MISSIONE 4
ISTRUZIONE
RICERCA

Finanziato dall'Unione europea
NextGenerationEU

Ministero dell'Università e della Ricerca

Italiadomani
PIANO NAZIONALE DI RIPRESA E RESILIENZA

Development of innovative biologics for the treatment of unmet medical needs in rare metabolic disorders and oncology

Università degli Studi di Urbino
23 maggio 2024

SPOKE 8 — PNRR MISSIONE 4

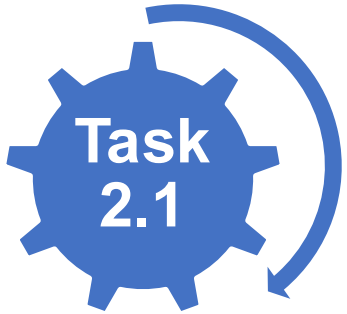
INNOVATIVE THERAPEUTIC APPROACHES: NEW CHEMICAL ENTITIES, BIOLOGICS AND DRUGS DELIVERY

WP1

WP2

WP3

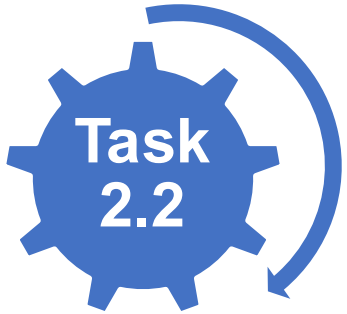
WP 2 is organized in three Tasks: 2.1, 2.2 and 2.3



Implementation of a new facility for the production and characterization of biologics to be used in preclinical investigations

2.1.1_Setup of an externally accessible, confidentiality compliant, infrastructure for the production of synthetic biologics including recombinant enzymes, antibodies and modified mRNA in bacteria, yeast and mammalian cells or by synthetic methods

2.1.2_Setup of a Quality Control laboratory (QC lab) for biologics



Selection of products to be developed and *in vitro* testing of biologics obtained in proper cell models

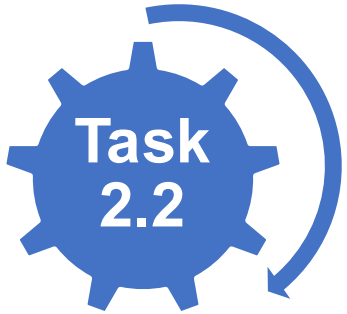
2.2.1_Representative product candidates selected by bioinformatic tools (and by availability of proper cellular and animal models):

- **recombinant enzymes***
- **recombinant antibodies (mAbs)*** in different formats (scFv, full length)

**produced and expressed in different hosts, including bacteria, yeast and plant cells, but also by transient transfection in CHO or other mammalian cells*

- **modified mRNAs** by synthetic methods

2.2.2_ Definition/development of **monoclonal antibody libraries** with different biotechnological applications: therapeutics; diagnostics and research tools; vaccine development.



Selection of products to be developed and *in vitro* testing of biologics obtained in proper cell models

2.2.3_Quality control laboratory for biologics:

- *All validated methods and procedures for the qualification of biologics (including chemical, physical and biological properties, tests for purity, impurities, endotoxin, residual host cell proteins, residual host cell DNA, potency of products*.
- *Qualification of raw materials*.
- *Bioburden and absence of viral agents*.

2.2.4_*In vitro* testing of the new biologics in proper cell models *(including biological activity of the product; cell toxicity and cell viability tests)*

Task 2.3

Animal models for *in vivo* testing of candidate products generated in Task 2.1 and Task 2.2

The University animal facility will be expanded for the *in vivo* testing of products generated under WPs 1-3 in proper animal models. The facility has already access to different animal models of human metabolic diseases; it will be expanded to additional models in the area of oncology to perform *in vivo* efficacy studies

2.3.1_ *In vivo* testing of the recombinant enzymes

2.3.2_ *In vivo* testing of the recombinant antibodies

2.3.3_ *In vivo* testing of the modified mRNAs

2.3.4_Screening of the comprehensive biological library for Ab selection against target antigens

New personnel recruited on the Project funds:

one researcher; two Post-Doc contracts renewal on the activity of Vitality project; two post-doc acquisition procedures are ongoing….

Different expertise in WP2 components:

biochemists, molecular biologists, chemists, clinical microbiologists, pharmacologists....

Feasibility of the project

Availability of most of the equipment needed for the project:

Homogenizer Orbitrap Exploris Basic 240, Imager iBright™ FL1500 Imaging System, RealTime PCR System, Nanodrop and Qubit, Hoods, Centrifuges…

Thank you for the attention