lipid nanoparticles

	NCT Number	Title	Authors	Description	Identifier	Dates
1	pubmed:36064263	Present and future of lipid nanoparticle-mRNA technology in phenylketonuria disease treatment	Ramon Diaz-Trelles Carlos G Perez-Garcia	Phenylketonuria (PKU) is a metabolic rare disease characterized by a failure of the body to clear out the high levels of Phenylalanine (Phe), leading to devastating neurological defects and growth retardation. PKU was discovered in 1934 by AsbjrØrn FØlling, and even though there have been continuous efforts from the scientific community to find therapeutic approaches to modulate the high levels of phenylalanine found in the body of the PKU patients, an efficient therapy still needs to be...	pmid:36064263 doi:10.1016/bs.ircmb.2022.04.008	Mon, 05 Sep 2022 06:00:00 -0400
2	pubmed:36064265	mRNA delivery technologies: Toward clinical translation	Itziar Gómez-Aguado Julen Rodríguez-Castejón Marina Beraza-Millor Alicia Rodríguez-Gascón Ana Del Pozo-Rodríguez María Ángeles Solinís	Messenger RNA (mRNA)-therapies have recently taken a huge step toward clinic thanks to the first mRNA-based medicinal products marketed. mRNA features for clinical purposes are improved by chemical modifications, but the inclusion in a delivery system is a regular requirement. mRNA nanomedicines must be designed for the specific therapeutic purpose, protecting the nucleic acid and facilitating the overcoming of biological barriers. Polymers, polypeptides, and cationic lipids are the main used...	pmid:36064265 doi:10.1016/bs.ircmb.2022.04.010	Mon, 05 Sep 2022 06:00:00 -0400
3	pubmed:36064267	Messenger RNA as a personalized therapy: The moment of truth for rare metabolic diseases	Karol M Córdoba Daniel Jericó Ana Sampedro Lei Jiang María J Iraburu Paolo G V Martini Pedro Berraondo Matías A Avila Antonio Fontanellas	Inborn errors of metabolism (IEM) encompass a group of monogenic diseases affecting both pediatric and adult populations and currently lack effective treatments. Some IEM such as familial hypercholesterolemia or X-linked protoporphyria are caused by gain of function mutations, while others are characterized by an impaired protein function, causing a metabolic pathway blockage. Pathophysiology classification includes intoxication, storage and energy-related metabolic disorders. Factors specific...	pmid:36064267 doi:10.1016/bs.ircmb.2022.03.005	Mon, 05 Sep 2022 06:00:00 -0400