

## Potential future IMI Call topics

### About this document

The following topics are under consideration for inclusion in future IMI Calls for proposals in the longer term. The discussions on these topics are still in their early stages. For this reason, the topics may change considerably and they will probably not be ready for inclusion in an IMI Call for proposals for several months. Furthermore, as the discussions advance, it is likely that some topics will be added to this list while others will be dropped. In any case, we hope that this list will give potential applicants a useful glimpse into what is under development in the longer term, and provide additional time to enhance their network. We will update this list whenever we have updates on the status of the topics.

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**All information regarding future IMI Call topics is indicative and subject to change. Final information about future IMI Calls will be communicated after approval by the IMI Governing Board.**

## Neurodegeneration and other neuroscience priorities

### Pain

#### Digital transformation of clinical trial endpoints in pain

As part of the programme on digital endpoints, started in 2018, with a specific focus on different diseases, the objective is to develop objective, continuous or high-frequency digital measures of clinical efficacy and disability experienced by patients with pain. Digital endpoints relevant to clinical pain (e.g. pain severity, daily activity, quality of life, sleep) will be validated in selected pain conditions with the goal of obtaining acceptance of digital endpoints by regulators and HTA bodies as primary or key secondary endpoints for use in pivotal pain trials.

#### Placebo effect in pain

The placebo response in clinical trials in pain can be substantial, and high placebo effect is associated with reduced drug-placebo difference, which, in turn, interferes with signal detection for new therapies. This topic aims to develop and validate effective methods for reduction of placebo response in randomised controlled trials in pain. It will support the identification of factors influencing the magnitude of the placebo response by analysing patient clinical data and site characteristics.

### Psychiatry

#### Psychiatric ratings using intermediate stratified markers -2 (PRISM-2)

This topic supports activities to further progress on delivery of a quantitative biological approach to the stratification of neuropsychiatric disorders, specifically Alzheimer's dementia and schizophrenia while also considering a wider application. Activities will focus on understanding neural substrates of the symptoms of social withdrawal and both the identification of biomarkers and clinical rating tools that can be proposed for regulatory validation.

### **Expected impact of the topic(s)**

- novel drug pathways for neurodegenerative and psychiatric disease interception and for pain, including validated biomarkers and clinical trial endpoints ;
- accelerate patient access to innovative medical treatments for neurological, psychiatric disorders and for pain;
- reduce the patient burden due to mental health disorders;
- enable more efficient and cost-effective clinical trials and real-world studies.

## **Immunology**

### **Psoriatic arthritis**

Early diagnosis, prediction of radiographic outcomes and the development of rational, personalised treatment strategies to improve outcomes in psoriatic arthritis (PsA). This topic will address the major unmet need to identify early predictors of progression to PsA in patients with skin psoriasis, thereby enabling earlier intervention and possibly even preventing development of PsA. The results will provide stratification of PsA patients in clinical trials and allow a potential reduction of the clinical and economic burden of disease for patients by postponing the start of the PsA symptoms.

### **Expected impact of the topic(s)**

- improved methods for recognition and diagnosis of autoimmune and inflammatory disorders and a range of treatment options;
- earlier availability of new, more cost-effective therapies for patients most likely to benefit;
- expanding our current knowledge will give rise to more precise, targeted treatments yielding long-lived reductions in disease and improved patient quality of life, and fulfilling unmet medical needs in patient care.

## **Infection control including vaccines**

### **New topic(s) under the AMR accelerator platform**

The objective is to expand the AMR Accelerator Programme launched in 2018, in particular under the Pillars A and C to expand activities and accelerate scientific discoveries in antimicrobial resistance (AMR) and to progress a pipeline of potential therapeutic, biologic and preventive medicines & procedures, e.g. diagnostics, to manage patients and/or populations with resistant bacterial infections in Europe and across the globe.

### **Expected impact of the topic(s)**

- a pipeline of promising new agents for tackling antibiotic-resistant bacterial infections;
- a significant contribution to the development of a vibrant AMR research environment in the EU and to the strengthening of its competitiveness and industrial leadership;
- major impact on the improvement of public health.

## **Translational safety**

### **Dosing in specific populations**

The term specific population has been used to describe patient attributes that may require alterations in the course of therapy when compared to typical patients; examples include renal and hepatic-impaired patients, children, the elderly and pregnant women. These populations are often excluded or under-represented in

pivotal trials. 50% to 80% of new molecular entities do not have explicit dosing recommendations for severe renal and hepatic impairment, respectively. Therefore, the objective of this topic is to establish a framework for developing models, criteria for establishing adequacy of predictions, and a drug-development-regulatory framework for incorporation of derived dosing recommendations into product labels.

#### **Expected impact of the topic(s)**

- better prediction and understanding of toxicities of drugs;
- reduce use of animals in toxicology studies;
- accelerate clinical development programmes and better identify treatments for specific groups of patients;
- reduced costs and enhanced commercial viability of drug development.

## **Big data, digital health, clinical trials and regulatory research**

### **ROADMAP 2: need and opportunity for public-private collaborative research to continue the RoadMap efforts**

This topic will leverage prior phase I work of the project 'Real-world outcomes across the Alzheimer's disease (AD) spectrum for better care: multi-modal data access platform', ROADMAP. The objective is to validate the outcomes of interest with a broader range of stakeholders including clinical experts and AD clinical centres, patient organisations, regulatory and national health authorities and payers across the EU. This should result in a standardised data set to be collected via a proof-of-concept study for real-world (RW) standardised prospective data collection in patients with AD.

Digital priorities are also addressed in the "Digital Transformation of Clinical Trial Endpoints in pain" topic in the section on Neurodegeneration above.

#### **Expected impact of the topic(s)**

- improved transparency of data reuse and of its impact on research & development;
- faster translation of insights from real-world health data to biomedical research and development approaches;
- enable more efficient and cost-effective clinical trials and real-world studies;
- enhanced drug efficacy and effectiveness via a better understanding and adherence to medicines.

## **Oncology**

The Oncology area is also addressed in the CAR-T topic in the "Facilitating the translation of advanced therapies to patients in Europe" section below.

## **Facilitating the translation of advanced therapies to patients in Europe**

### **ATMP Patient Registries Outcomes Data and Evidence**

This topic aims to: (1) pilot the benefits of a holistic, pan-EU registry for a specific rare disease (e.g. Duchene muscular dystrophy; haemophilia) serving the needs of academia, scientific associations, industry, patient organisations and healthcare payers; (2) to directly address the current gap in patient centricity and patient perspectives in advanced therapies medicinal products (ATMPs), which are not recorded in any existing efforts. Once completed, the pilot could potentially serve as a model for other rare diseases and provide optimal access of innovative medicines to patients.

## **Innovative Manufacturing of Advanced Therapeutic Medicinal Products, ATMPs**

This topic addresses the challenges of manufacturing of Advanced Therapies Medical Products (ATMPs). Activities will include developing closed and automated production systems, highly sensitive analytical tools/methods for increased scale and improved robustness of manufacturing processes. A further objective is to foster progress of manufacturing know-how and education related to ATMP acceptance / reimbursement and Current Good Manufacturing Practice (CGMP).

### **Expected impact of the topic(s)**

- to enhance research and development of advanced therapies in the EU and the Horizon 2020 Associated Countries as a fully-fledged industrial activity to make the EU more competitive and make advanced therapy products available to all patients in need;
- a powerful public-private innovation platform for efficiently addressing all challenges in the pathway from science to healthcare systems and patients including those with rare diseases.

## **Other enablers of research topics**

### **Handling of biologic drug products**

The objective is to get insight into the impact of the handling of biologic drug products – after they are released by the manufacturer and up to their administration – on the stability of the drug. Routine handling or unintentional mishandling of therapeutic protein products may cause degradation that can potentially compromise the clinical safety and efficacy of the product. This topic supports activities that should allow for identification of the risk factors and addressing them in drug production and supply processes.

### **Expected impact of the topic(s)**

- increased safety and efficacy of biological drug products;
- reduction of costs via increased efficiency of drug discovery and drug production.