UK CF Registry

Data Request Form

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| **UK CF Registry data request form** |
| All requests for data or information from the UK CF Registry must be submitted on this form to [registry@cysticfibrosis.org.uk](mailto:registry@cysticfibrosis.org.uk) .  Before starting a request please make sure you have read the UK CF Registry Data Sharing Policy, available at [Cystic Fibrosis Trust UK CF Registry](https://www.cysticfibrosis.org.uk/sites/default/files/2023-08/UK%20CF%20Registry%20Data%20Sharing%20Policy_July%202023_V2.0.pdf) |

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|  | **I/We have read the UK CF Registry Data Sharing Policy** |

# Lead Applicant

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| **Full name** | **Job title** | **Institution** | **Email address** |
| DHIVYA G | Research Scholar | VIT | dhivya.g@vit.ac.in |

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| **Date of Request** |
| 02-05-2025 |

# Research team

*Provide details of everyone in the research team requesting data. Add more rows to the table if required.*

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| **Full name** | **Job title** | **Institution** | **Email address** |
| DHIVYA G | Research Scholar | VIT | dhivya.g@vit.ac.in |
| MANOOV R | Senior Asst. Professor | VIT | manoov.r@vit.ac.in |
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| **Title of project / research question** |
| **A study on the detection and validation of Protein-Protein Interaction Inhibitors for Cystic Fibrosis treatment using Machine Learning virtual screening approach** |

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| **Plain English summary** (100 words max)  *If your request is granted this and the title of your project will be published on* https://www.cysticfibrosis.org.uk/the-work-we-do/uk-cf-registry/apply-for-data-from-the-uk-cf-registry |
| Cystic fibrosis (CF) is a clinically significant genetic disorder with limited therapeutic options targeting its complex molecular mechanisms, including protein-protein interactions (PPIs). In this project, we aim to identify novel small-molecule inhibitors that disrupt CF-relevant PPIs by developing an AI-driven virtual screening platform integrated with a curated database. We plan to apply machine learning models to predict compound-protein interactions, perform molecular docking to evaluate binding affinities at PPI interfaces, and employ cheminformatics tools to analyze molecular properties and prioritize promising candidates for further in silico validation.    (B) Functioning of healthy and unhealthy CFTR  (A) Cystic fibrosis and the CFTR gene in chromosome seven whose malfunction is responsible for the disease.    (B) Pathophysiological process   1. Normal CFTR in which the Cl- can pass; as a result, the concentration and ASL viscosity are maintained, and when Cl- cannot pass, CF can be caused |

Actions of cystic fibrosis transmembrane conductance regulator (*CFTR*) modulators as *correctors* and *potentiators*

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| **Description and rationale of project** |
| |  | | --- | | *Part 1 – Introduction & background (including short rationale)* | |
| Cystic fibrosis (CF) is a genetic disorder caused by mutations in the **CFTR gene**, leading to defective chloride transport and severe respiratory and digestive issues. While CFTR modulators have improved treatment outcomes, they only target specific mutations, leaving many patients without effective therapies.  Recent studies highlight the role of **protein-protein interactions (PPIs)** in CF pathophysiology, particularly in CFTR protein misfolding and degradation. Targeting these PPIs could offer new therapeutic strategies for CF.  This study aims to utilize **Machine learning techniques**, **molecular docking**, and **cheminformatics** to identify small molecules that inhibit PPIs involved in CF. By combining **genomic data** from CF patients with advanced computational tools, we aim to identify novel compounds to correct CFTR function or prevent its degradation, offering new therapeutic options for CF patients, especially those not benefiting from existing treatments.    Actions of cystic fibrosis transmembrane conductance regulator (*CFTR*) modulators as *correctors* and *potentiators* |
| |  | | --- | | *Part 2 – Methods (for individual patient level data request please provide a summary of your*  *methods. If requesting aggregate data please describe how you intend to use the data)* |   We aim to integrate patient-level data on gene expression and CFTR mutation with protein-protein interaction (PPI) networks to identify high-impact molecular targets for cystic fibrosis (CF). Using machine learning algorithms (e.g., Chemprop, DeepChem), we will correlate individual genetic profiles with perturbations in PPI networks. Virtual screening, molecular docking (AutoDock Vina, Glide), and molecular dynamics simulations (GROMACS) will then be used to identify and validate small-molecule inhibitors. In silico ADMET profiling will further prioritize drug candidates for experimental testing. Patient-level data will be anonymized and analyzed under strict compliance with data protection guidelines to ensure ethical use.  **Machine Learning Approach**  **Virtual Drug Screening Approach** |

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| **Intended dissemination of project results**  *Including anticipated publications, presentations or reports* |
| * Peer-Reviewed Publications * Scientific Conferences and Symposia * Project Summaries (Funding agencies) |

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| **Name and qualifications of person performing the statistical analysis** |
| DHIVYA G, Research Scholar, VIT, dhivya.g@vit.ac.in |

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| **Please name any funding bodies that are supporting this work. This should include names of pharmaceutical companies where relevant** |
| * University Support |

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|  | **Check this box if no funding has been received to support this work** |

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| **Exact details of data required** | |
| Year(s) required: |  |
| Variables required:  *(Demographic Pro forma /*  *Annual review Pro forma)* |  |
| Expected Completion Date: |  |

# *Please note it may take up to 8 weeks to extract, prepare and provide data following approval of a request*

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| If your data request is approved, we would be grateful if you could send the registry copies of any papers or abstracts where this data has been used |

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| *[[1]](#footnote-1)Checklist Pre-Review* | *Date* | *Initials* |
| *Research team includes CF Professional?* |  |  |
| *Is there sufficient methodology?* |  |  |
| *Does request meet all GDPR guidelines for data transfer?* |  |  |
| *Are all variables requested available in registry?* |  |  |

1. FOR OFFICE USE ONLY [↑](#footnote-ref-1)