

CF genetic therapy and ATMP trials

Training and information resources for research and
healthcare professionals

Training resources

| Source | Title | Description | Access | 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. | https://www.youtube.com/watch?v=C6PyZNpXAhQ&ab_channel=CysticFibrosisFoundation | 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 . | https://www.fda.gov/consumers/consumer-updates/what-gene-therapy-how-does-it-work | Article (10 minute read) with video (1.5 minutes) |
| Advanced Therapy Treatment Centres | Education and training resources | This section includes a broad array of training resources that enable staff to learn more about ATMPs. Topics include: <ul style="list-style-type: none"> • Advance therapy education needs training package • The UK training landscape in 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 | https://www.theattcnetwork.co.uk/toolkit_section/education-and-training-resources | List of external links, PDF documents, webinars and eLearning modules |

| | | | | |
|---|--|---|--|--|
| <p>Health Education England - eLearning for Healthcare</p> | <p>Advanced Therapy Medicinal Products Programme</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>https://www.e-lfh.org.uk/programmes/advanced-therapy-medicinal-products/</p> | <p>eLearning programme, each session lasts around 30 minutes</p> |
| <p>Health Education England</p> | <p>Introduction to Advanced Therapy Medicinal Products (ATMPs)</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>https://youtu.be/4f-yqtCfMH4</p> <p>https://youtu.be/Sm27qpLqFfQ</p> <p>https://youtu.be/RgvFvgCRh10</p> | <p>Videos (5-10 minutes each)</p> |
| <p>Advanced Therapy Treatment Centres</p> | <p>Advanced Therapy Education Series Webinar – gene editing</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>https://event.on24.com/wcc/r/2973921/86F25DDF8CB6E7D6FB771208C089B512</p> | <p>Webinar (39 minutes)</p> |
| <p>Advanced Therapy Treatment Centres</p> | <p>Advanced Therapy Education Series Webinar – gene therapy for respiratory diseases</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>https://event.on24.com/wcc/r/2973904/D5E203A48AE3120A09637F9C70B27C03</p> | <p>Webinar (46 minutes)</p> |

Information resources

| Source | Title | Description | Access | Resource type |
|---|---|--|---|---|
| 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 | https://www.cff.org/research-clinical-trials/research-genetic-therapies | Series of short articles (between 3 and 7 minutes read time each) |
| Cystic Fibrosis Foundation (CFF) | NACFC 2021 Plenary 2: Working Together to Pave the Path to a Cure | Recorded talk on the success of the CF Foundation's Path to a Cure initiative with emphasis placed on gene editing, gene therapy (replacement) and genetic therapy delivery. | https://www.youtube.com/watch?v=nZi55Blisy0&list=PLhoQ6vyZhggqoCiN1AQFnYi1KhpJcydljR&index=2 | Video (1 hour) |
| Cystic Fibrosis Foundation (CFF) | NACFC 2022 Plenary 1: Hope for All: Addressing the Needs of Those With Untreated CF Mutations | Recorded talk giving an overview of some of the more rare CF mutations and the different approaches to develop treatments for people with those mutations. Includes a talk from 1:10:26 to 1:17:29 around how to explain CF genetics / genetic therapy treatments in ways that are accessible and easy to understand | https://www.youtube.com/watch?v=k-H_hqkhu24 | Video (1 hour 20 minutes) |
| Public Policy Projects | Advanced treatments –gene and cell therapy | A white paper which brings together representatives from across the cell and gene ecosystem with a focus on increasing awareness of cell and gene therapy in education and early careers. The report emphasizes the importance of cross sector collaboration in order to advance cell and gene therapies. | https://publicpolicyprojects.com/wp-content/uploads/2023/09/Cell-Gene-Report.pdf | Report (28 pages; long read) |

| Source | Title | Description | Access | Resource type |
|--|---|--|---|---|
| Cystic Fibrosis Trust | Research in Focus report on genetic therapies | The report gives an overview of why we need genetic therapies for CF, how they may work and progress made so far | RES24C Research in focus_Gene editing_Dec 2020.pdf (cysticfibrosis.org.uk) | Report (20 minute read) |
| Association of the British Pharmaceutical Industry (ABPI) | ATMP Roadmap | 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 | https://www.abpi.org.uk/media/o1npmwck/atmp-roadmap-tool_final_03122021-fullscreen.pdf | Roadmap (interactive tool) |
| Advanced Therapy Treatment Centres | National Cell & Gene Vision for the UK | 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. | National Cell and Gene Therapy Vision for the UK | Report (36 pages; long read) |
| Advanced Therapy Treatment Centres | Advanced Therapies NHS Readiness Toolkit | Provides resources for healthcare organisations that are working towards the delivery of advanced therapies for patients | Advanced Therapies NHS Readiness Toolkit • ATTC Network - Advanced Therapy Treatment Centre (theattcnetwork.co.uk) | Interactive toolkit with resource links |
| Advanced Therapy Treatment Centres | Advanced therapy education webinar series | Link to the full page of all advanced therapy webinars (free to access) covering cell and gene therapy topics, from the basics about how they work to more operational aspects of delivering advanced therapies and clinical trials. The webinars are targeted at healthcare professionals, or anyone involved in cell and gene therapies. | https://www.theattcnetwork.co.uk/network/advanced-therapy-education-webinar-series | Webpage with a list of free webinars |