

How to make sense of RNA-based therapeutics

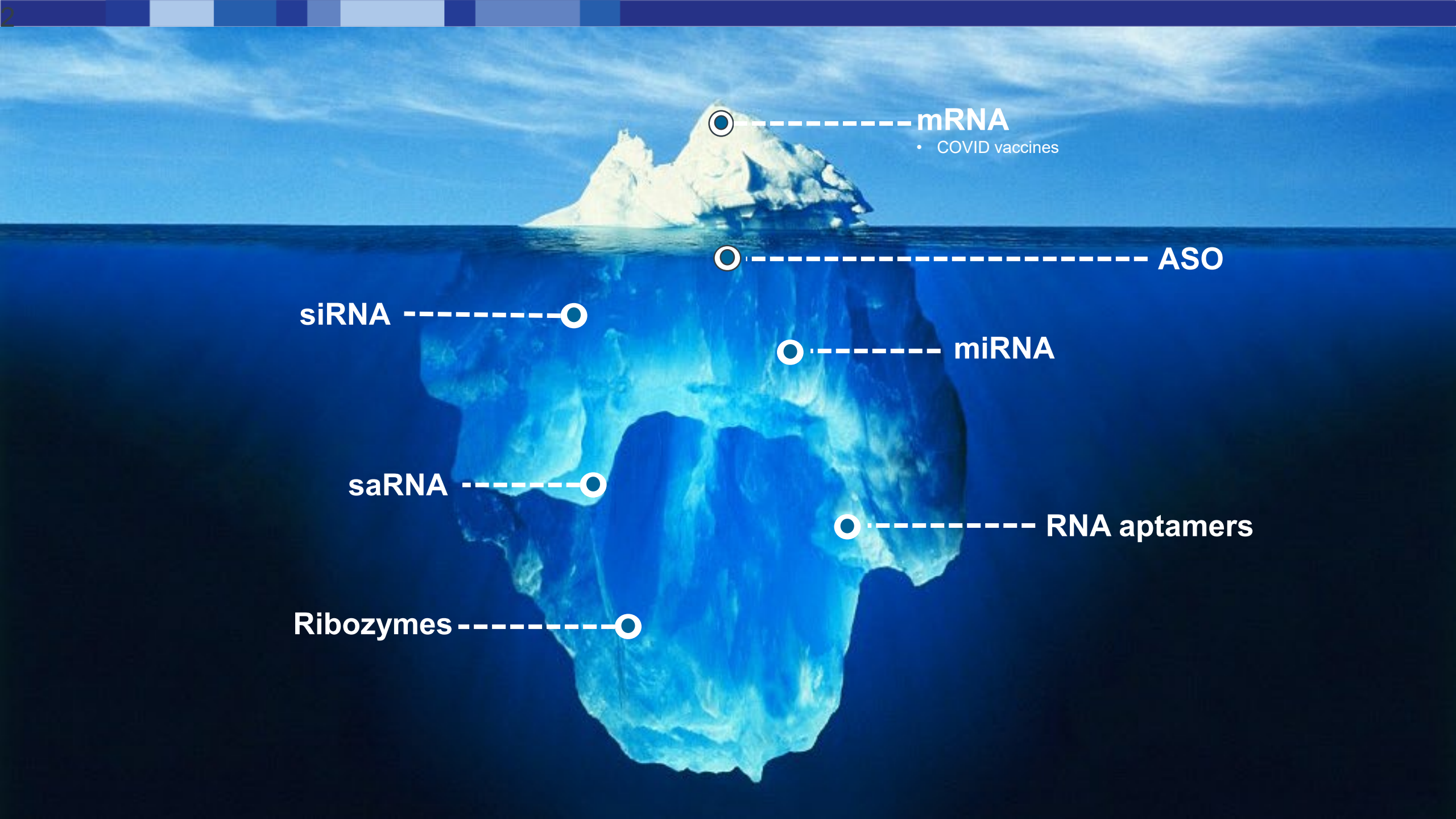
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OFFICIAL



mRNA
• COVID vaccines



ASO



siRNA



miRNA



saRNA



RNA aptamers



Ribozymes

Agenda

Overview of RNA-based therapeutics

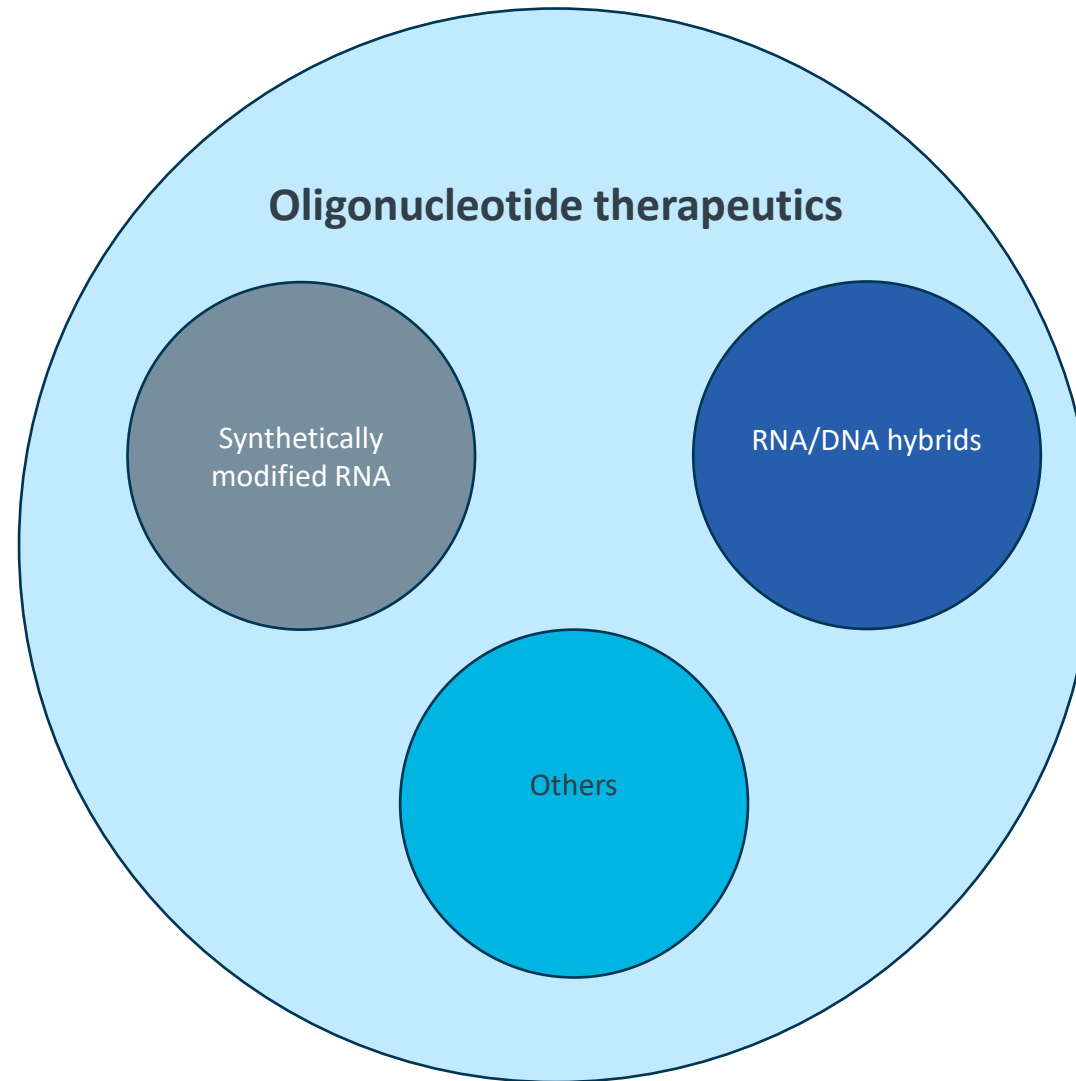
Landscape of RNA-based therapeutics development

Regulatory pathways

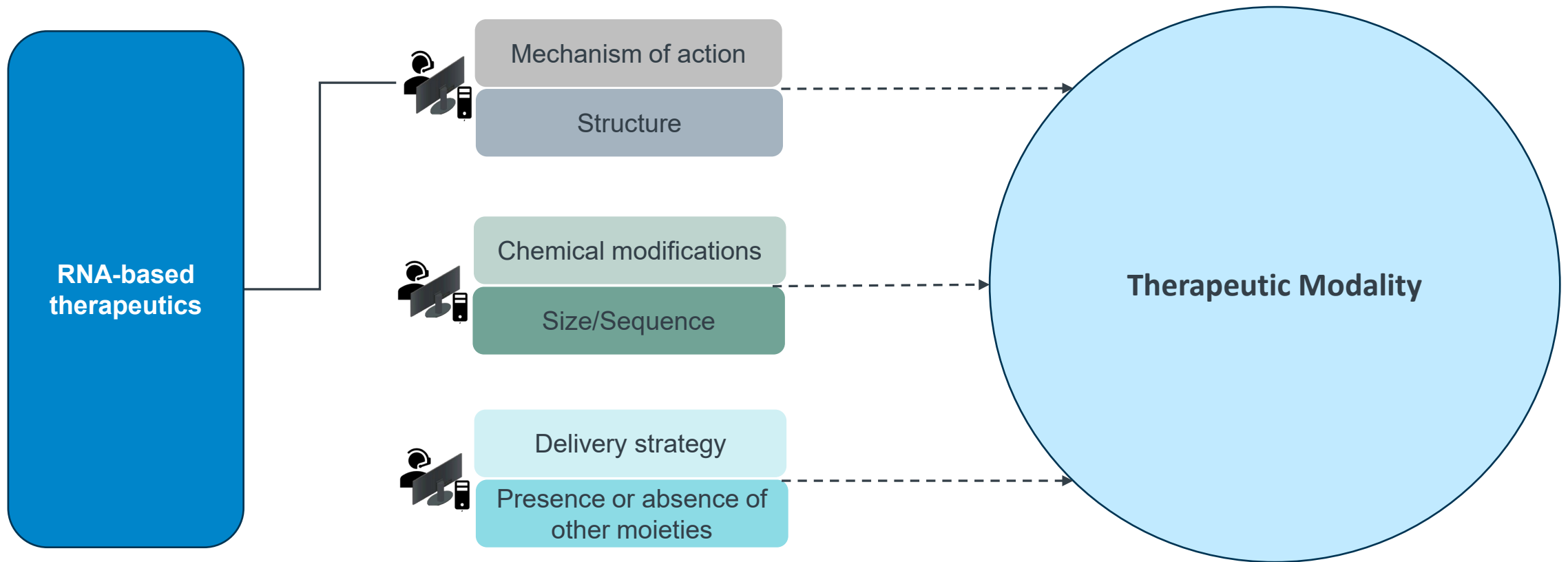
Q&A



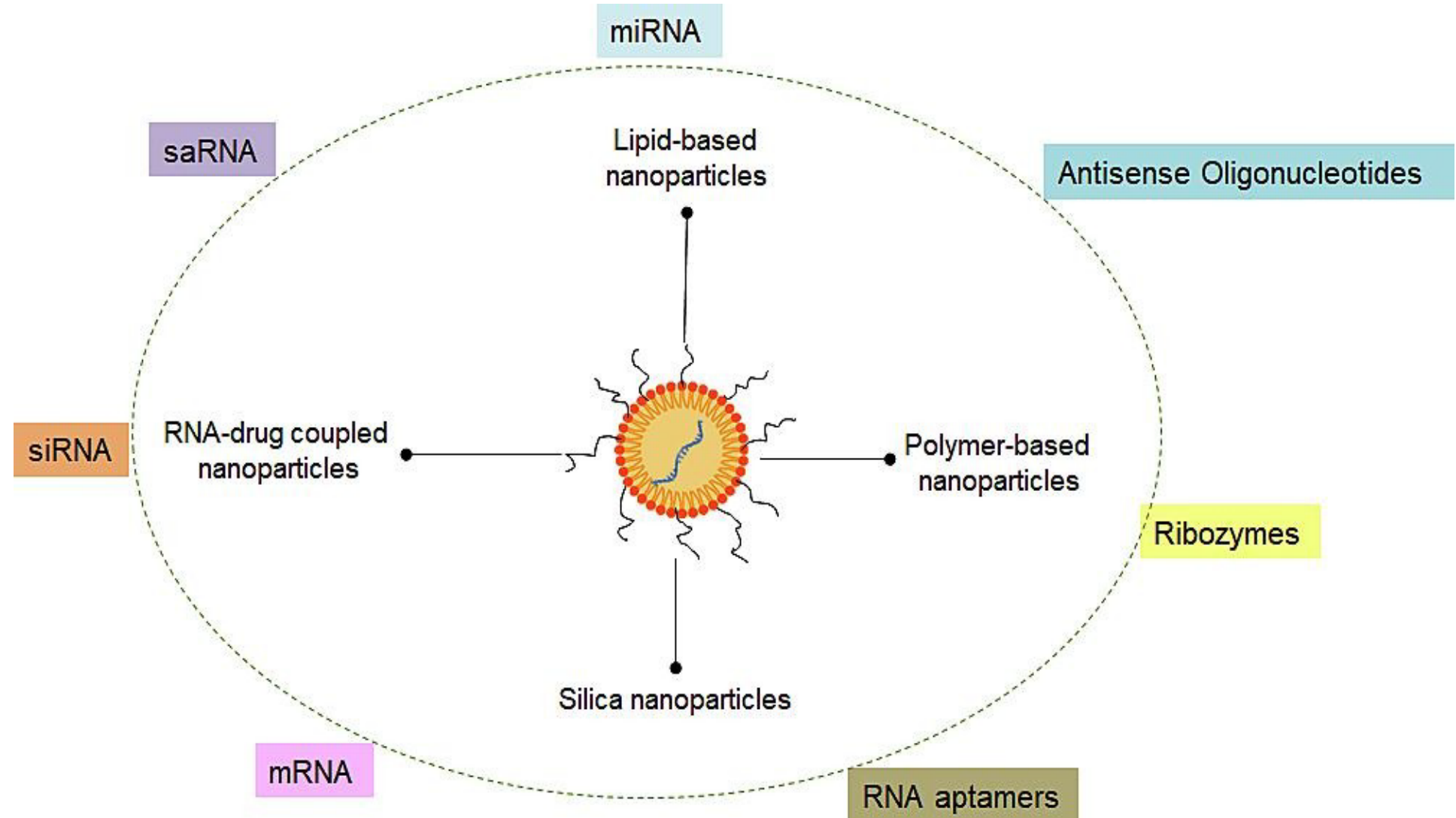
Oligonucleotide therapeutics – Where are we?



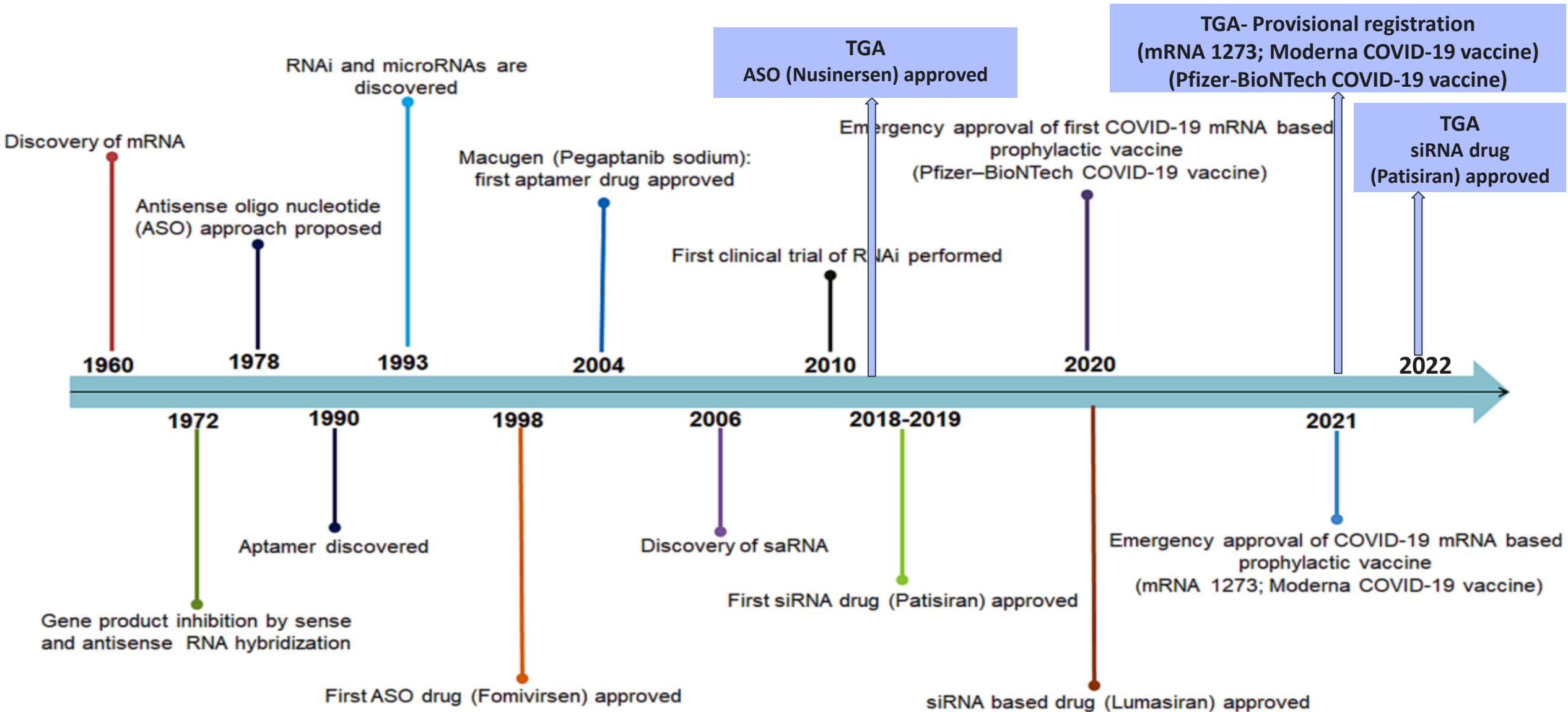
Oligonucleotide therapeutics – can differ?



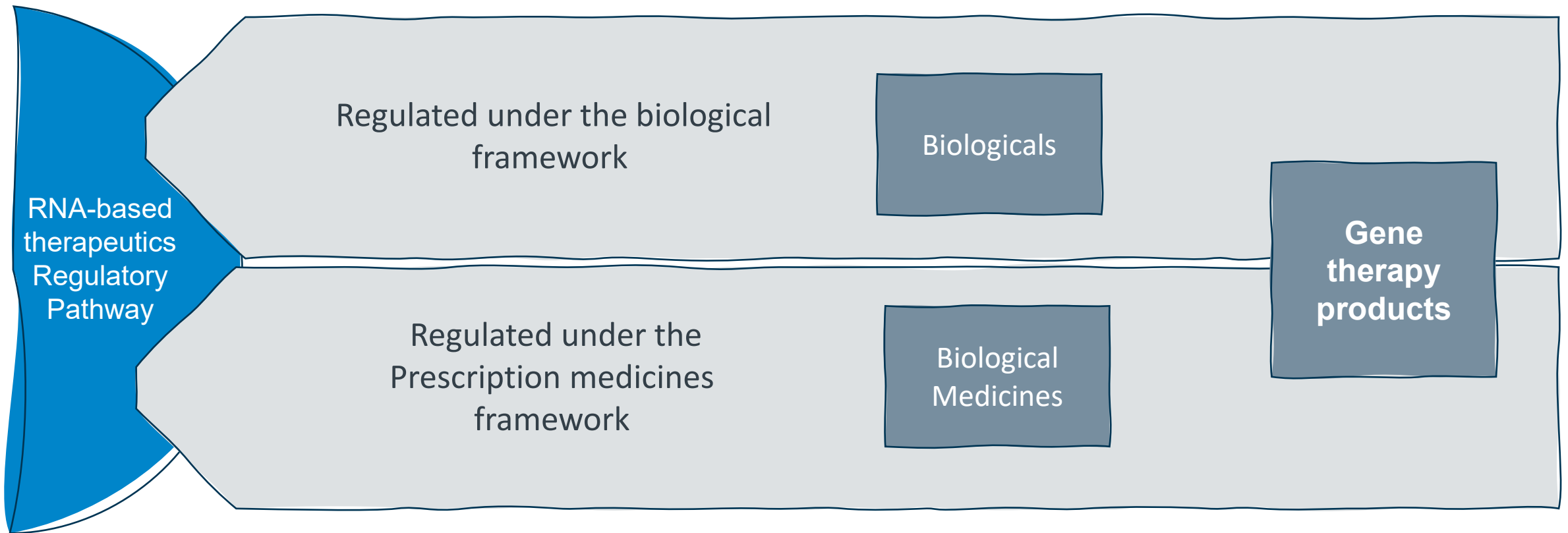
Overview of RNA-based therapeutics



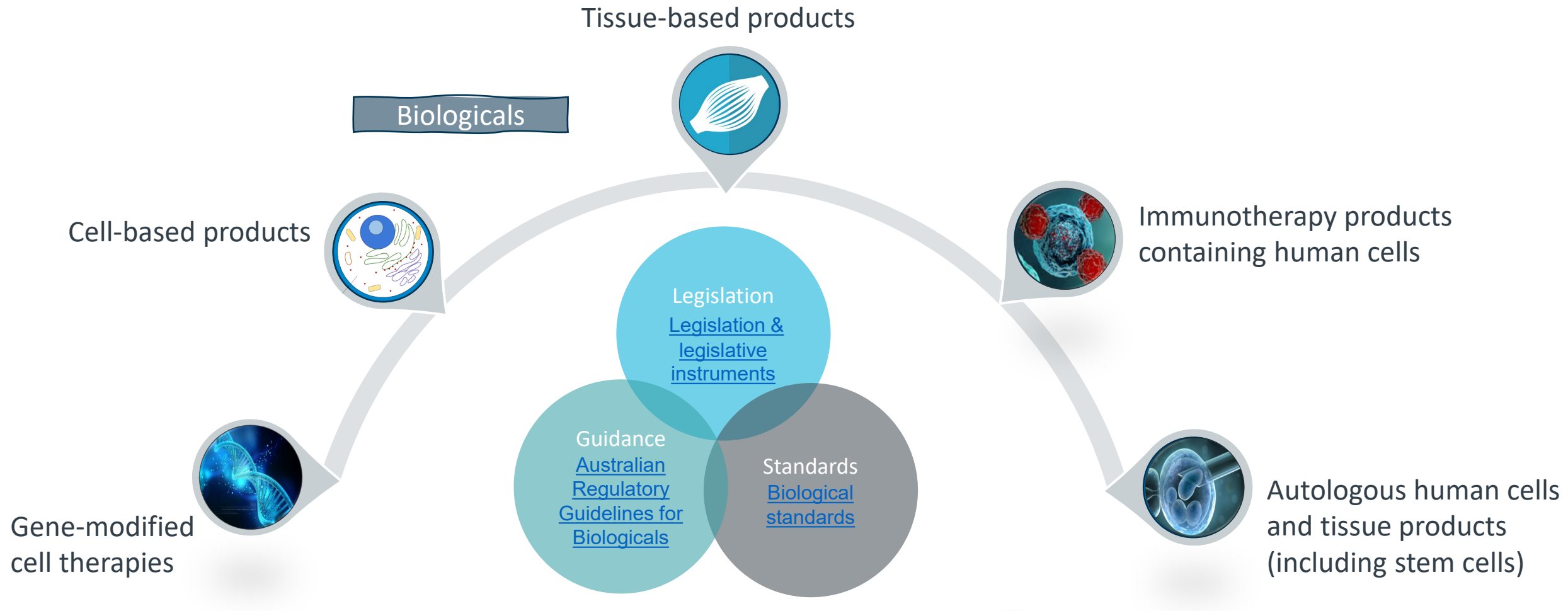
Milestones- the roadmap for RNA-based therapeutics



Regulatory Pathway- RNA-based therapeutics

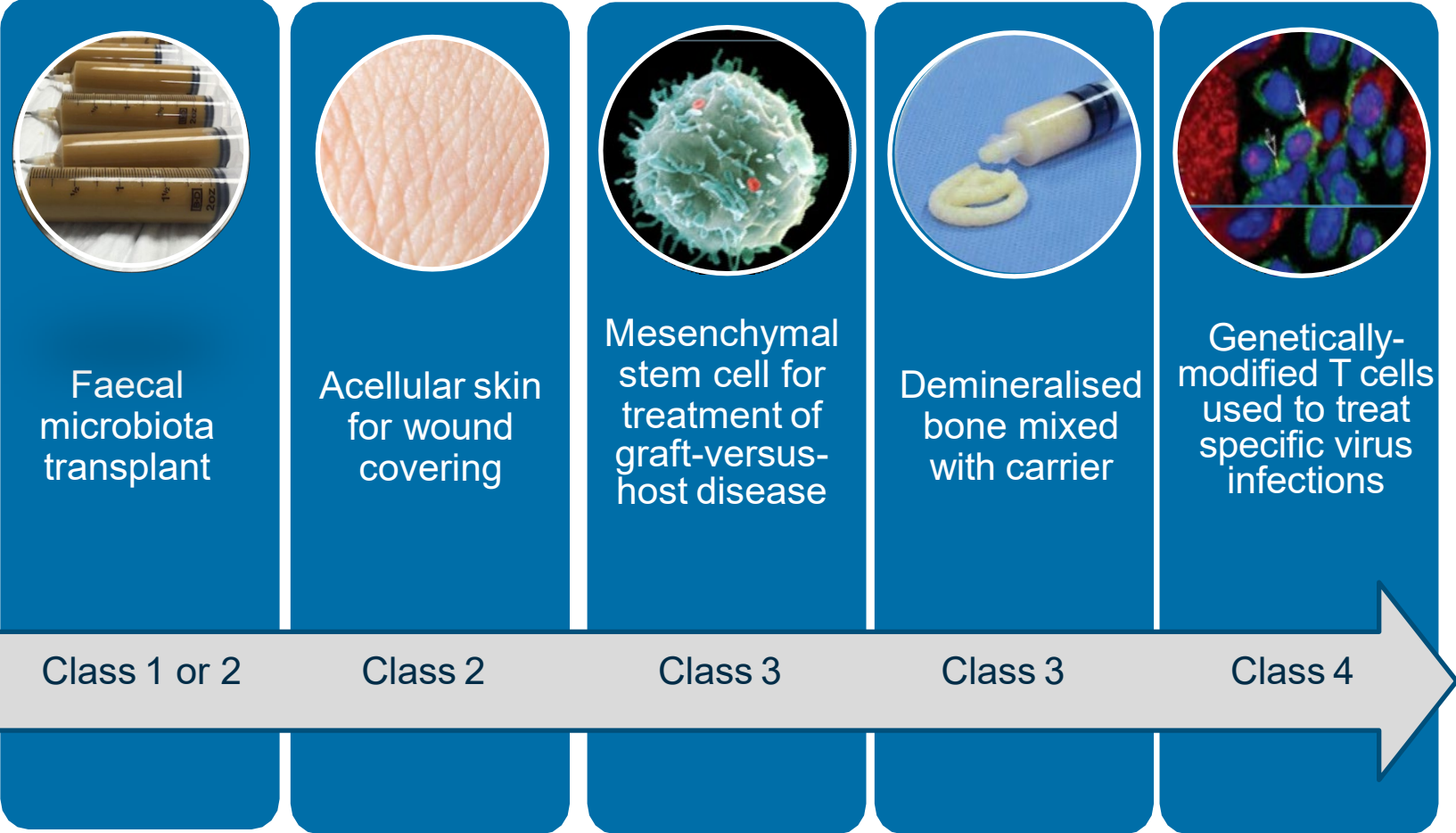


Biological framework



Biologicals are grouped into classes

Examples



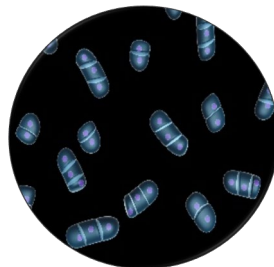
Class 3 biologicals

Definition

- for homologous use but have been prepared using more than minimal manipulation
- OR
- for non-homologous use, regardless of whether they have been prepared using minimal manipulation or more than minimal manipulation

Currently approved Class 3: chondrocytes

Examples of cell therapies: mesenchymal stem cells, T



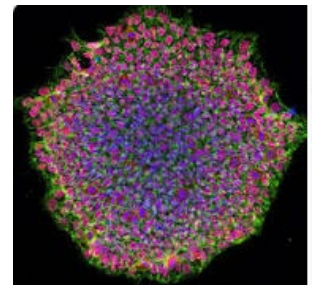
Class 4 biologicals

Definition (Schedule 16)

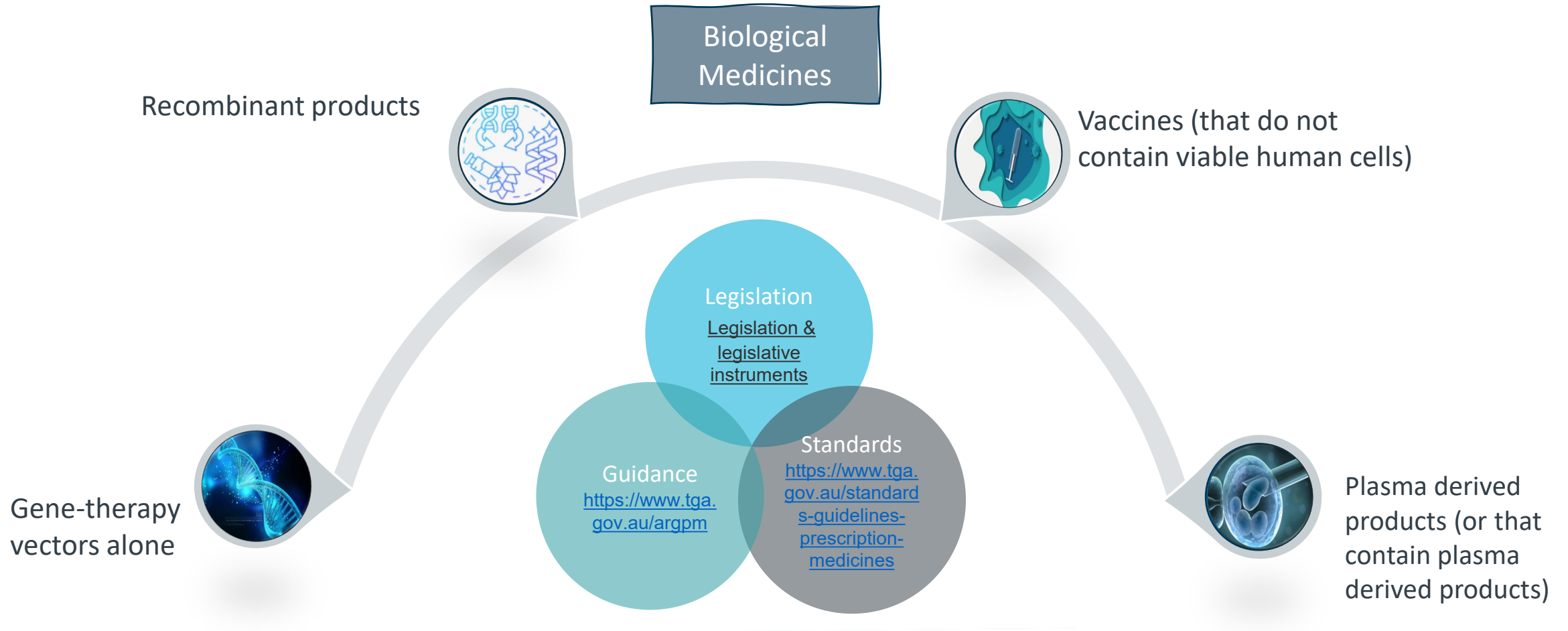
- Animal cells, tissues and organs
- Cells that have been modified to artificially introduce a function or functions of the cells or tissues; and
- the artificially introduced function or functions were not intrinsic to the cells
- pluripotent stem cells;
- biologicals derived from pluripotent stem cells

Currently approved Class 4: CAR-T therapies

Examples of cell therapies: iPSCs, xenogeneic cell therapy, gene-modified cell therapies



Prescription medicines framework



Gene therapy products

- The substance is used in or administered to human beings to regulate, repair, replace, add or delete a genetic sequence AND
- The substance is involved in the therapeutic, prophylactic, or diagnostic effect of the product

Regulatory Pathways for Gene therapy products

Type of gene therapy	Example	Regulatory pathway	Further information
Ex vivo (gene is delivered to cells outside of the body, which are then transferred back into the body)	CAR-T cells (human cells)	Class 4 biological	Australian regulatory guidelines for biologicals (ARGB)
In vivo (gene is transferred to cells inside the patient's body)	Adeno-associated virus, small silencing RNAs, CRISPR and other gene editing technologies	Prescription medicine	Australian Regulatory Guidelines for Prescription Medicines (ARGPM)

Takeaway– the evolving roadmap for RNA-based therapeutics



Therapeutic Goods Administration (TGA)

Exhibition booth No.1

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Website and link references

TGA website	www.tga.gov.au
Australian regulatory guidelines for biologicals (ARGB)	https://www.tga.gov.au/publication/australian-regulatory-guidelines-biologicals-argb
Australian Regulatory Guidelines for Prescription Medicines (ARGPM)	https://www.tga.gov.au/publication/australian-regulatory-guidelines-prescription-medicines-argpm
Manufacturing therapeutic goods	https://www.tga.gov.au/manufacturing-therapeutic-goods
Manufacturing biologicals	https://www.tga.gov.au/manufacturing-biologicals
Australian code of good manufacturing practice for human blood and blood components, human tissues and human cellular therapy products	https://www.tga.gov.au/publication/australian-code-good-manufacturing-practice-human-blood-and-blood-components-human-tissues-and-human-cellular-therapy-products

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