



# Health and Care Research Wales

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# Overview of Research Delivery Hub Services

## Setting up a study in Wales

01

Provide an overview  
of Health and Care  
Research Wales  
Research Delivery Hub  
services

02

Highlight some of  
our models of working

03

Provide some real  
examples of Studies  
which have  
been delivered in  
Wales using the  
different models

# Research Delivery Hub

Who we are  
and what we  
offer?

- ⌘ Commercial Site ID service
- ⌘ Horizon scanning – Research opportunities
- ⌘ Specialty Lead review and triage
- ⌘ Dedicated Heads of Research Delivery
- ⌘ Social Care, Primary Care and Secondary Care networks
- ⌘ Contracts – Review and advice service
- ⌘ Costings – ICT, AcoRD specialists, ETC budget management
- ⌘ Dedicated Research Managers and Coordinators
- ⌘ Booking and Support Team
- ⌘ Communications – National service and coordination
- ⌘ National RDOG and RMOG meetings

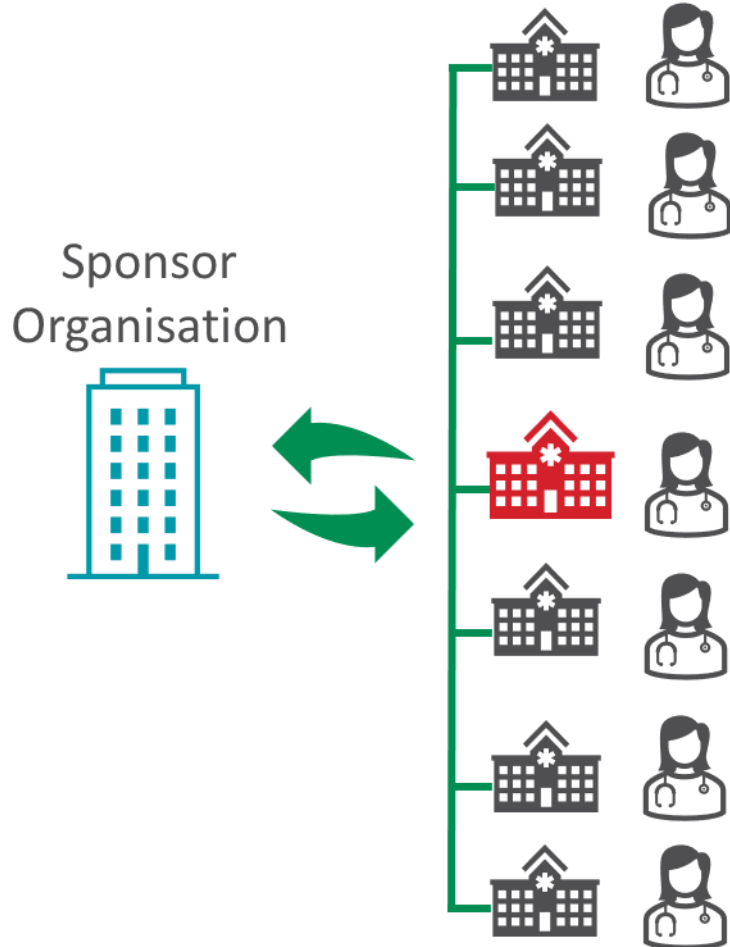
# Models of Working

Research Delivery hub

Once for Wales

One Site Wales

# Once for Wales



One costing and  
contract negotiation  
with the lead site

Agreements  
accepted by all  
participating  
organisations in  
Wales



# One Site Wales

Sponsor  
Organisation



One Wales  
Lead site & PI



One Site Wales is a research delivery model where multiple locations or Health Boards all contribute to a study as **one** national delivery team with **one** shared recruitment target.



# SYMPLIFY

Observational study to assess a multi-cancer early detection test in individuals referred with signs and symptoms of cancer

## Velindre NHS Trust

Sponsor Organisation



One Wales  
Lead site & PI

**Prof Dean Harris**  
**Prof Tom Crosby**

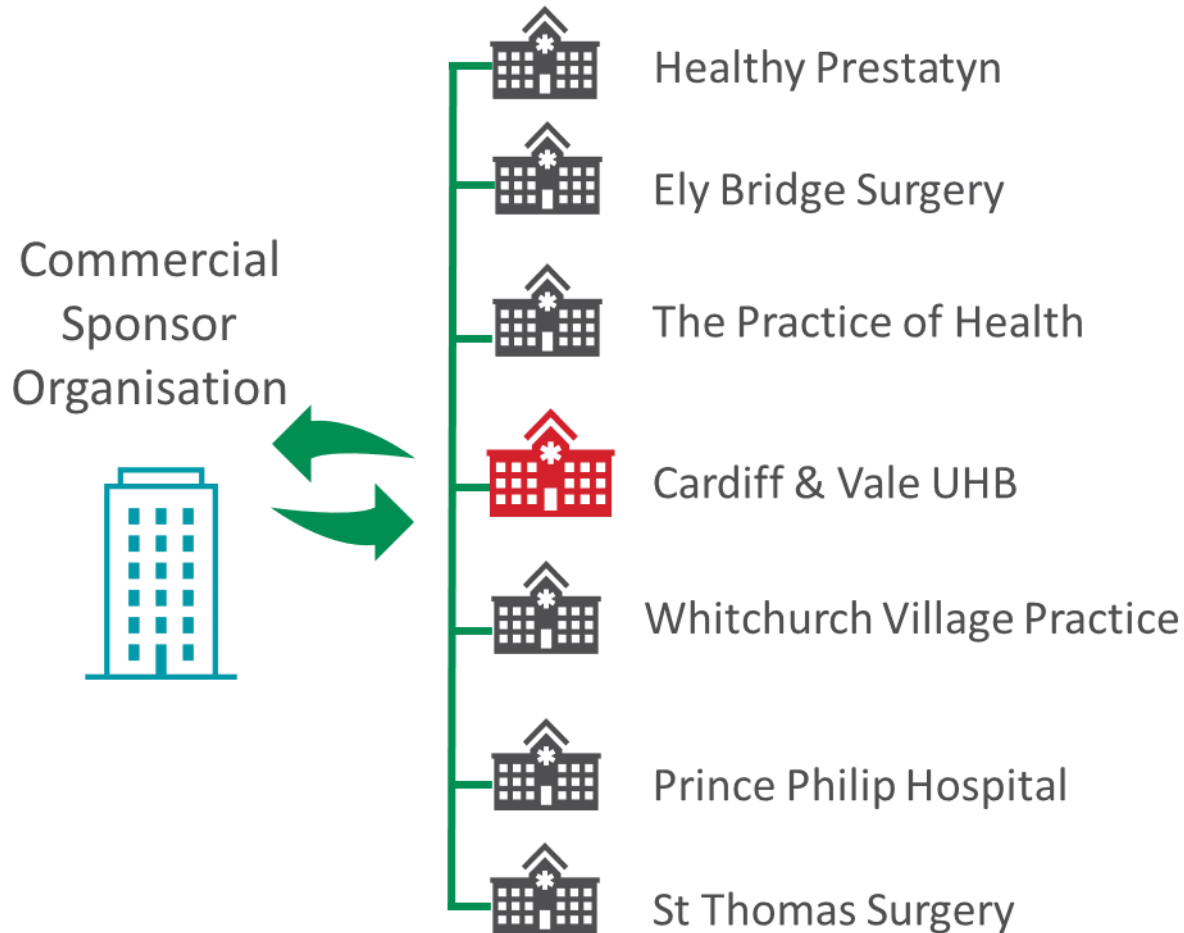
## Key outcomes

- ⌘ Rapid setup and delivery of the trial
- ⌘ Wales recruited 1161 against a target of 700  
Target achieved in 3 months
- ⌘ Delivery teams across 19 hospital sites contributed to the One Wales site

“Thank you for the terrific contribution from Wales to SYMPLIFY – with over 20% of total recruitment and comfortably exceeding your target it has been terrific.”

**Prof Mark Middleton**, SYMPLIFY Chief Investigator, University of Oxford

# Evaluate aspects of adherence to a single-inhaler triple therapy (SITT) as maintenance treatment of asthma



## Key outcomes

- ⌘ Networked approach
- ⌘ Real world obs study
- ⌘ Create commercial income
- ⌘ Flexible target
- ⌘ Over/under recruit
- ⌘ Shared review / contracts
- ⌘ Best practice and successful recruitment methods



# Industry collaboration at the NACHfW



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## **Rhian Thomas-Turner**

R&D Lead, Noah's Ark Children's Hospital for Wales

## **Dr Phil Connor**

Specialty Lead, Health and Care Research Wales  
R&D Lead, Children and Women Clinical Board,  
Cardiff and Vale UHB

# Cross sector working

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Industry requirement under the  
Paediatric Regulation

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Multi-stakeholder collaboration

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EFGCP/MRCT – collaborative  
projects

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# Cross-sector working

Academy of Medical Sciences

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Grants and Programmes > Programmes > Cross-Sector Programme - network, connect, collaborate

## Cross-Sector Programme - network, connect, collaborate

The Academy of Medical Sciences Cross-Sector Programme will bring together clinicians, regulators, policymakers and researchers from different sectors to promote health innovation.

**Launch event** took place on 20 April 2022 in Cardiff, Wales.



Health professionals, regulators, policymakers and patients and the public need to work together to solve the major challenges facing healthcare. Our research shows there are many ways to do this.

[Key contacts](#)

aparito

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## Cross-sector working to improve paediatric clinical trials

12/10/2022

Aparito were fortunate to have the brilliant Rhian Thomas-Turner join us on a secondment in the capacity of Head of Academic Partnerships.

Impact Factor: **2.599** / 5-Year Impact Factor: **2.989**

[JOURNAL HOMEPAGE](#)

12 November 5, 2022

### Medicines: Improving regulatory guidance

[Rhian Thomas-Turner](#) +7 [View all authors and affiliations](#)

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Share options



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Metrics and citations

Trials are completed, even when identical drug trials are completed. This has resulted in significant delays in the access to beneficial drugs. This study sought to address this by identifying common errors in regulatory guidance documents as they

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Disciplines  
Hubs**  
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# Sanofi

## Harmonie Study

- RSV study - condition has a big impact on children and the NHS every winter
- Antibody vaccine (Nirsevimab)
- Phase III study with the aim of reducing severe RSV/hospitalisation in infants
- Results showed that the vaccine reduced all hospitalisation by 83%
- Pump prime funding – up to £100,000 to spend to fund staff for the project



# Vertex

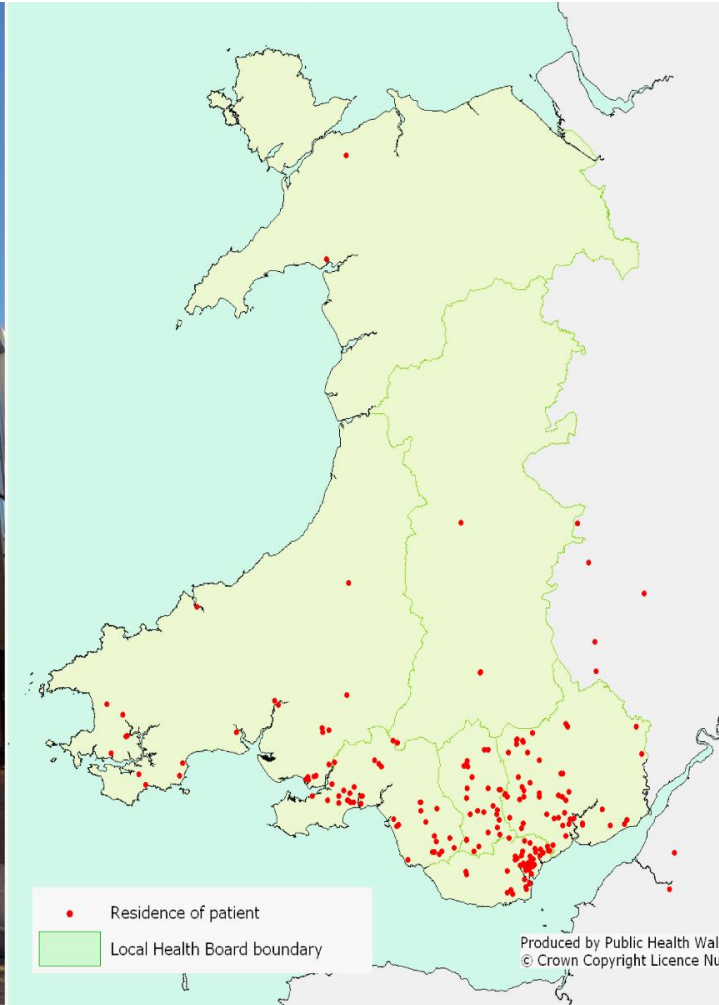
- First paediatric study was Galileo
- Phase III study – long term effects of Kaftrio in children with CF 6 years and over
- For our patient – reduced hospitalisation
- Two new studies offering access to younger age groups



# Astellas Pharma

- Children and adolescents - FLT3 positive /AML
- First global recruit to the study, two recruits to date
- Patient opportunity to access innovative therapy
- Cost saving to the NHS





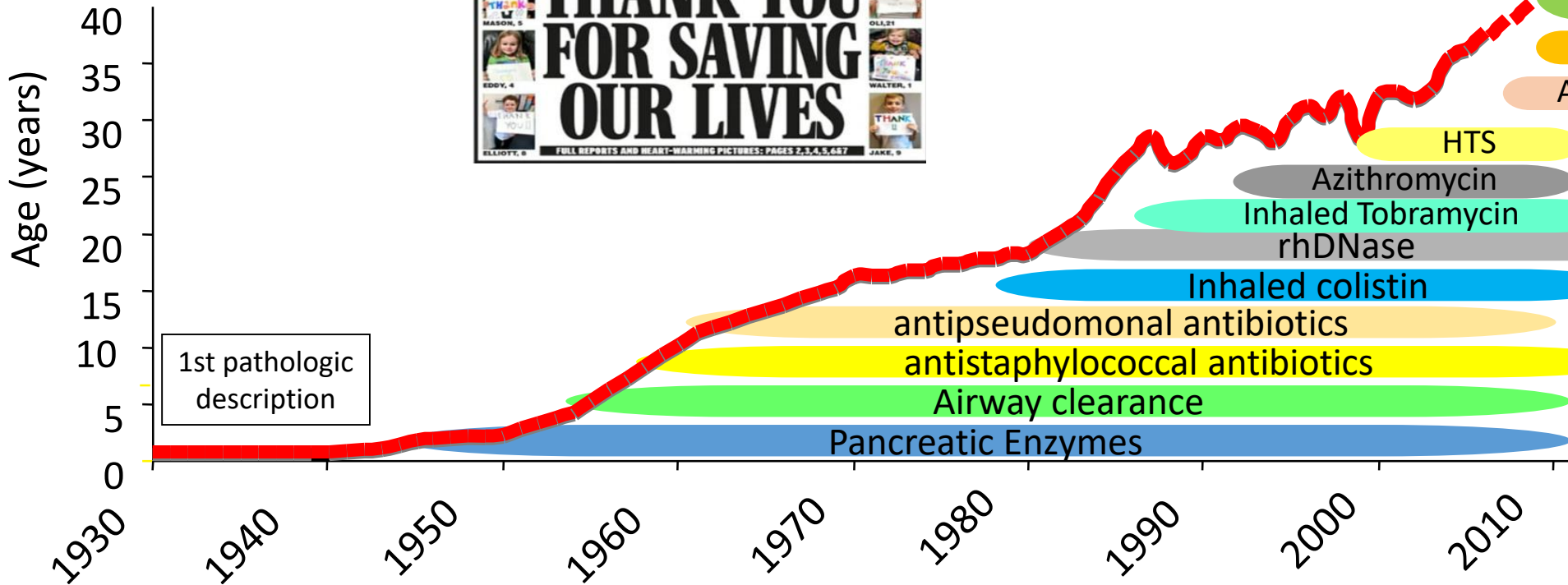
# MediWales

Jamie.duckers@wales.nhs.uk

 29 June 2023



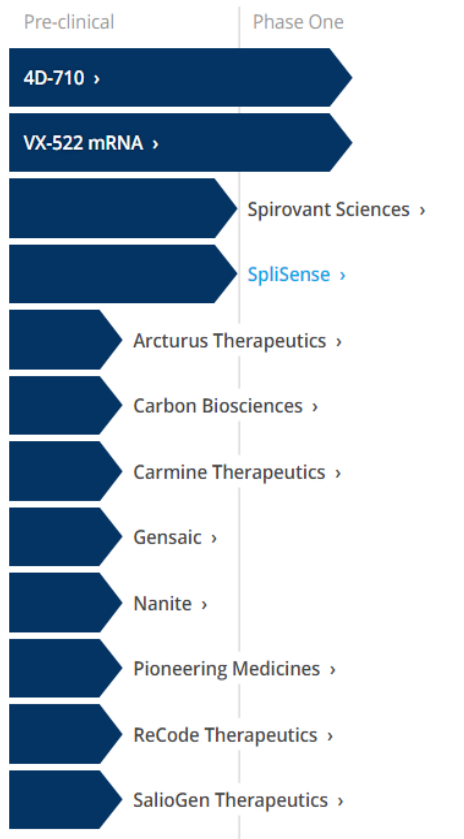
# The journey to Improved Survival (+life unlimited with CF)



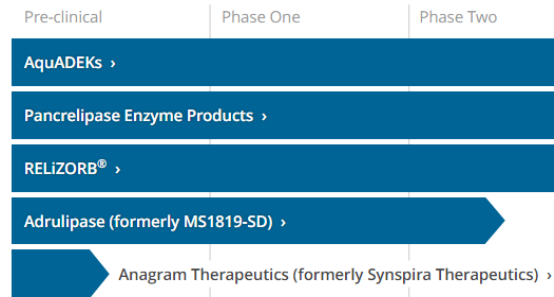


## Genetic Therapy [Learn more >](#)

Gene therapy, RNA therapy, and antisense oligonucleotide

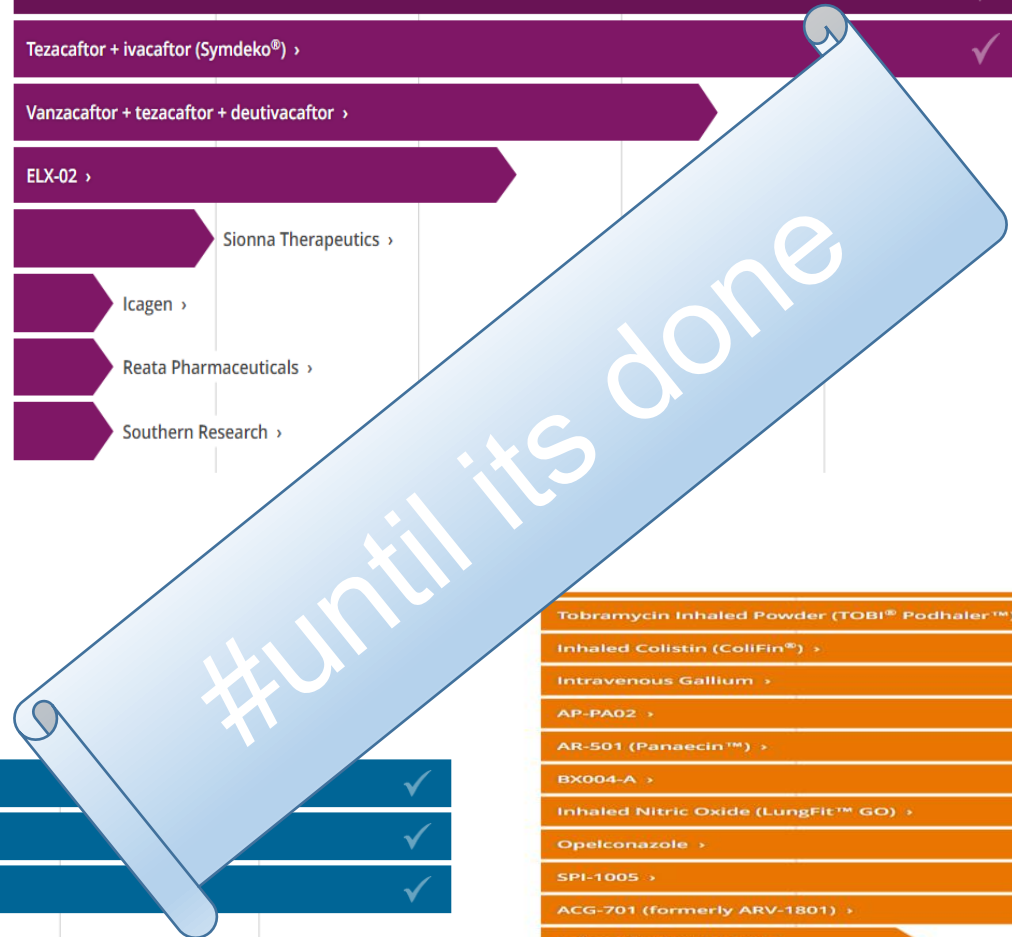
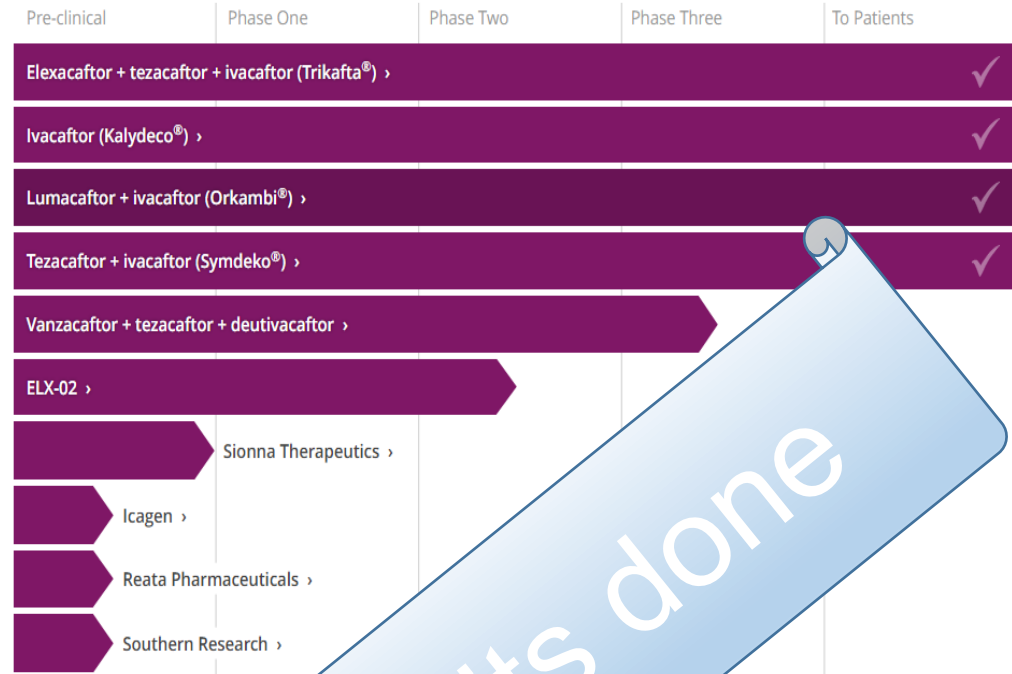


## Nutritional-GI [Learn more >](#)

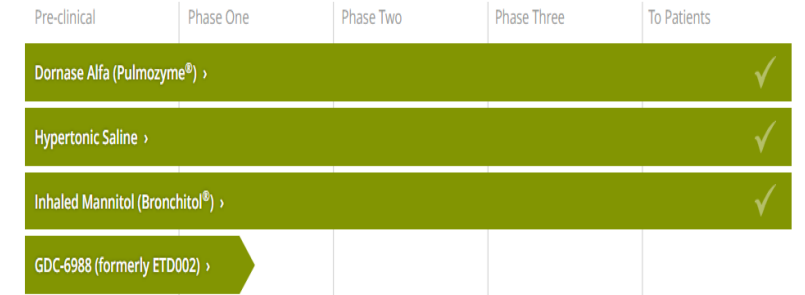


## Restore CFTR Protein [Learn more >](#)

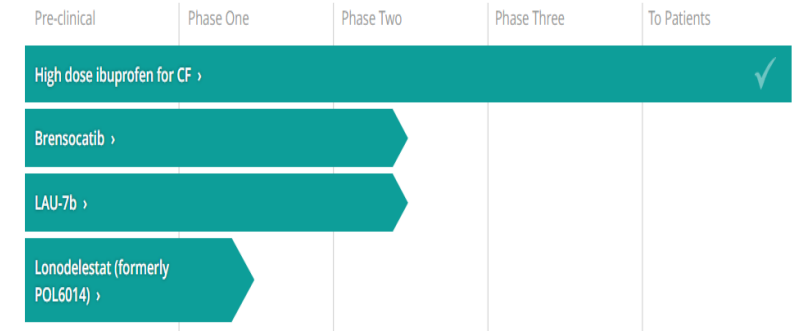
Modulators and nonsense readthrough therapies



## Mucociliary Clearance [Learn more >](#)



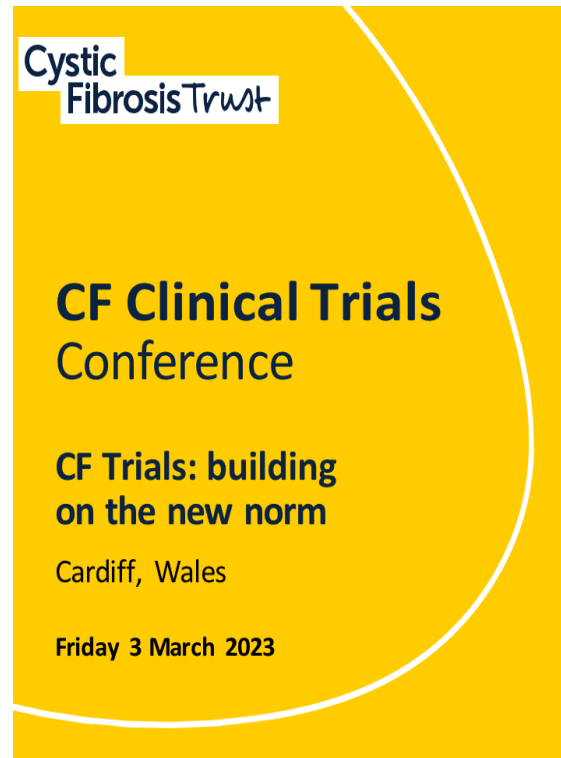
## Anti-Inflammatory [Learn more >](#)



# Progress in CF = success of research model



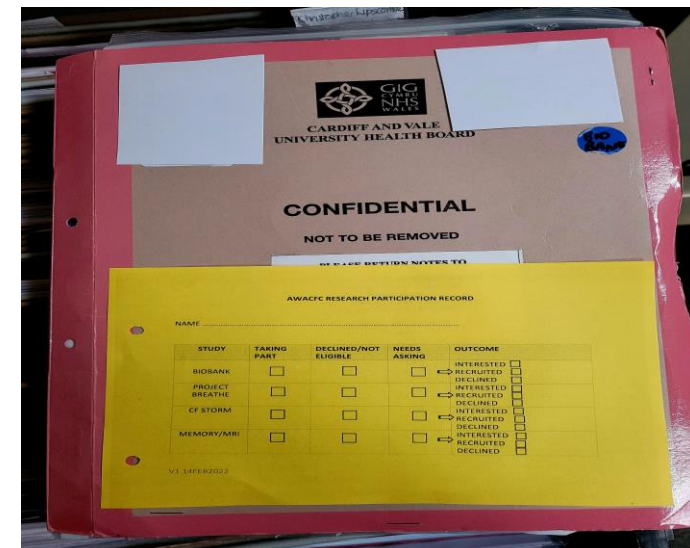
- Embedding research into clinical care
- Engagement with charities
- Coordinated approach-networks
- PPI PPI PPI
- Engagement with sponsors
- Power of registry data
- Power of biobanks



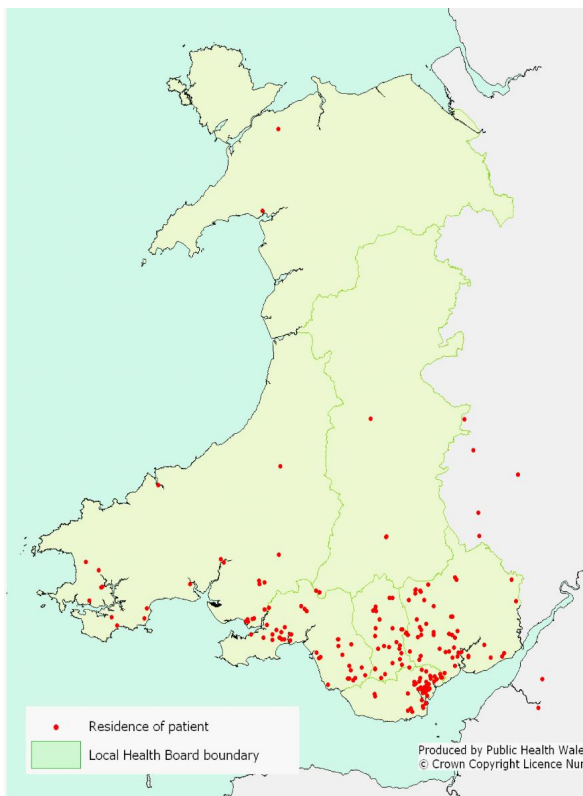
# Embedding research into clinical care



- Improved healthcare outcomes for those in research
- Benefits for centre and staff:
  - morale in team- R+D award
  - first into research grants
  - learning and sharing good practice
- MDT/ clinics/ regular meetings
- Website
- 10 completed commercial trials
- 4 current commercial/ 4 non commercial
- 3 commercial in set up including early phase MRNA and gene therapy on ward



# Networks: CF Clinical Trials Accelerator Platform / ECTN



## EQUITY OF ACCESS TO LATEST TRIALS

- 27 centres
- 6 Early Phase centres
- **89%** of UK CF population
- 25 CTAP Trial Coordinators

57 CF centres  
17 different countries  
21500 CF patients

# Patient & Public Involvement (PPI)



- PPI leads to **better recruitment and retention rates**
- Involvement activity **provides significant insight that could otherwise not have been accessed or considered** whereupon material changes are made to trial designs or other projects
- Mutually beneficial for investigators and the CF community: **the community feel valued and empowered**
- The **CF Trust Involvement Group** are an established group of CF representatives (>50 people with CF, and parents of children with CF) ready to share their lived experience to support Sponsors and researchers with study design and delivery
- In 2022, 100% of sponsors strongly agreed that involving the CF community improved their study

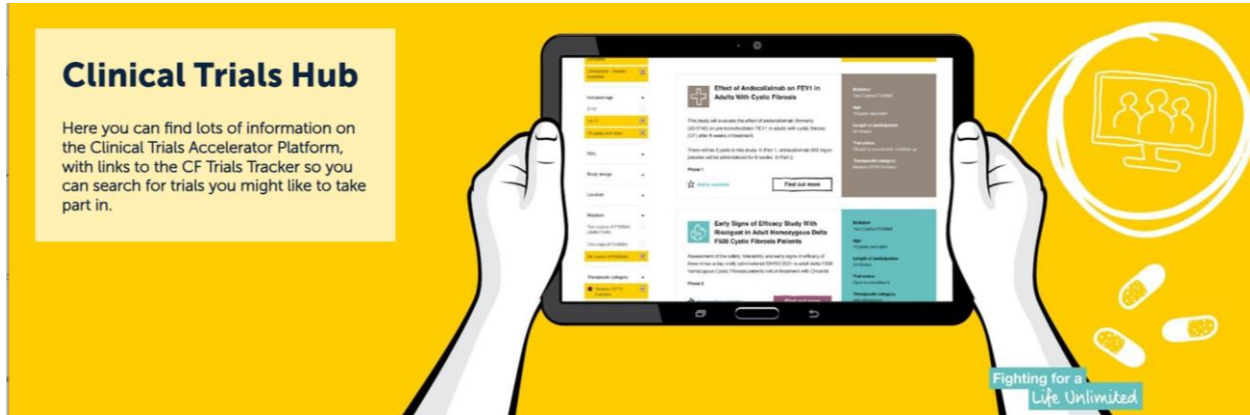


You are in: Home » The PSPs » Cystic Fibrosis

## Cystic Fibrosis



# CF Clinical Trials Digital Hub



## Find out more about CF genetic therapies

### CF Professionals training resources on genetic therapies

We've compiled a list of resources for CF professionals – or anyone – to learn more about genetic therapies and Advanced Therapeutic Medicinal Product (ATMP) trials. They range from a 10 minute read and short video clips to recordings of 1 hour webinars.

[Learn more about genetic therapies and ATMPs](#)

### FAQs on genetic therapy clinical trials

Read our Frequently Asked Questions on genetic therapy clinical trials, topics range from can I take part, to what will be involved.

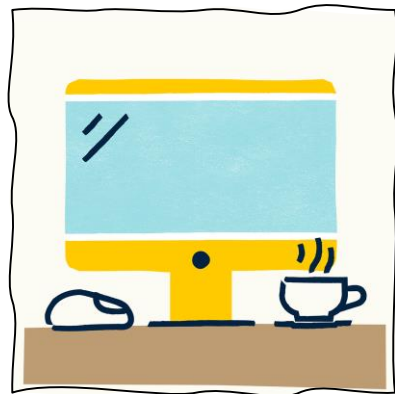
[Find out more](#)

### Research in focus on genetic therapies

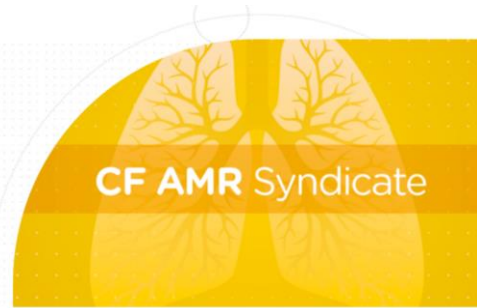
Our Research in focus report on genetic therapies contains an overview of why we need genetic therapies for CF, how they may work and progress made so far.

[Read the report](#)

- Introduction to clinical trials
- Case studies
- FAQs
- Trials Tracker database



# Supporting Sponsors: CTAP/ ECTN



Medicines Discovery Catapult and Cystic Fibrosis Trust join forces to drive drug-discovery and address urgent unmet need

Cystic Fibrosis Syndicate in Antimicrobial Resistance launches

## Target Product Profiles



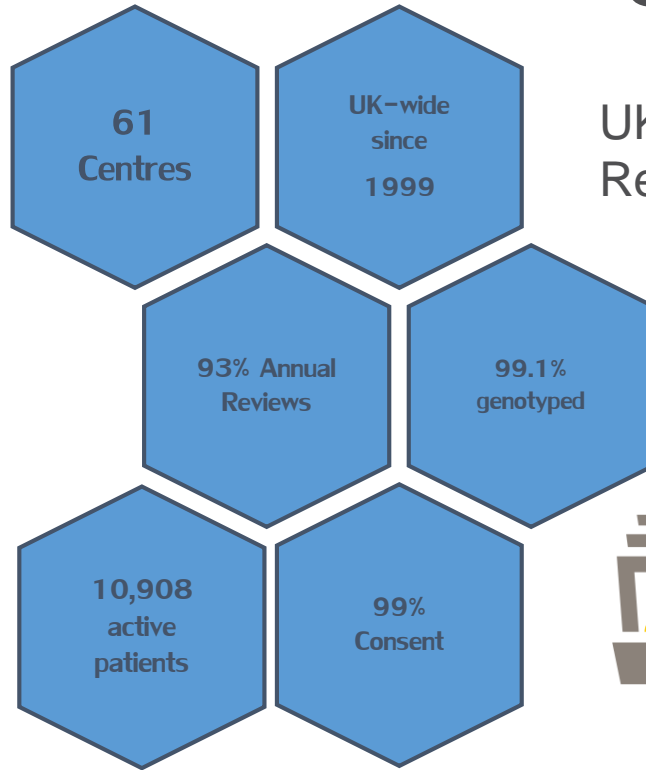
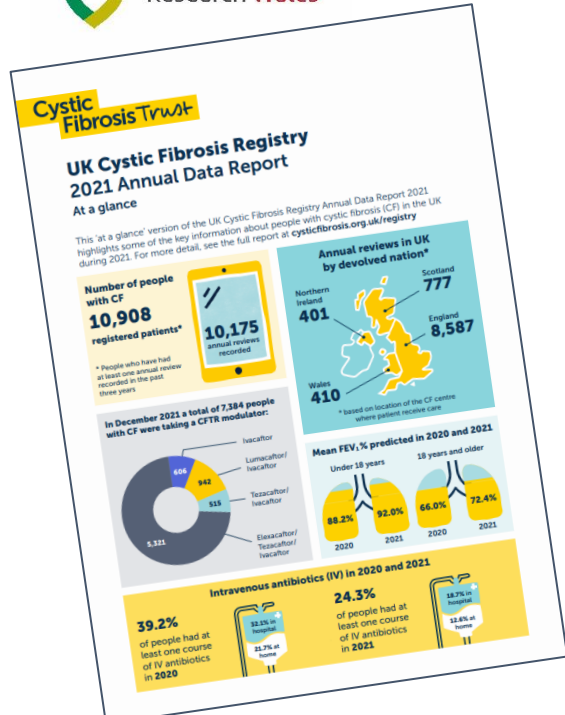
- Support identification of recruitment & referral centres
- Research oversight board and protocol review
- Centralised Feasibility Review – **national and centre specific**
- Aligned with ECFS-CTN
- UK Lead clinician and business development manager



The aim of ECFS-CTN is to intensify clinical research in the area of CF and to bring new medicines to people with CF as quickly as possible.

-  Increases cooperation between the whole CF community (people with CF, patient organisations, pharmaceutical industry and academic researchers)
-  Shares expertise across countries to standardise research procedures and measures
-  Accelerates study feasibility and setup
-  Encourages high quality research by training staff and monitoring site performance

# CF Registry Data



UK Cystic Fibrosis Registry:  
Real World Data



**Pharmacovigilance**  
Long term safety and effectiveness monitoring of new treatments

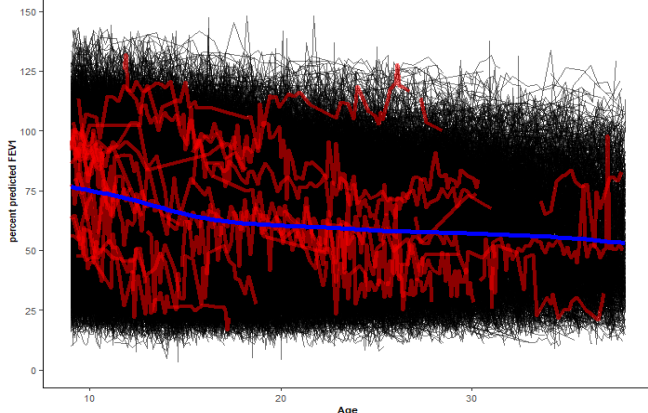


**Health Technology Assessments (HTA)**  
Medical, economic, social & ethical issues related to the use of a health technology (e.g., medicinal products)



**Pharmacoepidemiology**  
Utilisation and effects of drugs in large numbers of people.

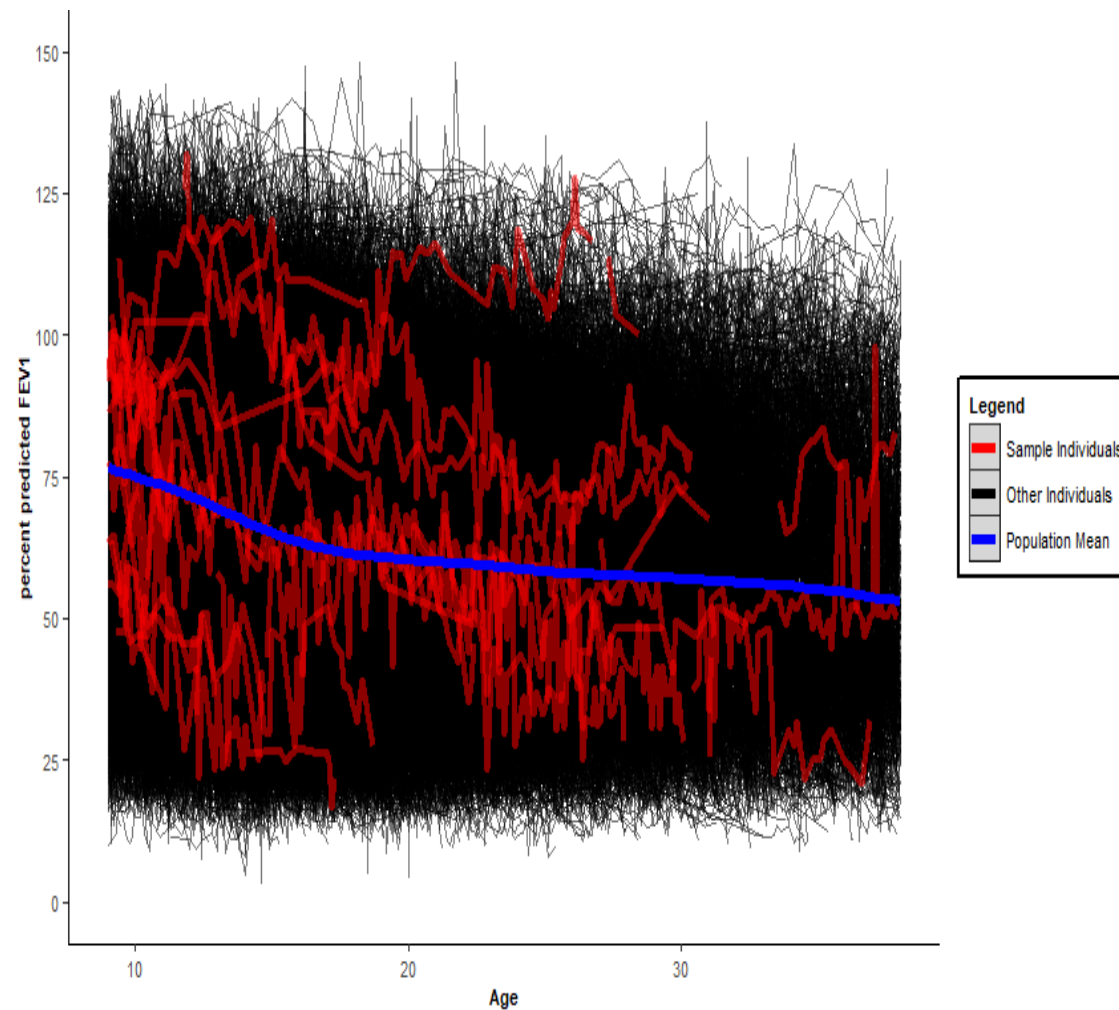
Figure 2a Spaghetti plots of %FEV1 trajectory of US Women aged 15-44 years, 2003-2017



**Legend**  
— Sample Individuals  
— Other Individuals  
— Population Mean



# Biobank







J. Ducken, K. Fitzgerald, D. Prasad et al.

Journal of Cystic Fibrosis 21 (2022) 551-552

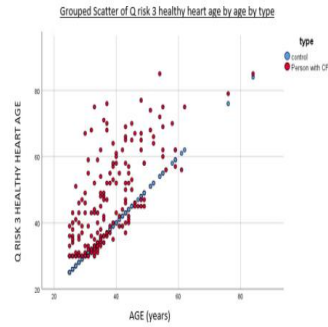
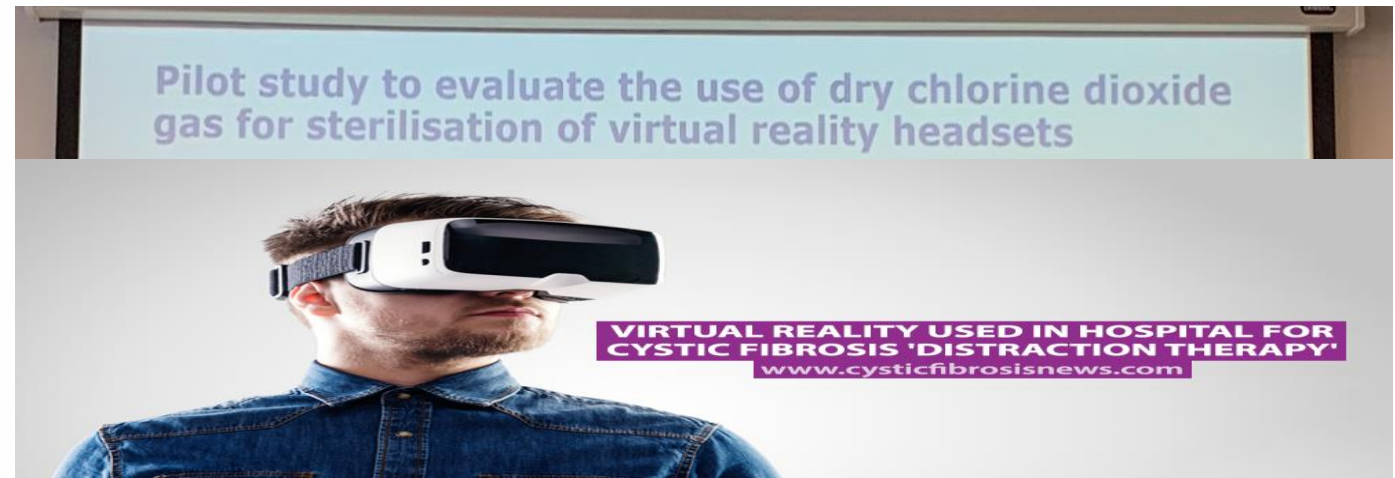
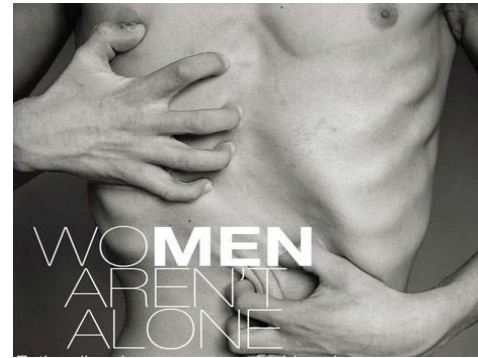
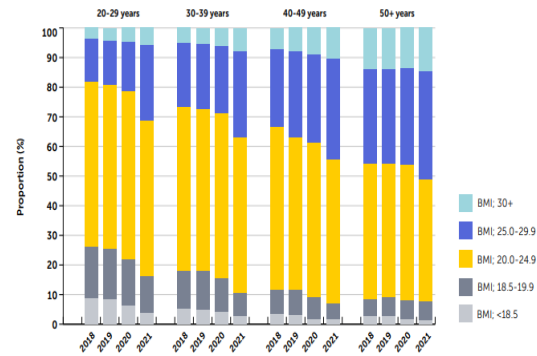
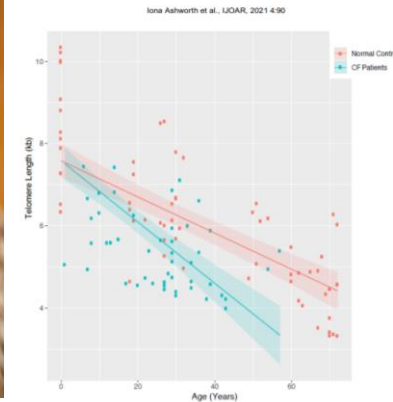


Fig. 1. A scattergram of the 207 patients at the 'all Wales adult CF centre' comparing their Q risk 3 healthy heart ages (y-axis) against their actual ages (x-axis).

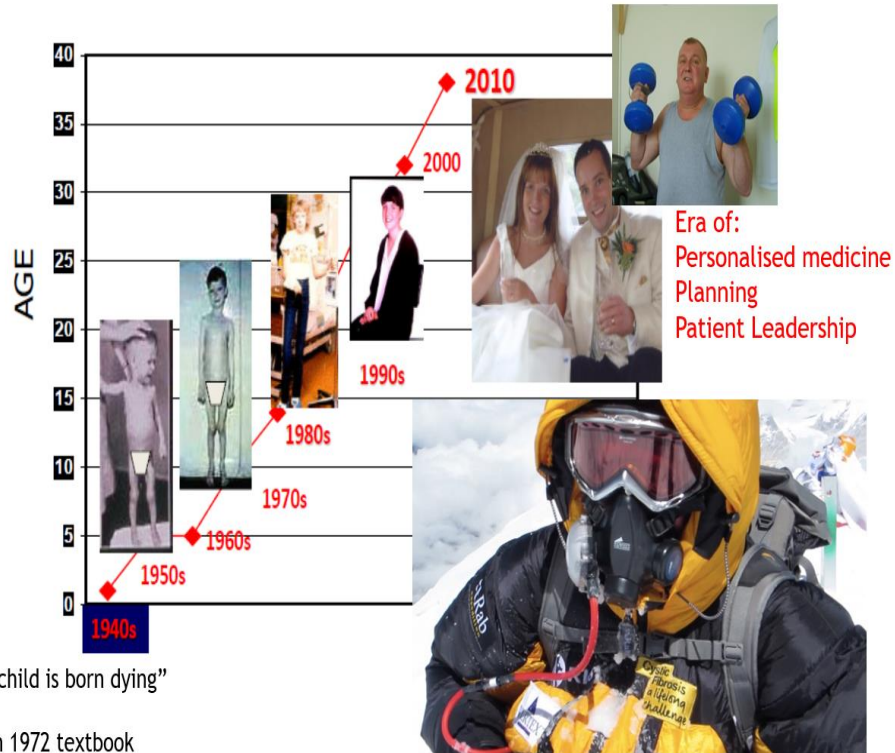


### 1.9 Body Mass Index (BMI) in adults for 2018 – 2021

The following graph shows the change in the proportion of people in each BMI group from 2018 to 2021.

# Where next...



- CF Pipeline incl early phase/ gene therapies
- Early links with sponsors, researchers and CF Community
- JLA priorities
- Networks
- PPI
- Power of registries and biobanks
- Repurposing of meds/ lessons for other respiratory conditions/ rare diseases

[Jamie.duckers@wales.nhs.uk](mailto:Jamie.duckers@wales.nhs.uk)

**Cystic  
Fibrosis Trust**



↑ / Get involved / Trials Tracker



GIG  
CYMRU  
NHS  
WALES

Bwrdd Iechyd Prifysgol  
Caerdydd a'r Fro  
Cardiff and Vale  
University Health Board



# Pharmacovigilance and working with Industry

- The UK CF Registry pharmacovigilance programme was initiated in 2012
- Completed 3 studies and 3 are ongoing
- The Registry has supported Post Authorisation Safety and Efficacy Studies (PASS and PAES)
- Long-term studies spanning several years of data collection – up to 9 years.



# Benefits of working with the UK CF Registry

- Long-term monitoring of outcomes in patients prescribed the drug
- Comparator groups of those not on drug can also be analysed
- Historical data can also be accessed – e.g. historical matched cohorts.
- Possible for us to add bespoke data collection variable eg. Specific outcome measures, start/stop dates



# Independence is paramount

- A UK lead investigator (a CF physician) will work collaboratively with the company, providing input to the study protocol, interpretation of results, and interim and final reports.
- We will provide summary data only No patient level data relating to individuals are provided to the pharmaceutical companies.
- UK CF Registry statisticians conduct the analysis.
- It also ensures the results of the analysis are agreed by independent experts.

Decisions on working with a company, and the development of agreements that safeguard the integrity of the Registry and the anonymity of the data, are made by the CF Registry Steering Committee





# Health and Care Research Wales

Research Delivery Hub

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