

Gene Therapy Educational Resources List

Organisation	Name of resource	Type of resource	Description	Link	For healthcare professionals or patient community	DMD or non-DMD specific
Parent Project Muscular Dystrophy (PPMD)	Webinar Q&A: Gene therapy - turning obstacles into possibilities	Webinar Q&A and FAQ	Webinar to discuss the lessons learned in Gene Therapy, current challenges for our community, and what the future holds for these promising treatments. FAQ section.	Link	Both	DMD
	PPMD 2020 Annual conference - Gene therapy	YouTube video	Features panelists from Sarepta, Pfizer, Astellas Gene Therapies, Solid Biosciences	Link	Patients	DMD
	PPMD 2019 Conference - Gene Therapy: What We Know Today & Will Need to Know Tomorrow	YouTube video	Gene Therapy: What We Know Today & Will Need to Know Tomorrow	Link	Patients	DMD
Sarepta Therapeutics	Gene therapy and Duchenne	Guide	An overview of micro-dystrophin gene therapy research for DMD	Link	Patients	DMD
NA-ATTC	Advanced therapy education webinar series	Webinar series	This series of webinars will cover many aspects of cell and gene therapies, from the basics providing an introduction to how this new technology can modify diseases though to a more in-depth look into operational aspects of delivering advanced therapies within the hospital environment.	Link	Healthcare professionals	non-DMD
	Advanced Therapies NHS Readiness Toolkit	Toolkit	The NHS readiness toolkit provides resources for healthcare organisations that are working towards the delivery of advanced therapies for patients. The toolkit is intended for	Link	Healthcare professionals	non-DMD



			senior hospital management, operational managers, clinicians, pharmacists, nurses, laboratory teams and others involved in the delivery of advanced therapies.			
	Advanced Therapies Clinical Trial Toolbox	Toolbox	includes organograms, costing guidelines, local regulatory approval documentation and a map of clinical referral and treatment networks for exemplar products	Link	Healthcare professionals	non-DMD
The Association of the British Pharmaceutical Industry (ABPI)	ATMP roadmap	Roadmap	This Roadmap Tool sets out the key steps and activities in the end-to-end pathway for Advanced Therapy Medicinal Products (ATMPs) in England from non-clinical research through to patient treatment.	Link	Healthcare professionals	non-DMD
NIHR	Future of Health: Focus on Cell and Gene Therapy	Training video	The Future of Health: Focus on Cell and Gene Therapy' explores some of the key questions associated with 'advanced therapies' (often referred to as Cell & Gene Therapy or ATMPs). This course is designed to equip the research delivery workforce with a better understanding of these innovations. This module has been developed by the National Institute for Health Research with support from Cell and Gene Therapy Catapult UK	Link	Healthcare professionals	non-DMD
Pfizer	Gene therapy FAQ	FAQ	FAQ for gene therapy aimed at families	Link	Patients	non-DMD
SOLID Biosciences	Gene therapy FAQ	FAQ	FAQ for gene therapy aimed at families	Link	Patients	non-DMD
American Society of Gene & Cell Therapy	Gene Therapy webinar series	Webinar series	ASGCT and the National Organization for Rare Disorders (NORD) have partnered to produce a series of five patient education webinars.	Link	Patients	non-DMD



Muscular Dystrophy Association (MDA)	Clinical Implementation of Gene-Targeted Therapy Including Gene Replacement Therapy for Neuromuscular Diseases	Training video	There are a growing number of treatments that target the underlying genetic cause of neuromuscular diseases. This webinar will examine two approaches, exon skipping and gene replacement using viral vectors, being used to treat spinal muscular atrophy and Duchenne muscular dystrophy. Topics addressed include mechanism of action, technical issues, practical considerations, and future directions.	Link	Healthcare professionals	non-DMD
World Duchenne Organisation	Gene Therapy library	Library	Library of gene therapy papers	Link	Both	DMD
Duchenne UK	Gene therapy and the immunity challenges in gene therapy	YouTube video	To understand more about gene therapy and the related immunity challenges, please watch this short film.	Link	Patients	DMD
	Duchenne UK research	Information on website	Information about gene therapy, gene editing and exon skipping in DMD	Link	Both	DMD