PERMIT

PERsonalised MedicIne Trials

PERMIT will produce recommendations for robust and reproducible personalised medicine research

Mission: *To develop and disseminate recommendations on personalised medicine research methodology, aiming to ensure the scientific excellence, validity, robustness, reproducibility, and acceptability of results*

Vision: *Enhanced personalised medicine research and optimised, innovative healthcare solutions*

The onset of high throughput data generation technology was a major enabler for the patient-centred 'personalised medicine' paradigm. Personalised medical practice requires research on patient stratification, leading to the identification of homogeneous patient clusters. Identification of patient subgroups based on a limited number of determinants (companion diagnostics) is now increasingly being replaced by data-driven patient stratification. Such stratification may be used to define a new disease taxonomy, to refine diagnostic procedures, or to propose more targeted treatments for each of the homogeneous patient clusters.

Both scientific excellence and acceptance by health authorities of results derived from such personalised medicine approaches require established standards. These standards must address issues such as high-throughput data generation techniques, data quality, security and traceability, cohort design and methodology, statistical power, use of algorithms, scoring and validation approaches, clinical trial design, choice of comparator, etc. The PERMIT project was designed to drive the development of established standards.

CONSORTIUM

ECRIN – European Clinical Research Infrastructure Network

Parc Sanitari Sant Joan de Déu

elixir Luxembourg

TMF

KCE – Federaal Kenniscentrum voor de Gezondheidszorg / Centre Fédéral d'Expertise des Soins de Santé / Belgian Health Care Knowledge Centre

EPF – European Patients Forum

Université de Paris

DLR – Deutsches Zentrum für Luft- und Raumfahrt

eatris

Universitas Bergensis

Instituto de Salud Carlos III

IRCCS – Istituto di Ricerche Farmacologiche Mario Negri

Istituto Superiore di Sanità

EXPECTED OUTCOMES

In the first year of the project, the literature on methods in personalised medicine was mapped and a detailed gap analysis was undertaken.

Building on the gap analysis recommendations will be developed for the various stages in personalised medicine development, through a series of dedicated workshops with the PERMIT Consortium and field experts.

The recommendations will address, among other key issues:

- *Sample size definition for stratification/validation cohorts*
- *Methods for the integration of retrospective cohorts*
- *Stratification and algorithm validation methods and approaches*
- *Data quality requirements from a regulatory and research perspective*
- *Optimal selection of clinical trial designs for PM*
- *Reducing bias and gaps in PM study documentation, reporting and publication*

The findings will be disseminated through scientific publications and training to foster adoption and facilitate implementation

For more information on the PERMIT project and the upcoming workshops visit: **https://permit-eu.org/**