

Supplementary Materials:

Nano-Encapsulation and Conjugation Applied in the Development of Lipid Nanoparticles Delivering Nucleic Acid Materials to Enable Gene Therapies

Linh Dinh, Lanesa Mahon and Bingfang Yan *

Division of Pharmaceutical Sciences, James L. Winkle College of Pharmacy, University of Cincinnati, Cincinnati, OH 45229, USA; dinhlk@ucmail.uc.edu (L.D.); mahonls@mail.uc.edu (L.M.)

* Correspondence: yanbg@ucmail.uc.edu; Tel.: 513-558-6279

Table S1. Available LNP products for nucleic therapeutics.

Name	Trade Name	Sponsor	Approval Date	Cargo	Phase	Indication
<i>In Clinical Trials</i>						
mRNA-2752		Moderna, Inc. (Cambridge, MA, USA) and AstraZeneca (Cambridge, UK)		mRNA	I	Advanced Malignancies
mRNA-1345		Moderna, Inc. (Cambridge, MA, USA)		mRNA	I	Respiratory Syncytial Virus
mRNA-2416				mRNA	I/II	Advanced Malignancies
PF-07252220		Pfizer Inc. (New York, NY, USA) and BioNTech (Mainz, Germany)		mRNA	III	Influenza
PF-07845104				saRNA	I	
PF-07926307				mRNA	I	COVID-19 and Influenza
PF-07911145				mRNA	I/II	Varicella
ARB-001467				siRNA	II	
AB-729	Imdusiran (in combination with Peg-IFN α -2a)	Arbutus Biopharma Corporation (Warminster Township, PA, USA)		RNAi	I/II	Hepatitis B
PRO-040201				siRNA	Terminated, phase I	Hypercholesterolemia
JCXH-212				srRNA	I/II	Cancers
JCXH-211				srRNA	I/II	Glioma
JCXH-105				srRNA	I/II	Shingles
JCXH-221		Immorna Biotechnologies (Hangzhou, Zhejiang, China)		mRNA	I/II	COVID-19
JCXH-107				mRNA	Preclinical/I	Influenza
JCXH-108				mRNA	Preclinical/I	Respiratory Syncytial Virus
JCXH-301				mRNA	Preclinical/I	Gaucher's Disease

EphA2 siRNA		M.D. Anderson Cancer Center	siRNA	I	Advanced or Recurrent Solid Tumors
NTLA-2002		Intellia Therapeutics, Inc. (Cambridge, MA, USA)	CRISPR Cas9 mRNA, gRNA	I/II	Hereditary Angioedema
DCR-MYC		Dicerna Pharmaceuticals, Inc., a Novo Nordisk company (Lexington, MA, USA)	siRNA	Terminated, phase I/II	Solid tumors, hepatocellular carcinoma
DCR HBVS			RNAi	I	Chronic Hepatitis B Virus
TKM-080301		Arbutus Biopharma (Warminster Township, PA, USA) and National Cancer Institute	siRNA	Completed phase I/II	Gastrointestinal neuroendocrine tumors, adrenocortical carcinoma tumors, advanced hepatocellular carcinoma
ALN-PCS02		Alnylam Pharmaceuticals (Cambridge, MA, USA)	siRNA	Completed, phase I	elevated LDL-cholesterol
ALN-VSP02			siRNA	Completed, phase I,	Solid tumors
ND-L02-s0201		Bristol-Myers Squibb Pharmaceuticals (Princeton, NJ, USA)	siRNA	Completed, phase I	Hepatic fibrosis
ARCT-810	LUNAR®-OTC	Arcturus Therapeutics, Inc. (San Diego, CA, USA)	mRNA	I/II	Ornithine Transcarbamylase Deficiency
ARCT-032	LUNAR®-CF		mRNA	I	Cystic Fibrosis
ARCT-2138	LUNAR®-FLU	Arcturus Therapeutics, Inc. (San Diego, CA, USA) and CSL Behring (King of Prussia, PA, USA)		I	Influenza
ARCT-2301	Kostaive® Bivalent		mRNA	III	COVID-19
ARCT-2303	Kostaive® XBB.1.5		mRNA	III	COVID-19
ARCT-2304	LUNAR®-FLU	Arcturus Therapeutics, Inc. (San Diego, CA, USA) and Biomedical Advanced Research and Development Authority (BARDA)	mRNA	Pre-clinical/I	Influenza
ARC-520		Arrowhead Pharmaceuticals	RNAi	Terminated, phase II	Chronic Hepatitis B

		(Pasadena, CA, USA) and ICON plc Clinical Research Organization (Dublin, Ireland)			
<i>Approved</i>					
ARCT-154	Kostaive® Monovalent: Ancestral	Arcturus Therapeutics, Inc. (San Diego, CA, USA) and CSL Behring (King of Prussia, PA, USA)	August 5, 2024 Approved (JP) MAA Filed (EU)	self-amplifying mRNA	COVID-19
	SPIKEVAX®	Moderna, Inc. (Cambridge, MA, USA)	January 31, 2022	mRNA	
	COMIRNATY®	Pfizer Inc. (New York, NY, USA)	August 23, 2021	mRNA	
	COMIRNATY® BNT162b2 bivalent (BA.4/BA.5)	and BioNTech (Mainz, Germany)	Product Enhancement	mRNA	
	ABRYSVO®			mRNA	Respiratory Syncytial Virus
Patisiran	ONPATTRO®	Alnylam Pharmaceuticals, Inc. and Sanofi Genzyme (Cambridge, MA, USA)	August 10, 2018	siRNA	Hereditary Transthyretin-Mediated Amyloidosis
Givosiran, ALN-AS1	GIVLAARI™	Alnylam Pharmaceuticals, Inc. (Cambridge, MA, USA)	November, 2019	siRNA	Acute Hepatic Porphyria