Replaced

REGULATORY IMPACT

the rabbit pyrogen test, by non-animal methods



A common animal test is no longer required in Europe

THE CHALLENGE

Vaccines, some antibiotics and blood products must be tested for pyrogens, contaminants that can trigger fever. For decades, the standard test used rabbits.

WHAT VAC2VAC DID

VAC2VAC validated the monocyte activation test (MAT), a cell-based method, and showed that non-animal tests can reliably detect those contaminants.

WHAT CHANGED

The European Pharmacopoeia Commission removed the rabbit pyrogen test from 57 texts. Manufacturers can now use in vitro tests instead.

> *A major achievement for animal welfare. This historic step will have a significant impact on the replacement, reduction and refinement of animal tests in the quality control of medicines.*
>
> **European Directorate for the Quality of Medicines & HealthCare (EDQM)**

VAC2VAC is supported by the Innovative Medicines Initiative, a partnership between the European Union and the European pharmaceutical industry.

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Over 4

companies now use Mobilise-D algorithms in their products

INDUSTRY UPTAKE



The way we walk becomes a measure of health

THE CHALLENGE

Declining mobility signals the progression of diseases such as Parkinson's, multiple sclerosis and COPD. Yet clinicians only see patients for a few minutes.

WHAT MOBILISE-D DID

Mobilise-D validated open-source algorithms that turn data from a single wearable sensor into 24 digital mobility outcomes, measured in everyday life.

WHAT CHANGED

EMPATICA, Clario, McRoberts and ActiGraph have integrated the algorithms into their products, including platforms used in clinical trials.

> *The feedback we've gotten from patients is that they are quite happy to do things at home. It even improves compliance because people feel more empowered.*
>
> **Mike Jackson**, iXscient

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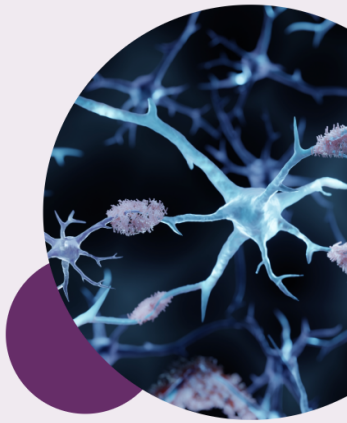
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Mobilise-D is supported by the Innovative Medicines Initiative, a partnership between the European Union and the European pharmaceutical industry.

Endorsed

REGULATORY IMPACT

by the European Medicines Agency, for use in clinical trials



A common scale to measure Alzheimer's disease on brain scans

THE CHALLENGE

Until now, clinicians looked at brain scans and drew subjective conclusions about how far a person's Alzheimer's disease had developed.

WHAT AMYPAD DID

AMYPAD validated the Centiloid method: a 0–100 scale that measures how much amyloid, the first protein to build up in Alzheimer's, is visible on a PET brain scan.

WHAT CHANGED

The European Medicines Agency (EMA) endorsed the method. It can now be used with confidence in clinical trials to measure whether new treatments work.

> *It lends credibility for it to be used as an outcome measure in clinical trials, and it's a stepping stone to routine use by clinicians.*
>
> **Gill Farrar**, GE Healthcare Life Sciences

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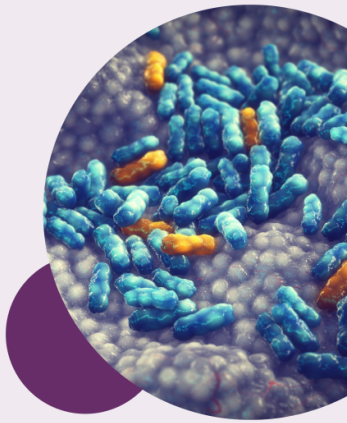
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30

countries where EMBLAVEO® is now authorised

REGULATORY IMPACT



A new treatment against drug-resistant bacteria, approved across the EU

THE CHALLENGE

Gram-negative bacteria resistant to multiple antibiotics cause an estimated 35,000 deaths in the EU every year. Patients often have few treatment options left.

WHAT COMBACTE-CARE DID

COMBACTE-CARE supported two clinical studies showing that the treatment (aztreonam-avibactam) is effective and well tolerated.

WHAT CHANGED

The European Commission authorised Pfizer's EMBLAVEO®, the first treatment of its kind in the EU, valid in all 27 Member States plus Iceland, Liechtenstein and Norway.

> *It provides a new tool to fight antibiotic resistant bacteria, an area where any new breakthrough treatment makes a real difference to public health.*
>
> **Marc Lemaître**, DG Research & Innovation, European Commission

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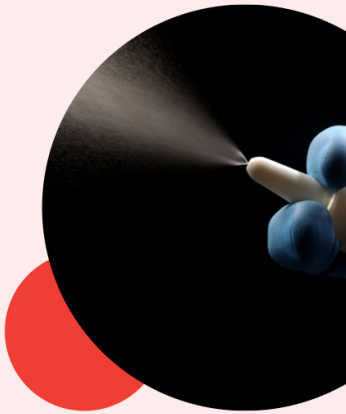
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COMBACTE-CARE is supported by the Innovative Medicines Initiative, a partnership between the European Union and the European pharmaceutical industry.

Faster

HEALTH RESILIENCE

vaccine trials, thanks to a controlled human infection model



Testing a vaccine that stops whooping cough spreading

THE CHALLENGE

Around 85% of infants worldwide are vaccinated against whooping cough, yet outbreaks persist: the vaccine prevents the disease, not infection and spread.

WHAT PERISCOPE DID

PERISCOPE built a controlled human infection model and a high-quality bacterial bank to measure quickly whether a vaccine prevents infection.

WHAT CHANGED

A new nasal vaccine is now being tested with these tools. Results from the final trial are expected soon.

> *The beauty of using this controlled human infection model is that it speeds up the clinical trial process considerably.*
>
> **Camille Locht**, Institut Pasteur de Lille

PERISCOPE is supported by the Innovative Medicines Initiative, a partnership between the European Union and the European pharmaceutical industry.

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300+

RESULTS IN USE

models of over 20 childhood cancer types, open to researchers



Getting medicines to children with cancer faster

THE CHALLENGE

Only 9 of the 150 cancer medicines approved in the last decade targeted children's cancers. One reason: a lack of research tools designed for them.

WHAT ITCC-P4 DID

ITCC-P4 created more than 300 tumour models derived from patient tumours, covering over 20 types of paediatric cancer.

WHAT CHANGED

The models are now available to drug developers through a non-profit organisation: the first sustainable platform for preclinical testing of childhood cancer drugs.

> *For the first time paediatric research is on an even footing with what's been available to adult cancer research for decades.*
>
> **Lou Stancato**, formerly of Eli Lilly, now Indiana Biosciences Research Institute and Indiana University School of Medicine

ITCC-P4 is supported by the Innovative Medicines Initiative, a partnership between the European Union and the European pharmaceutical industry.

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Accelerated

CLINICAL TRIALS

the search for depression treatments, several being tested at once



A Europe-wide depression trial built on EU-PEARL designs

THE CHALLENGE

About half of people with depression respond only partly, or not at all, to antidepressants. Testing alternatives one trial at a time is slow and costly.

WHAT EU-PEARL DID

EU-PEARL designed platform trials that test several treatments at once, so results come faster, recruitment is easier and fewer patients get a placebo.

WHAT CHANGED

Its master protocol for depression will be used in the first Europe-wide platform study on depression treatments, funded by the Wellcome Trust, in six countries.

> *Because of EU-PEARL, we had a track record of successful collaboration and all the pieces were in place to demonstrate that this would actually work in practice.*
>
> **Stefan Gold**, Charité – Universitätsmedizin Berlin

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350m

HEALTH RESILIENCE

harmonised health records, comparable across countries



The largest study yet on the global effect of drug shortages

THE CHALLENGE

Drug shortages delay treatment and push patients towards less suitable alternatives. Anticipating them requires better, comparable data.

WHAT EHDEN DID

EHDEN harmonised more than 350 million pseudonymised health records to a common data model (OMOP), making real-world data comparable across countries.

WHAT CHANGED

This made it possible to measure how shortages of 16 medicines changed their use across Europe and North America.

> *With more than 50 data partners and many European countries represented, this study is the largest real world evidence study ever conducted, demonstrating the value of the EHDEN network.*
>
> **Daniel Prieto-Alhambra**, University of Oxford & Erasmus MC

EHDEN is supported by the Innovative Medicines Initiative, a partnership between the European Union and the European pharmaceutical industry.

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Rolled out

POLICY UPTAKE

the EDENT1FI screening protocol and non-invasive type 1 diabetes test, across Italy



Italy screens children for type 1 diabetes with EDENT1FI tools

THE CHALLENGE

Undiagnosed type 1 diabetes can lead to ketoacidosis, a life-threatening condition. Screening means children can get care before complications arise.

WHAT EDENT1FI DID

EDENT1FI developed a master protocol for screening and a non-invasive capillary test that detects multiple markers of the disease.

WHAT CHANGED

Italy uses both in its national screening programme. In the Milan region, all screening follows the EDENT1FI protocol, and other countries are now asking for advice.

> *We feed them, they feed us. It's a very good form of collaboration.*
> **Chantal Mathieu**, KU Leuven

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1 day

of low-dose treatment: as effective, with fewer side effects

CLINICAL TRIALS



An answer in 4 years, not 10: faster trials for type 1 diabetes

THE CHALLENGE

In Europe there is no approved therapy that stops type 1 diabetes from progressing.

WHAT INNODIA DID

INNODIA's adaptive trial tested four doses of an existing drug, anti-thymocyte globulin, and a placebo at the same time, in children and young adults.

WHAT CHANGED

One day of treatment at a low dose (0.5 mg) proved as effective as a higher dose, with fewer side effects. The benefit was greatest in children aged 5–9.

> *If you were doing this in a classical way, one dose versus placebo, it would take you 10 years before you could get this answer*
>
> **Chantal Mathieu**, KU Leuven

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Piloted

INDUSTRY UPTAKE

by GSK and Sanofi in their clinical trials

Giving clinical trial participants their data back



THE CHALLENGE

Trial participants have the right to their own data. But releasing it too early can compromise a trial, and some data needs expert interpretation.

WHAT FACILITATE DID

FACILITATE built a GDPR-aligned ethical framework: sponsors offer participants their data after the trial and explain when and how they will receive it.

WHAT CHANGED

GSK and Sanofi are piloting the framework, and other companies have shown interest.

> *If you enhance trust, you have a higher recruitment rate and a lower attrition rate.*
>
> **Johanna Blom**, Università degli Studi di Modena e Reggio Emilia

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FACILITATE is supported by the Innovative Medicines Initiative, a partnership between the European Union and the European pharmaceutical industry.

Measured

CLINICAL TRIALS

quality of life, as rigorously as survival or tumour response



Making quality of life count in cancer trials

THE CHALLENGE

Cancer treatments can be gruelling. Yet quality of life was measured so inconsistently in trials that results could not be compared, and were often overlooked.

WHAT SISAQOL DID

SISAQOL developed tools to make quality-of-life endpoints meaningful and standardised, together with patients, researchers, industry, regulators and HTA bodies.

WHAT CHANGED

Quality of life in cancer trials can now be analysed as rigorously as other endpoints. An interactive table guides researchers; checklists help patient advocates review protocols.

> *The idea was to make these endpoints as relevant as the other clinical endpoints.*
>
> **Madeline Pe**, European Organisation for Research and Treatment of Cancer

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SISAQOL is supported by the Innovative Medicines Initiative, a partnership between the European Union and the European pharmaceutical industry.

Saved

RESULTS IN USE

a vaccine batch in Brazil, proven undamaged by Inno4Vac modelling



Faster, more efficient vaccines with predictive models and simulations

THE CHALLENGE

Vaccines are fragile: heat or shaking can make them ineffective, and stability studies take around three years. Yet in a crisis, vaccines are needed fast.

WHAT INNO4VAC DID

Inno4Vac built open-source models, algorithms and tools to speed up vaccine manufacturing. One example is CADET-Hub, which simulates the process end to end.

WHAT CHANGED

In Brazil, a vaccine batch exposed to the wrong temperature was due to be destroyed. An Inno4Vac simulation using advanced modelling showed it was safe to use, reducing waste.

> *Inno4Vac is building digital tools that allow for the acceleration and optimisation of the vaccine production process.*
>
> **Nicola Viebig**, European Vaccine Initiative

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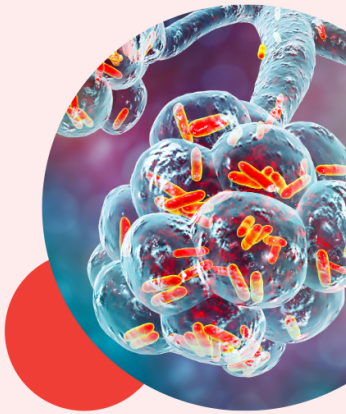
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Inno4Vac is supported by the Innovative Medicines Initiative, a partnership between the European Union and the European pharmaceutical industry.

Compared

HEALTH RESILIENCE

results across labs, thanks to a standard pneumonia model



A shared model to speed up new antibiotics for pneumonia

THE CHALLENGE

Drug-resistant pneumonia kills over 400,000 people a year. New antibiotics are needed, but test protocols vary between labs, so results are hard to compare.

WHAT COMBINE DID

COMBINE developed a standardised lung infection model and a repository of seven well-characterised reference bacterial strains.

WHAT CHANGED

Results can now be compared across labs. The strains are already being used in studies beyond the project. These tools will help to accelerate antibiotic development.

> *Drug-resistant pneumonia kills over 400,000 people a year. New antibiotics are needed, but test protocols vary between labs, so results are hard to compare.*
>
> **Jon Ulf Hansen**, Statens Serum Institut

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Discovered

UNDERSTANDING DISEASE

previously unknown ‘bridge genes’, new leads for treatments



Reading data in new ways reveals hidden genes behind psoriasis

THE CHALLENGE

Psoriasis and atopic dermatitis affect over 300 million people worldwide and remain incurable. At the molecular level, researchers used to study one gene at a time.

WHAT BIOMAP DID

BIOMAP combined existing genetic datasets from people with psoriasis and analysed them together, to see how genes work as a network rather than in isolation.

WHAT CHANGED

‘Bridge genes’ were found linking psoriasis genes, pointing to new drug targets and biomarkers. Even healthy-looking skin from people with psoriasis proved different from that of those without.

> *When you interrogate multiple data sets together, you start appreciating patterns that you wouldn’t be able to appreciate when looking at individual data sets one by one.*
>
> **Dario Greco**, Tampere University

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BIOMAP is supported by the Innovative Medicines Initiative, a partnership between the European Union and the European pharmaceutical industry.