CLUST-ER HEALTH SALUTE E BENESSERE

HORIZON-HLTH-2022-TOOL-11-01:
Optimising effectiveness in patients of existing prescription drugs for major diseases (except cancer) with the use of biomarkers

Expected Outcome:

This topic aims at supporting activities that are enabling or contributing to one or several expected impacts of destination 5 “Unlocking the full potential of new tools, technologies and digital solutions for a healthy society”. To that end, proposals under this topic should aim for delivering results that are directed, tailored and contributing to all of the following expected outcomes:
Diagnostics industries move towards market approval for companion diagnostics.
Regulatory authorities approve companion diagnostics and make recommendations for the prescription of existing drugs.
Health care providers use biomarkers with existing pharmaceuticals to treat more efficiently and cost-effectively patients, with less adverse effects

CLUST-ER HEALTH SALUTE E BENESSERE

HORIZON-HLTH-2022-TOOL-11-01:
Optimising effectiveness in patients of existing prescription drugs for major diseases (except cancer) with the use of biomarkers

Scope:

The applicants should perform the clinical validation of qualified biomarkers (not limited to molecular biomarkers) that will enable the identification of appropriate patients to ensure an effective and efficient use of existing pharmaceuticals in the treatment of major diseases and conditions. The relevant biomarkers should allow providing the right medicinal product, at the right dose and the right time, according to the concept of personalised medicine, taking into account, among others, differences of sex, age, ethnicity and gender identity.

CLUST-ER HEALTH SALUTE E BENESSERE

HORIZON-HLTH-2022-TOOL-11-01:
Optimising effectiveness in patients of existing prescription drugs for major diseases (except cancer) with the use of biomarkers

This topic refers to medicines that are already on the market and not to the validation of biomarkers for the development of new medicinal products. It addresses broadly prescribed medicines for major diseases and conditions, including but not limited to cardiovascular diseases. A condition is that preliminary studies or publications have demonstrated that the pharmaceuticals considered are efficient in less than 50% of the population treated. This topic excludes cancer and rare disease treatments. The applicants should consider existing guidelines, standards and regulations, as appropriate. Synergies with relevant European Research Infrastructures are encouraged.

HORIZON-HLTH-2022-TOOL-11-01:
Optimising effectiveness in patients of existing prescription drugs for major diseases (except cancer) with the use of biomarkers

The EU estimates that an EU contribution of 6.00 million would allow these outcomes to be addressed appropriately.

The total indicative budget for the topic is EUR 60.00 million. Number of projects expected to be funded: 10

Provisional deadlines

Opening: 06 Oct 2021

Deadline(s): 21 Apr 2022

HORIZON-HLTH-2022-TOOL-11-02:
New methods for the effective use of real-world data and/or synthetic data in regulatory decision-making and/or in health technology assessment.

Expected Outcome: This topic aims at supporting activities that are enabling or contributing to one or several expected impacts of destination 5 “Unlocking the full potential of new tools, technologies and digital solutions for a healthy society”. To that end, proposals under this topic should aim for delivering results that are directed, tailored towards and contributing to all of the following expected outcomes:

CLUST-ER HEALTH
SALUTE E BENESSERE

HORIZON-HLTH-2022-TOOL-11-02:

Scope:

With the emerging use of real-world data (RWD) and synthetic data by the pharmaceutical industry and medical devices industry, regulators and HTA bodies need to perform targeted validation of claims through independent analysis. The principal aim of this topic is to address the data needs of health regulatory bodies and HTA bodies across the EU, as outlined in the recently published “HMA-EMA Joint Big Data Taskforce Phase II report: ‘Evolving Data-Driven Regulation’”188 and its associated DARWIN (Data Analysis and Real World Interrogation Network) project189.

To harness the potential of RWD and synthetic data from digital twins and advanced analytical models, and make them actionable for health regulatory decision-making and for health technology assessment, targeted research is needed on the evidentiary value of these data for a number of relevant use cases. In addition, methods need to be developed to increase the usability of such data by different stakeholder groups. Doing so will contribute to the European Health Data Space and maximise the positive impact of DARWIN in driving up the quality of evidence and decisions on the development and use of medicines and digital health innovations.

Access to and analysis of RWD and synthetic data can inform regulatory decision-making throughout the product lifecycle, namely: 1) support product development (e.g. scientific advice, PRIME190),); 2) support authorisation of new medicines and digital health innovations; and 3) monitor the performance of medicines and digital health innovations on the market (effectiveness and safety). Eventually, this will put in place methods and processes that will enable continuous learning from pre-authorisation procedures and authorisation applications on the use of RWD and/or synthetic data.

HORIZON-HLTH-2022-TOOL-11-02:

Proposals should address all of the following areas:

- Develop a set of evidentiary standards to be pre-specified and used in the analysis of real-word evidence and/or synthetic data applied to different types of regulatory advice and/or health technology assessment and decisions on the safety and efficacy/effectiveness of medicines and digital health innovations (e.g. in complement to clinical trial data in an authorisation application, or for extension of indications, post marketing surveillance, amendment of product information or regulatory actions on the marketing authorisation due to safety concerns). This includes validating the use of advanced analytical methods for regulatory decision-making and/or health technology assessment.
- Address aspects that would enable moving towards a standard data quality framework reproducible across different types of RWD and/or synthetic data sources for regulatory decision-making and/or health technology assessment, with a characterisation of the data collection, management and reporting and an empirical data quality validation. In this regard, it will be important that successful proposals liaise with and closely monitor the work carried out in the context of the European Health Data Space.

HORIZON-HLTH-2022-TOOL-11-02:

Proposals should address all of the following areas:

- Enhance the performance and efficiency of large randomised clinical trials and new models of clinical trials by developing standardised processes and methods to access RWD and/or synthetic data (e.g., facilitating the detection of various types of health outcomes during the treatment period of a double-blinded trial by linkage to appropriate electronic health care record databases, etc.), for regulatory decision-making and/or health technology assessment.
- Define methodological standards for the regulatory acceptability of RWD, and/or synthetic data in the context of clinical trials augmented with RWD, and/or synthetic data, for regulatory decision making and/or health technology assessment.
- Test the ability of machine learning methods to help identify relevant RWD, and/or synthetic data to match with and to interpret clinical trials, for regulatory decision-making and/or health technology assessment.
- Assess and validate how machine learning methods can be systematically harnessed to screen a large amount of data, including unstructured data, in many electronic databases to identify factors affecting efficacy and safety of treatments and/or digital health innovations, for regulatory decision-making and/or health technology assessment. The cross-border interoperability dimension should be taken into account.

HORIZON-HLTH-2022-TOOL-11-02:

The EU estimates that an EU contribution of 7.00 million would allow these outcomes to be addressed appropriately.

The total indicative budget for the topic is EUR 35.00 million.
Number of projects expected to be funded: 5

Provisional deadlines
Opening: 06 Oct 2021
Deadline(s): 21 Apr 2022