

CF genetic therapy and ATMP trials

Training and information resources for research and
healthcare professionals

Training resources

Source	Title and Link	Description	Published	Resource type
Cystic Fibrosis Foundation (CFF)	1 hour webinar, Genetic Therapies 101	Genetic therapies hold the potential to treat the underlying cause of the disease for everyone with cystic fibrosis, regardless of mutation. In this webinar an overview of RNA and gene therapy is given, starting from the basics.	Recorded - December 14 2021	Video (1 hour)
Cystic Fibrosis Foundation (CFF)	CFF Research update: Genetic Therapies for CF	This webinar shares an overview of genetic therapies, starting from the basics: How do genetic therapies work in the body? How are they different from existing therapies? What kind of benefits could they provide?	Recorded - March 20 2025	Video (1 hour)
FDA	Basic overview of gene therapy	FDA website that provides a basic overview of gene therapy. Includes a short video with a very basic overview of gene therapy .	Content current from July 28 2022	Article (10 minute read) with video (1.5 minutes)
Advanced Therapy Treatment Centres	Education and training resources	Includes a broad array of training resources to learn more about ATMPs. Topics include: <ul style="list-style-type: none"> • Advance therapy education needs training package • UK training landscape, advanced therapies in the NHS • Introduction to advanced therapy medicinal products • In vivo gene therapies • The logistics of ATMPs in hospitals • Patient and public perspectives on cell and gene therapies • Gene therapy for respiratory diseases • Governance & operational considerations for delivering ATMPs in hospitals 	Links from October 2020 and February 2023	List of external links, PDF documents, webinars and eLearning modules

<p>Health Education England - eLearning for Healthcare</p>	<p><u>Advanced Therapy Medicinal Products Programme</u></p>	<p>An eLearning programme developed by the Advanced Therapy Treatment Centre (ATTC) network, London Advanced Therapies, the Cell and Gene Therapy Catapult (CGTC) in partnership with Health Education England.</p> <p>The sessions cover the following topics (feel free to mix and match depending on your individual learning needs):</p> <ul style="list-style-type: none"> • Introduction to advanced therapy medicinal products • In vivo gene therapies • Immune effector cell therapy • Focus on CAR-T cell therapy • The logistics of ATMPs in hospitals • Safe use of low temperature transport vessels 	<p>eLearning for healthcare 2025</p>	<p>eLearning programme, each session lasts around 30 minutes</p>
<p>Health Education England</p>	<p>Introduction to Advanced Therapy Medicinal Products (ATMPs) <u>Part 1</u> <u>Part 2</u> <u>Part 3</u></p>	<p>Three introductory videos on Advanced Therapy Medicinal Products which are recommended to be watched alongside completion of the ATMPs eLearning programme (above).</p> <p>These videos may be especially useful for information from a pharmacy perspective and provide a basic overview of different types of cell and gene therapies which may be of interest to other healthcare professionals or those involved in operational delivery of ATMP treatments (e.g. clinical trials).</p>	<p>October 10 2022</p>	<p>Videos (5-10 minsutes each)</p>
<p>Advanced Therapy Treatment Centres</p>	<p><u>Advanced Therapy Education Series Webinar – gene editing_</u></p>	<p>This webinar will present a basic introduction to gene editing and highlight the differences between gene editing and gene therapy. It will provide an overview of the current clinical landscape and examine some of the available safety data.</p>	<p>February 9 2021</p>	<p>Powerpoint presentation</p>
<p>Advanced Therapy Treatment Centres</p>	<p><u>Advanced Therapy Education Series Webinar – gene therapy for respiratory diseases</u></p>	<p>The seminar summarises the progress of the UK Respiratory Gene Therapy Consortium’s translational research programme primarily focused on developing clinically relevant gene therapy for cystic fibrosis and including development of novel non-viral and viral vectors.</p>	<p>January 28 2021</p>	<p>Powerpoint presentation</p>

Cystic Fibrosis Foundation (CFF)	Research into Genetic Therapies landing page	<p>An online resource which provides information on the following areas:</p> <ul style="list-style-type: none"> • Gene Delivery for CF • Gene Therapy for CF • Gene Editing for CF • Stem cells 		<p>Series of short articles (between 3 and 7 minutes read time each)</p>
Cystic Fibrosis Foundation (CFF)	CFF Research Con 2025: Gene-based therapy in action: Behind the scenes look at genetic therapy trials	<p>Genetic therapies are a critical next step in finding a cure for everyone with cystic fibrosis. This session followed the journey through a gene therapy-based clinical trial from the lens of an adult with CF, a research coordinator, and principal investigator. Learn about how gene-based therapies work, finding a trial, safety considerations, what participation looks like, as well as challenges and reflections along the way.</p>	<p>Recorded - May 6 2025</p>	<p>Video (1 hour)</p>
Cystic Fibrosis Foundation (CFF)	NACFC 2024 Plenary 1: Challenges & Progress in the Pursuit of Genetic Therapies for CF	<p>During plenary 1 of the North American Cystic Fibrosis Conference, the speakers explore different approaches to overcoming the biological obstacles and unique clinical trial challenges for developing cystic fibrosis genetic therapies.</p>	<p>Recorded - September 26 2024</p>	<p>Video (1 hour and 25 minutes)</p>
Cystic Fibrosis Foundation (CFF)	NACFC 2023 Plenary 1: Genetic Therapies for All: Harnessing Cross-Disease Knowledge for Breakthroughs in CF	<p>During plenary 1 of our North American Cystic Fibrosis Conference, the speakers share how we can gain valuable insights from the development of genetic therapies for other diseases and apply these approaches to cystic fibrosis.</p>	<p>Recorded - November 2 2023</p>	<p>Video (1 hour and 25 minutes)</p>

Public Policy Projects	<u>Advanced treatments –gene and cell therapy.</u>	A white paper which brings together representatives from across the cell and gene ecosystem with a focus on the patient perspective, economics, and what gene and cell therapy look like in a modern age	2023	Report (34 pages; long read)
Cystic Fibrosis Trust	<u>Research in Focus report on genetic therapies</u>	The report gives an overview of why we need genetic therapies for CF, how they may work and progress made so far	December 2020	Report (20 minute read)
Association of the British Pharmaceutical Industry (ABPI)	<u>ATMP Roadmap</u>	The 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 treatment provision and monitoring	November 2021	Roadmap (interactive tool)
Advanced Therapy Treatment Centres	<u>National Cell & Gene Vision for the UK</u>	This document has been developed to outline the recommended content that a national cell and gene therapy vision should incorporate to help ensure the UK remains a world leader in the provision of cell and gene therapies.	March 2022	Report (36 pages; long read)
Advanced Therapy Treatment Centres	<u>Advanced Therapies NHS Readiness Toolkit</u>	Provides resources for healthcare organisations that are working towards the delivery of advanced therapies for patients		Interactive toolkit with resource links

Resources in other languages

Source	Title and Link	Description	Published	Resource type
Nerdelandse Cystic Fibrosis Stichting (NCFS)	Getherapie: Een Nieuuwe Aanpak voor cystic fibrosis	<p>Dutch - Onze animatie legt eenvoudig uit hoe drie verschillende vormen van getherapie werken en wat dit in de toekomst kan betekenen voor de behandeling van CF.</p> <p>Gene therapy may be the next step in the treatment of CF, but how does it actually work? Our animation simply explains how three different types of gene therapy work and what this could mean for the treatment of CF in the future.</p>	March 26 2025	Video (2 minutes)
VAINCRE LA MUCOVISCIDOSE	Thérapie génique et thérapie cellulaire	<p>French</p> <p>Read this short article on how Genetic Therapies can be used in Cystic Fibrosis, how they work and where you can find a clinical trial to take part.</p>		Article (5 minute read)
Cystic Fibrosis Foundation (CFF)	CFF Research Con 2025: Terapia Genética en acción	<p>Spanish - Las terapias genéticas son un próximo paso fundamental para encontrar una cura para todas las personas con fibrosis quística. Obten información sobre cómo funcionan las terapias basadas en genes, cómo encontrar un ensayo, las consideraciones de seguridad, cómo es la participación, así como los desafíos y las reflexiones a lo largo del camino.</p> <p>Video on how genetic therapies are the next step to finding a cure for everyone with cystic fibrosis and informing people what it means to and how to take part.</p>	Recorded - May 6 2025	Video (1 hour)