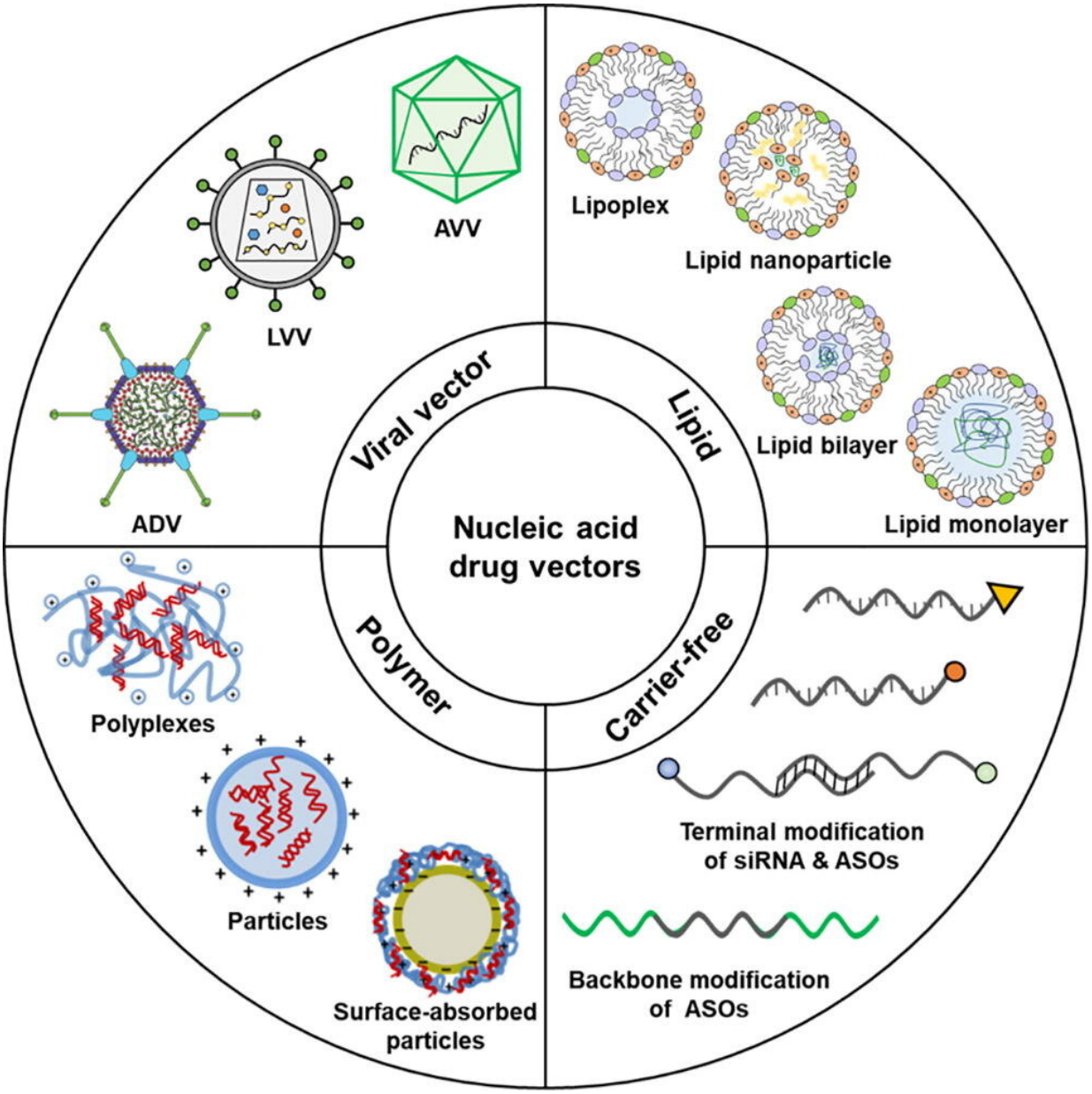
Biosafety assessment of delivery systems for clinical nucleic acid therapeutics

May 5 2022



Current nucleic acid drug delivery systems in clinical trials. Schematic of AdV is from Parks et al. [6], copyright 2020. Schematics of polyplexes, particles, and surface-absorbed particles are from Goycoolea et al. [7], copyright 2018. Credit: *Biosafety and Health* (2022). DOI: 10.1016/j.bsheal.2022.03.003

Nucleic acid therapeutics, which involve transferring exogenous genes inside target cells, are a promising clinical treatment option that can regulate gene expression at the transcriptional or post-transcriptional level.

Ideally, this kind of treatment modality will not lead to an unwanted immune response. Compared with traditional treatment methods, nucleic acid therapeutics can achieve prolonged and stable curative effects. As an emerging treatment method, nucleic acid therapeutics have played an increasingly important role in clinical settings for the treatment of various conditions, including infectious diseases, cancer, immune-related diseases, and monogenetic diseases.

To date, a large number of clinical trials have been conducted, and more than 30 nucleic acid drugs have been approved, highlighting the strong potential of this approach in clinical practice. Diverse carriers are used to protect nucleic acids from being degraded and to help them reach their targets accurately. However, some carriers are known to cause negative effects on the release and expression of nucleic acid drugs as well as adverse effects such as allergic reactions and accumulation in the liver. Therefore, biosafety assessment of delivery systems before their application in clinical settings is critical.

In this review appearing in *Biosafety and Health*, the authors describe different delivery systems for nucleic acid drugs and discuss their biosafety in both preclinical and clinical studies, with particular focus on

the carriers themselves, drug administration method, and overall treatment of the disease.

More information: Zhimin Li et al, Biosafety assessment of delivery systems for clinical nucleic acid therapeutics, *Biosafety and Health* (2022). DOI: 10.1016/j.bsheal.2022.03.003