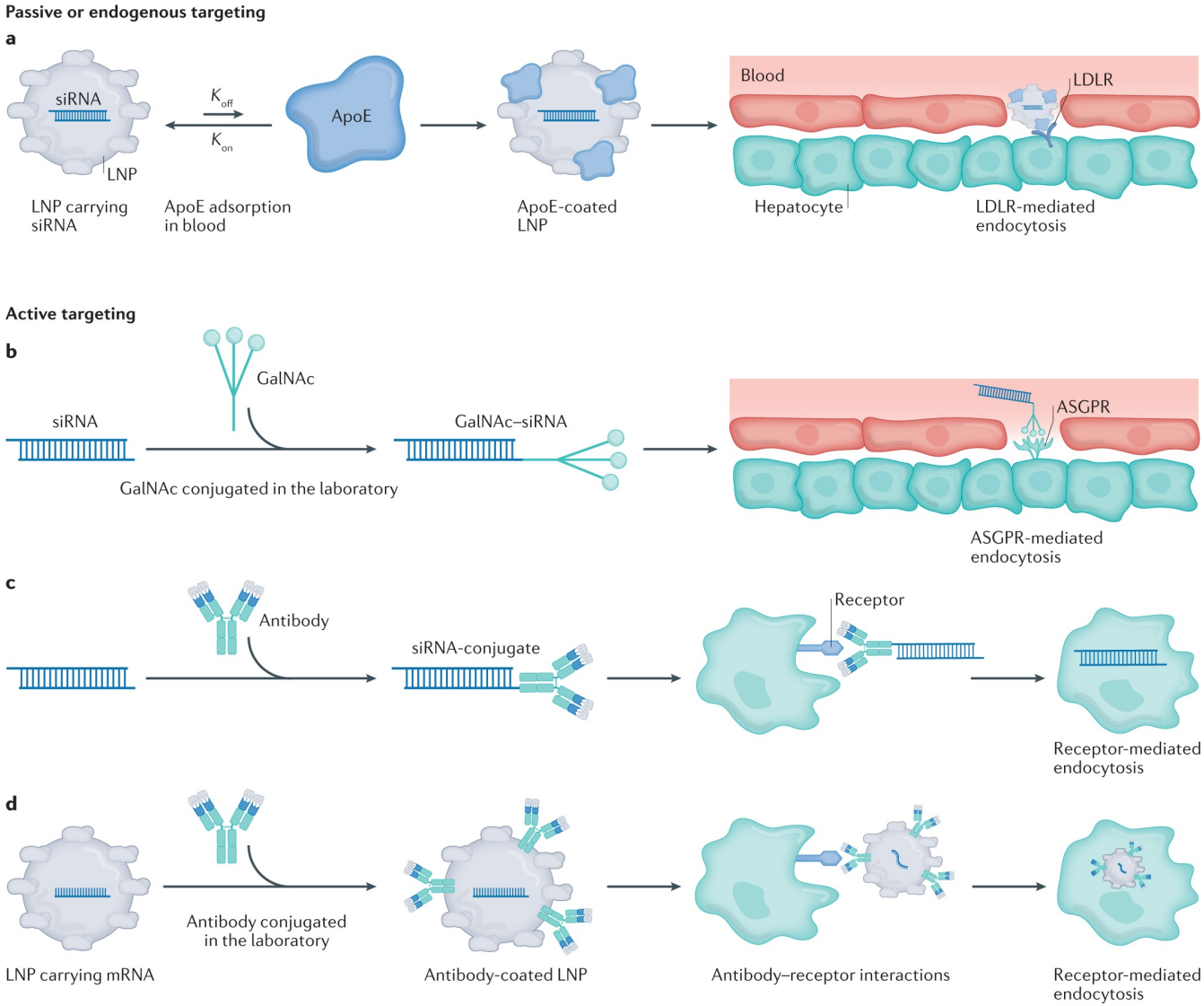
Gao, M., *et al.* **Acta Biomaterialia**, 2021





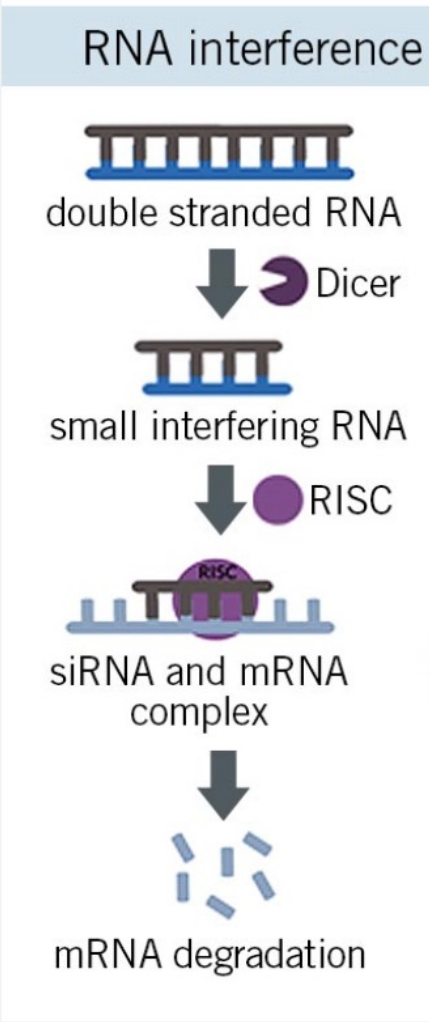
Source: www.ornatx.com

Delivery of RNA therapeutics



Paunovska, K., *et al.* **Nature Reviews Genetics**, 2022

Therapeutic applications of RNA

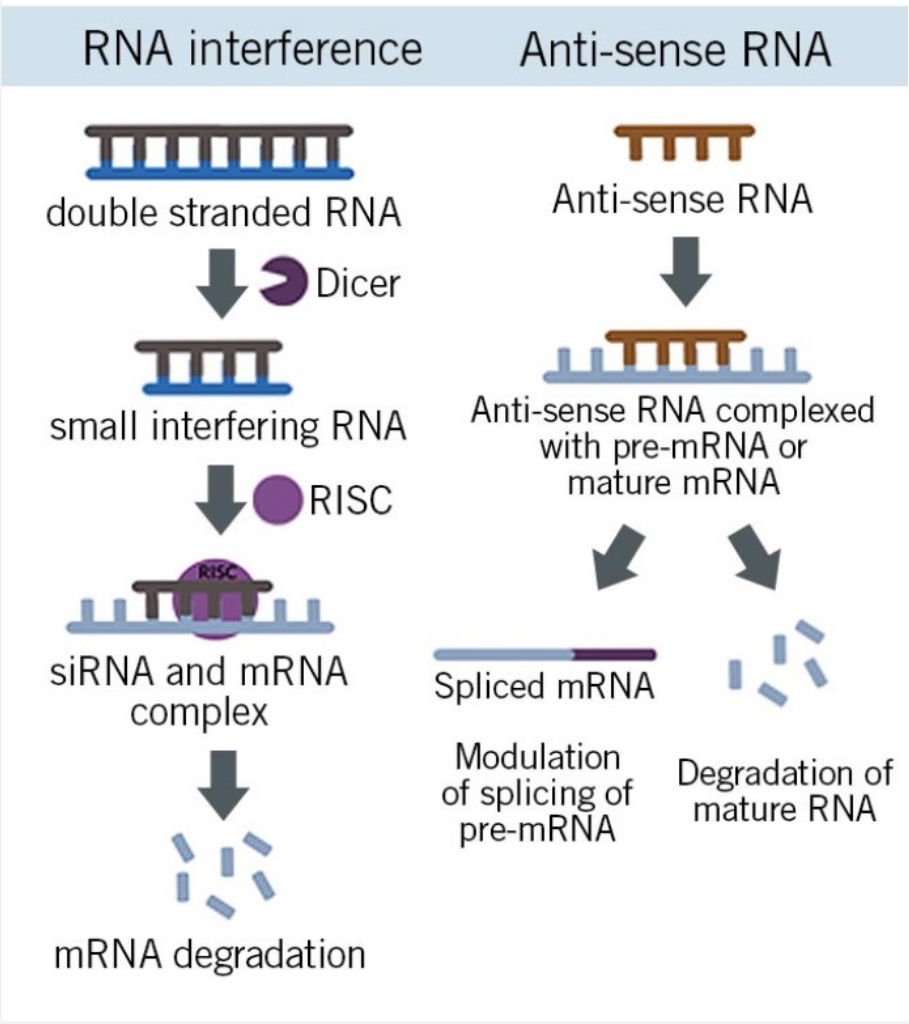


1. **Onpattro (Alnylam):** Polyneuropathy (FDA 2018) [siRNA]
2. **Givlaari (Alnylam):** Acute hepatic porphyria (FDA 2019) [siRNA]
3. **MRG-106 (Viridian):** Blood cancers (Phase II) [miRNA]

**Many more in various stages of clinical trials.

Source: the-dna-universe.com

Therapeutic applications of RNA

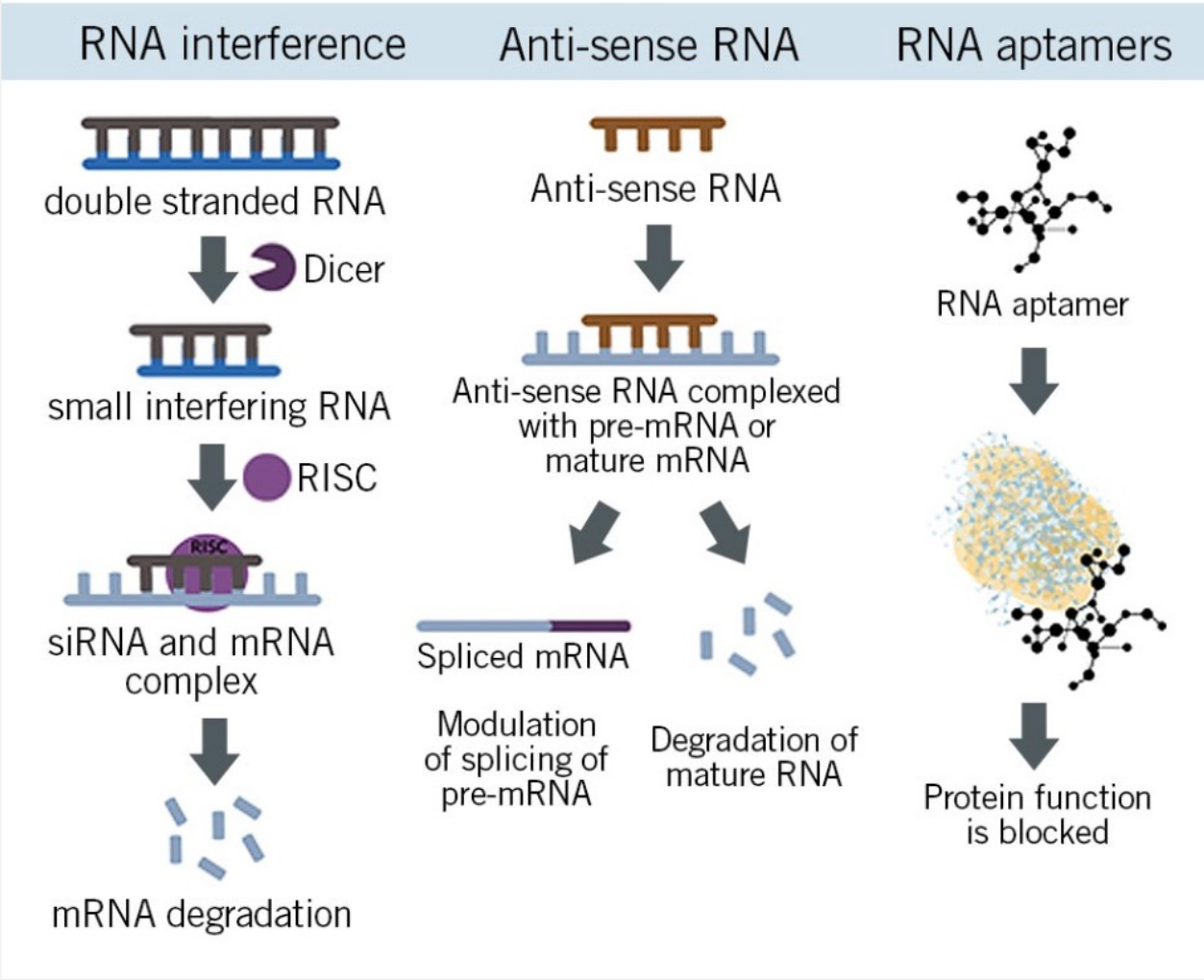


1. **Spinraza (Ionis):** Spinal muscle atrophy (FDA 2016)
2. **Exondys 51 (Sarepta):** Duchenne muscular dystrophy (FDA 2016)
3. **Tegsedi (Ionis):** Familial amyloid polyneuropathy (FDA 2018)

**Many more FDA approved and in various stages of clinical trials.

Source: the-dna-universe.com

Therapeutic applications of RNA

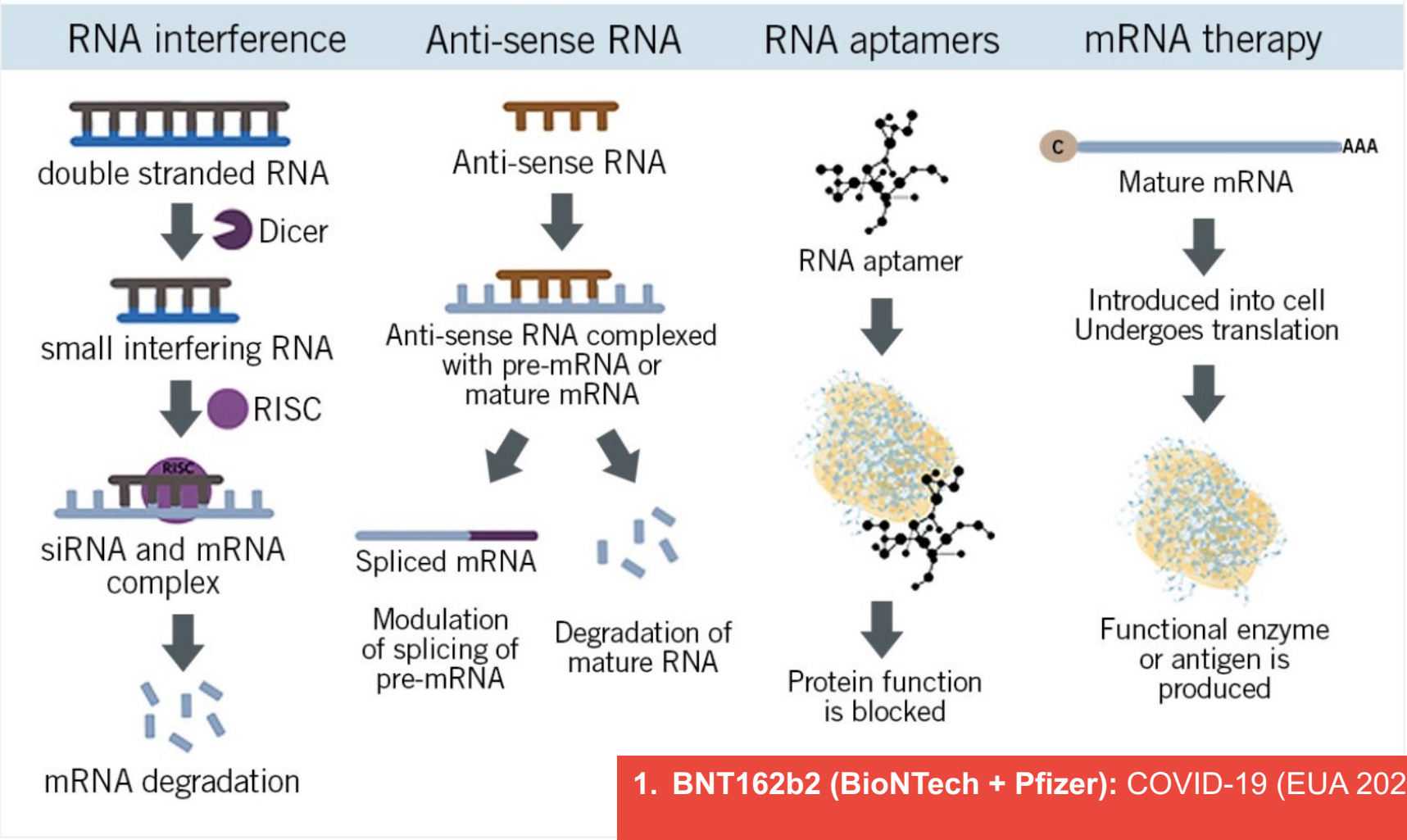


1. **Macugen (Bausch + Lomb):** Macular degeneration (FDA 2014)
2. **NOX-E36 (NOXXON):** Diabetic neuropathy (Phase II)
3. **NOX-A12 (NOXXON):** Brain cancer (Phase I/II)

**Many more in various stages of clinical trials.

Source: the-dna-universe.com

Therapeutic applications of RNA

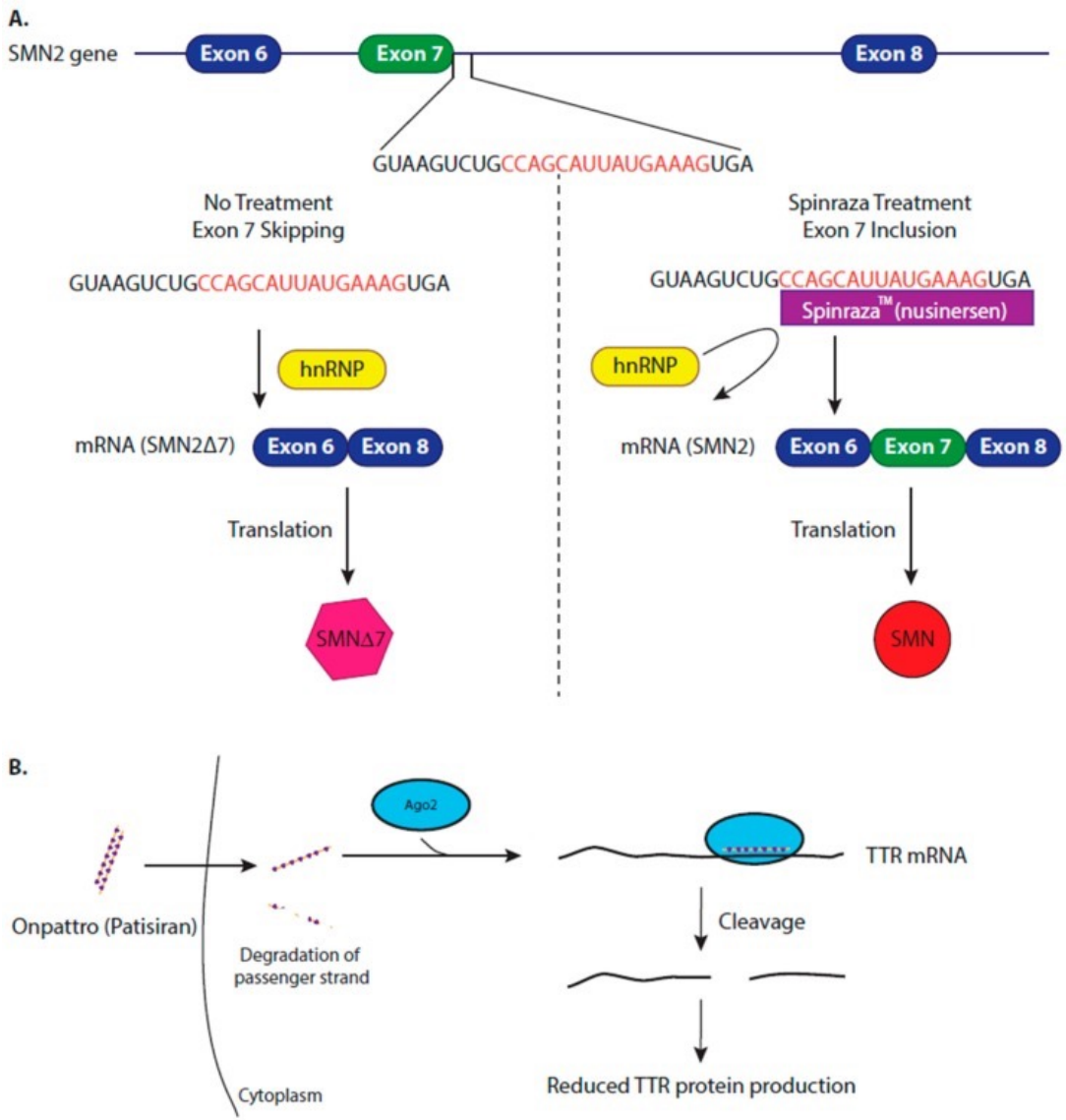


1. **BNT162b2 (BioNTech + Pfizer):** COVID-19 (EUA 2020)
2. **mRNA-1273 (Moderna):** COVID-19 (EUA 2020)

** Many more in various stages of clinical trials.

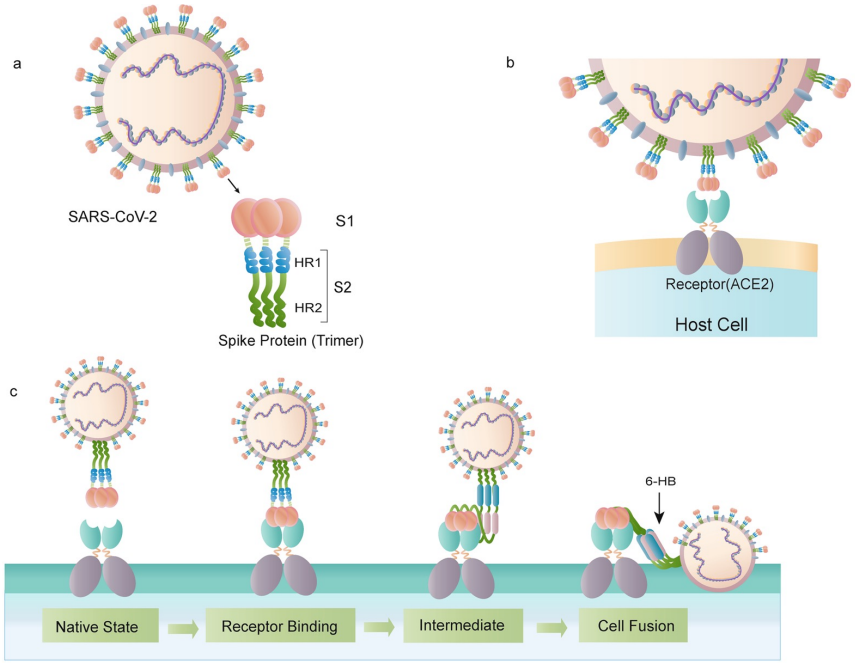
Source: the-dna-universe.com

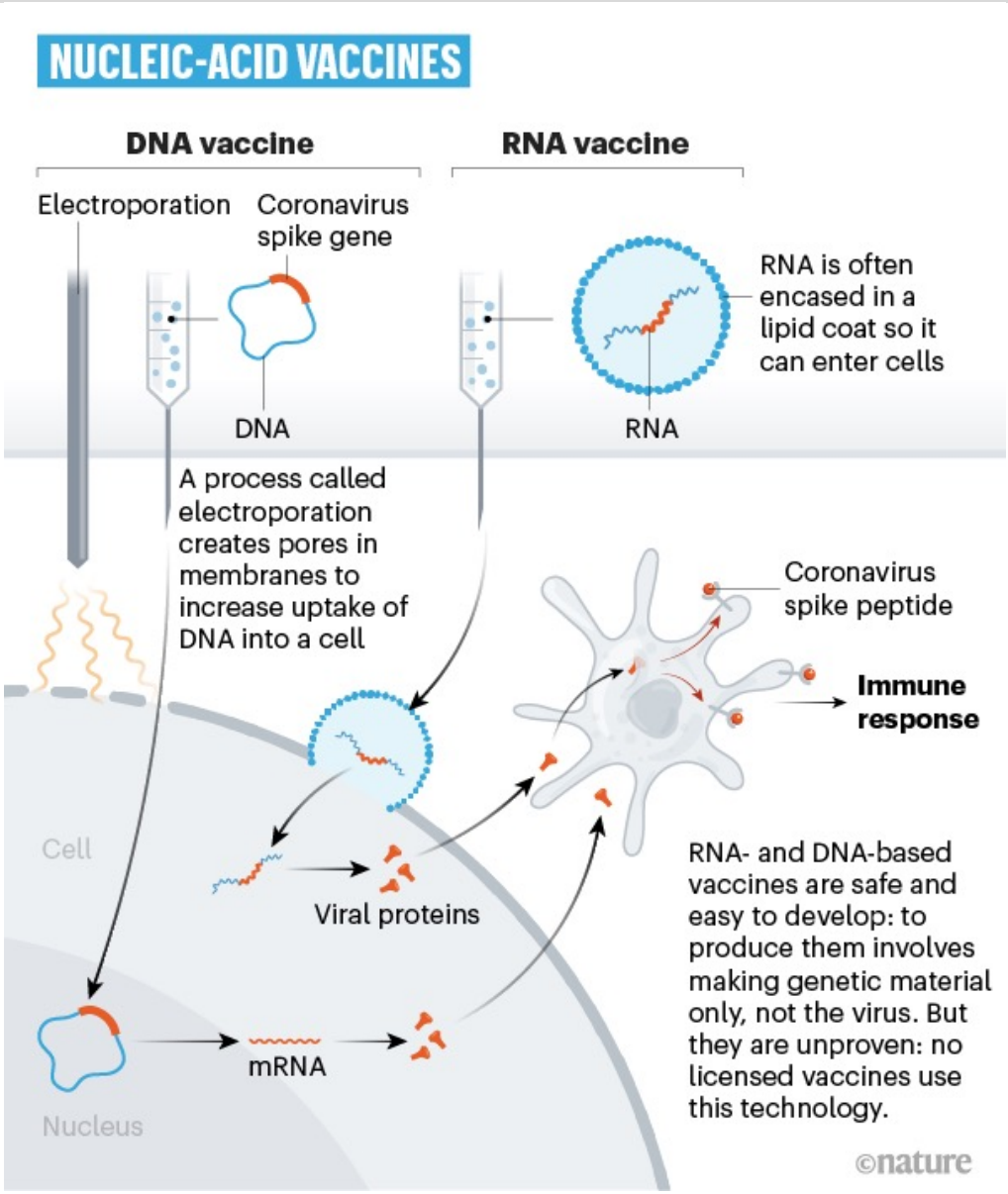
Mechanism of approved therapeutics



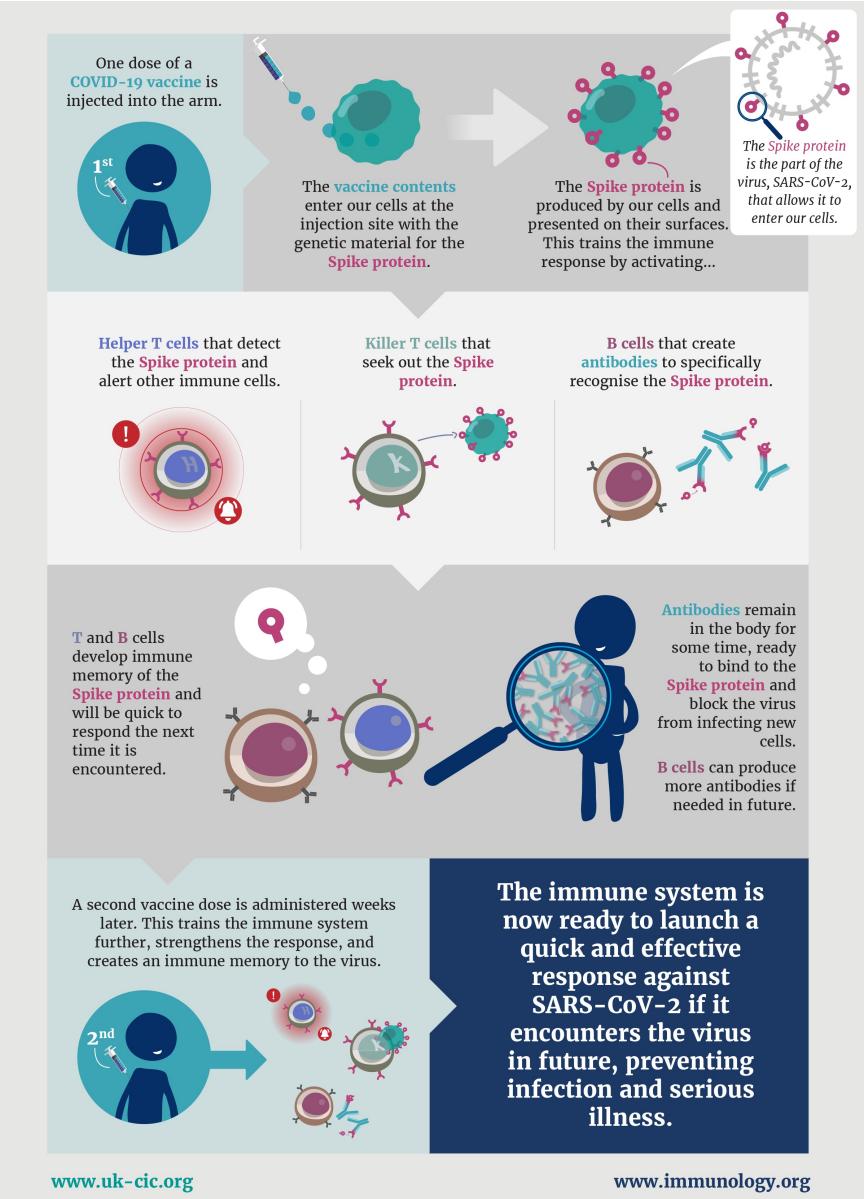
Goel, RR., *et al.* **Science**, 2021

mRNA vaccines (COVID-19)

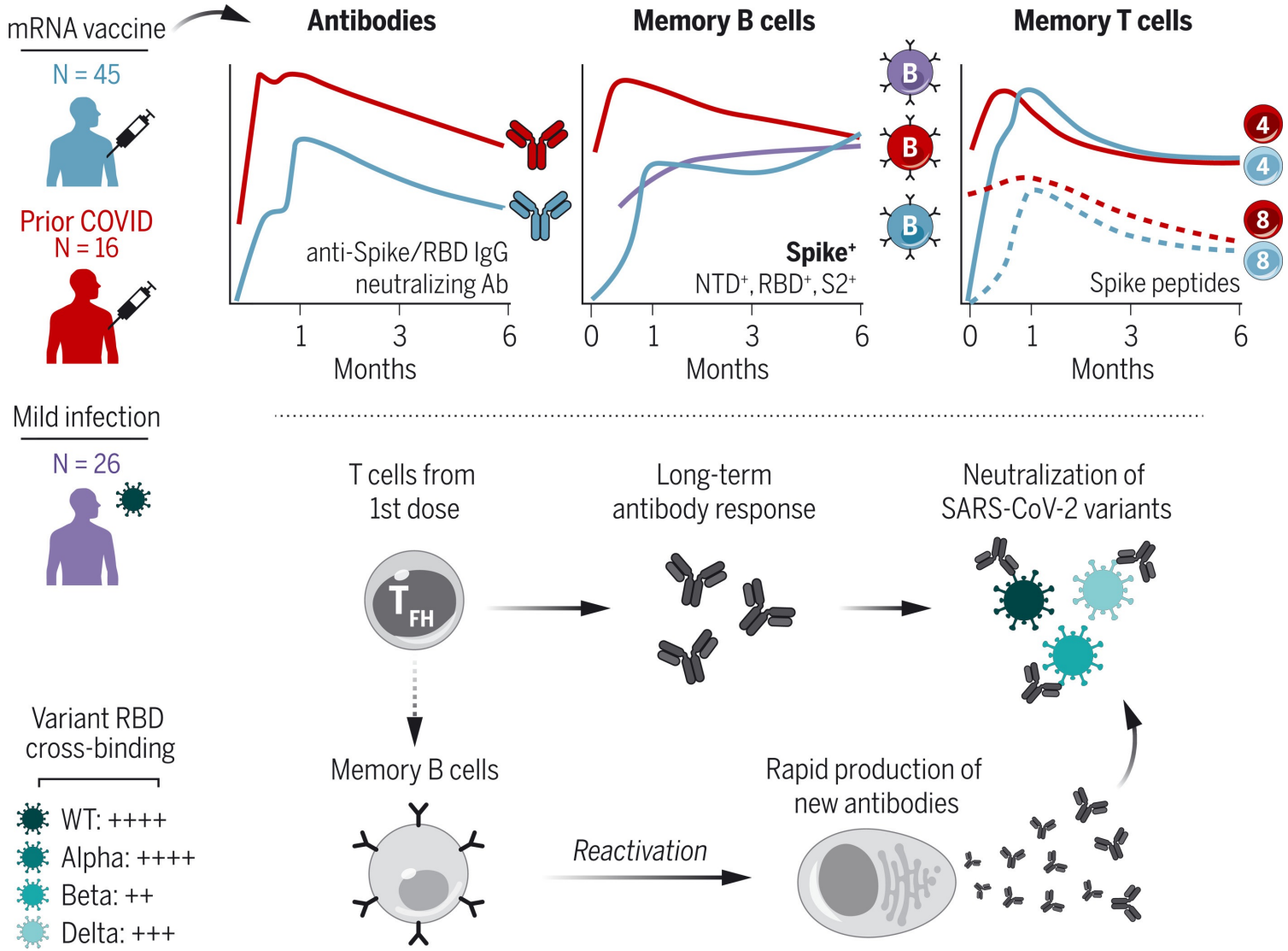




Immune response after COVID vaccination

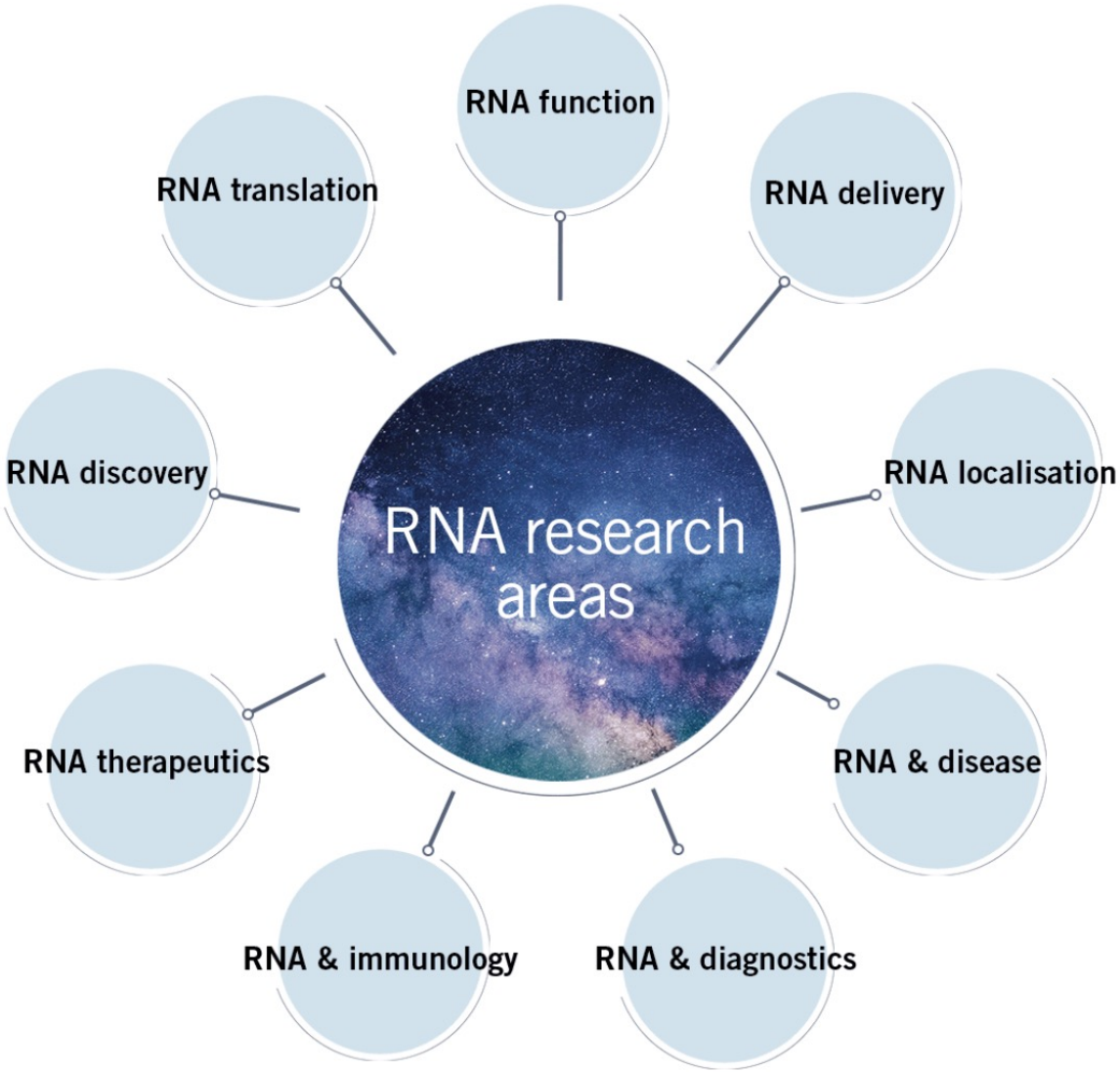


mRNA vaccines induce immune "memory"



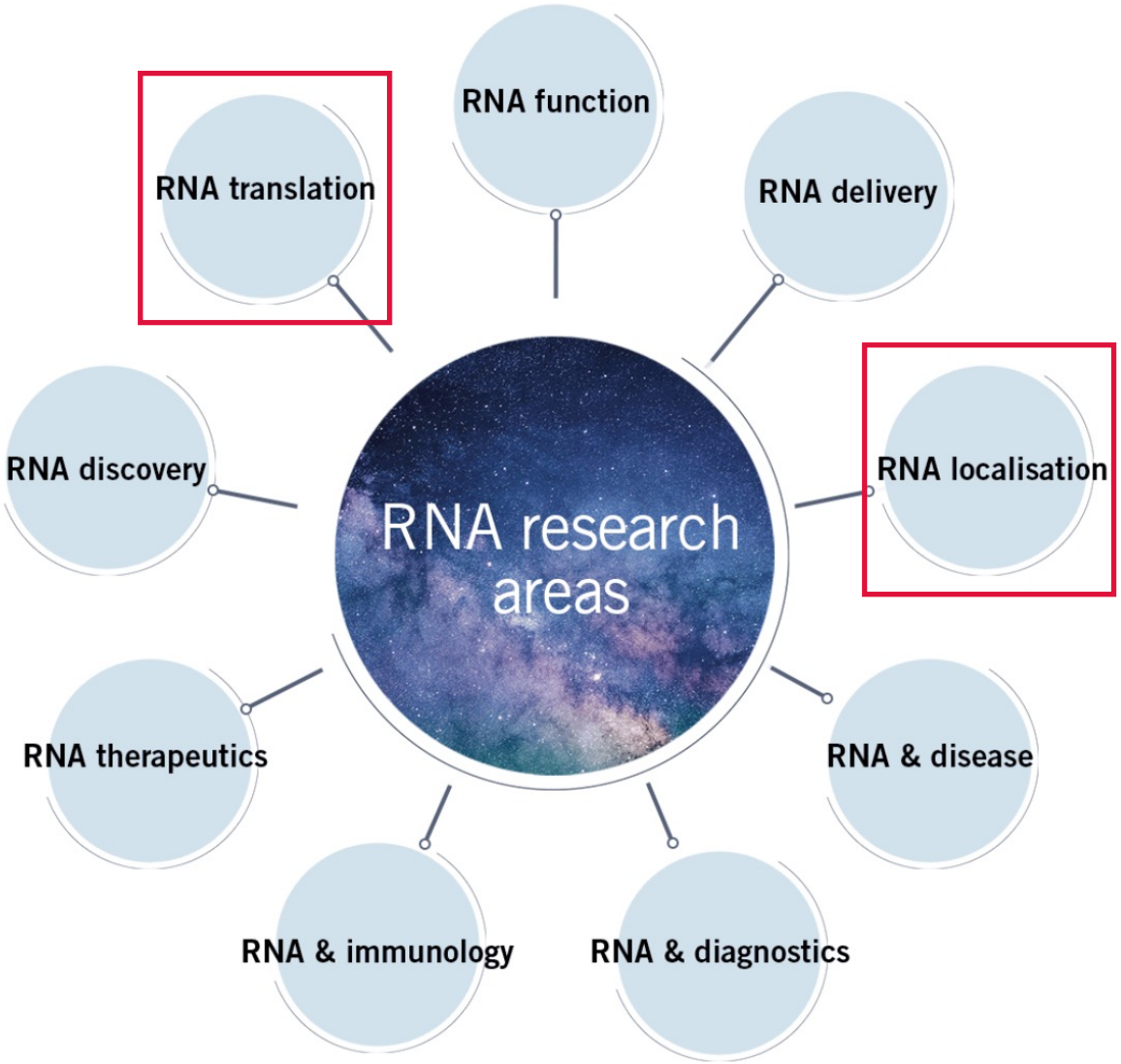
Goel, RR., *et al.* **Science**, 2021

RNA research areas



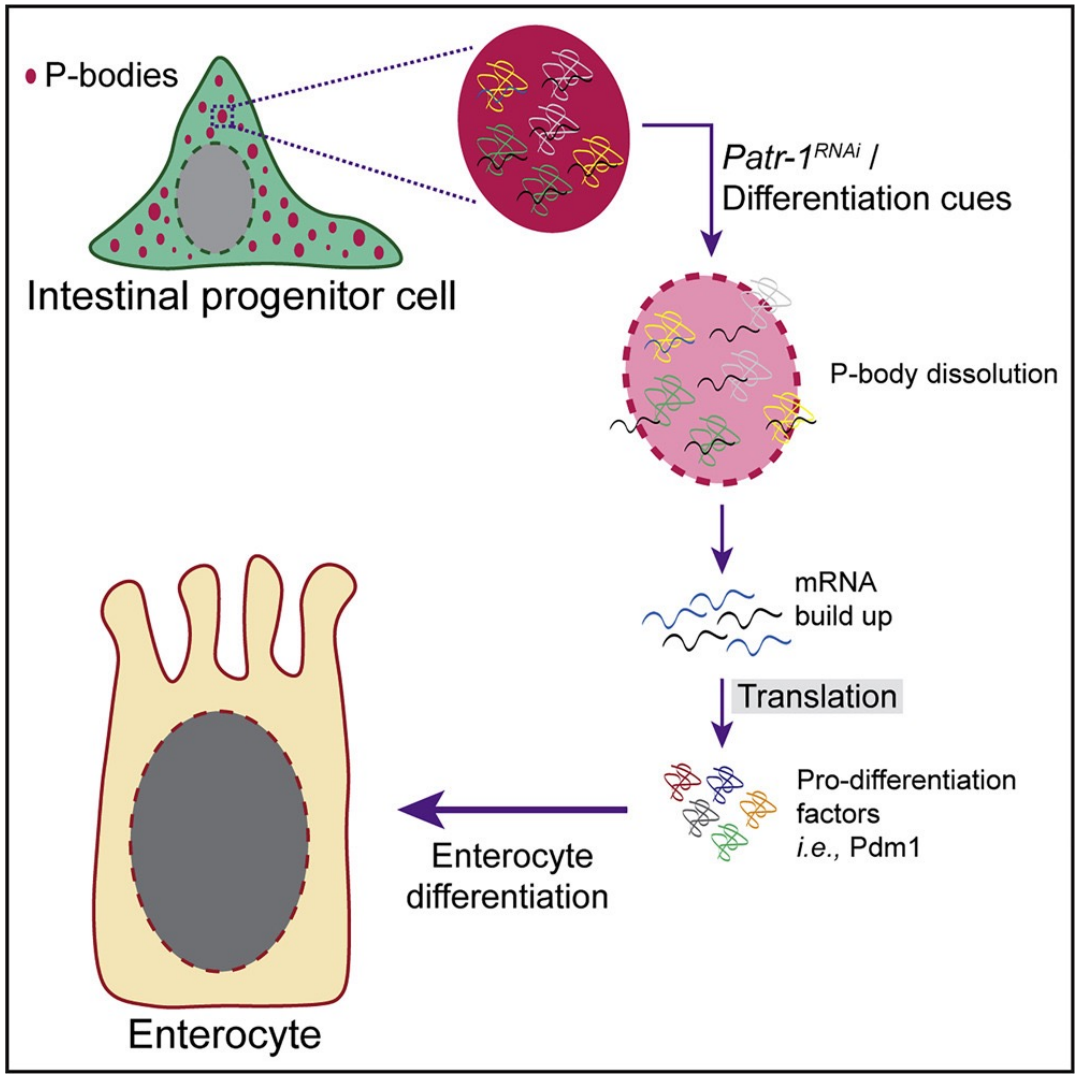
Source: the-dna-universe.com

RNA research areas



Source: the-dna-universe.com

P-bodies are required to block precocious stem cell differentiation



Buddika, K., et al., **Current Biology**, 2022

Stay in touch

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