IMI2 JU Scientific Committee recommendations regarding Involvement of regulators and regulatory science in public private partnerships

Recommendation 1

Identifying the relevant regulatory authorities for implementation of project outputs is crucial. Failure to seek early regulatory guidance can result in unnecessary delays and impede sustainability. Therefore, the Scientific Committee recommends that:

1. the identification of the relevant regulatory authorities, i.e. European Medicines Agency (EMA), national competent authorities, and other relevant entities such as the health technology assessment bodies (HTA), ideally occurs at the topic development and/or call formulation stage
2. project consortia develop a regulatory strategy, which is outlined in the proposal and the description of action (DoA)
3. project consortia document that the relevant regulatory bodies and decision makers have been identified and contacted early in the project life span (i.e. as deliverables in annual report)
4. regulatory affairs experts from industry partners (EFPIA companies) provide support during topic development and within the projects.

Recommendation 2

Regulatory research is an important component of IMI2 and future public private partnerships (PPP). Research questions in this area need to be well defined and addressed with state-of-the-art research tools. The scope of this research is to produce scientific data as a basis for regulatory decision-making. This research will strengthen research projects in areas where new regulatory solutions seem to be beneficial or necessary for further evolvement and sustainability of the research field and the project outputs. In addition, research topics in IMI2 (or future health PPP) can be selected with the primary (or secondary) goal to improve the validity of benefit-risk evaluations, the harmonisation of regulatory decision-making and the quality of surveillance measures.

Recommendation 3

The IMI Regulatory Science Summit is an excellent establishment and should be continued in the new framework programme. The regulatory expert opinion should also be called upon to define regulatory research questions to be included in the topics.

Recommendation 4

When regulators participate to strengthen the projects´ regulatory strategy and impact, they provide regulatory guidance and expertise. In these cases, the potential conflict of interest cannot always be avoided, but it can be limited and made transparent.

If the project itself deals with the scientific evaluation of novel regulatory pathways, e.g. IMI project ADAPT-SMART, the regulators participate as “regulatory science experts” and can be considered as “researchers” (rather than regulators), acknowledging that within the project framework they create and evaluate something new.

Some regulators have dual roles because they continue to work as researchers, or even have two affiliations, e.g. a regulatory and an academic affiliation. Whenever individual regulators, who participate in IMI projects, engage as researchers in development of novel methodology and concepts, their participation should be based on their scientific record of accomplishment. In addition, their duties as regulators should

remain unaffected by the participation in the project. In these cases, it is important that the project structure and governance model take this into account, and are designed to avoid any conflict of interest that would preclude their participation. Thus, provisions in structure and governance can protect the regulators neutrality and provide transparency and clarity to the regulators themselves, to their regulatory agency, and to all involved within the consortia.

Finally, in all cases where regulatory guidance is sought, it should be obtained via the formal procedures offered by the regulatory authorities. This avoids the conflict of interest of the individual regulators involved in the project. In addition, regulatory consultants and regulatory affairs experts from industry partners could assist in engaging formally with the regulatory authorities and preparing the relevant documentation.

Recommendation 5

Regulatory agencies willing to participate in IMI2 (or future health PPP) projects should provide guidance on identifying and dealing with potential conflicts of interest for their employees. They should actively screen for and define potential conflicts of interest prior to the start of the project.

The governance structure of the IMI2 (or future health PPP) projects should be designed to create a neutral platform for stakeholders with potential conflicts of interest. The regulatory agencies can support and actively contribute to the development of project governance models that allow their participation without compromising their credibility as regulatory authorities. The governance model should be justified against this background in the grant application, the grant agreement (DoA) and the consortium agreement.

Background

With the implementation of the IMI2 programme the European Commission and EFPIA have committed to research that enables translation and implementation of innovative diagnostics and therapies. It is obvious that with this aim partnering of the private and the public sector became a prerequisite for success. Notably, the ambition of the IMI2 initiative goes far beyond an exchange of expertise, tools or views among sectors; it was designed to be transformative in regards to innovation of the health sector. It is, therefore, important to understand which determinants constitute success of an implementation in health care. The IMI2 programme has been very successful in making us aware of the large portfolio of stakeholders that need to be involved in this process and has actively reached out to these stakeholders to engage in the IMI2 projects. This is a great virtue of the programme, which successfully brought aboard patient representatives, regulatory agencies, in particular the European Medicines Agency (EMA) and health technology agencies. Together, these stakeholders resolve open questions on the value of IMI2 outputs to both the individual patient and the health care system as a whole.

Both translation and implementation programmes often benefit from early regulatory feedback on potential issues that could impede implementation. Implementation usually requires regulatory approval by one or more regulatory authorities relevant to the asset developed in the project. With innovative assets, regulators on the governmental side are often required to (re-)define acceptability criteria, set (new) thresholds for benefit/risk evaluation and (re-)evaluate the current regulatory and legal framework in response to the needs arising from the evaluation process. Sometimes, the existent regulatory procedures need to be overhauled, require new regulatory coherence or even new regulations. To ensure that the implementation of IMI2 project outputs is not impeded by subsequent regulatory processes early involvement of regulators is recommended, facilitated and ultimately enforced in IMI2 projects.

Thus, engagement of regulators is strongly encouraged in IMI2. It is a great virtue of the IMI2 programme that many efforts from the IMI2 programme office, EFPIA, EC and EMA have been made to accommodate the early interaction of researchers and regulators. This includes guidance documents, the regulatory science summit and a recently installed regulatory support team from EFPIA. However, in spite of the obvious importance, involvement of regulators has not yet been widely accepted and implemented in IMI2 projects. Research consortia often seem to be hesitant to engage with regulators, possibly due to

inexperience in dealing with the regulatory authorities; and, there is reluctance of regulators to participate in IMI2, often due to the concern of generating a conflict of interest that could potentially compromise their regular duties. In light of these unresolved issues, the IMI2 Scientific Committee (SC) would like to take the opportunity to address this issue in a discussion paper and provide recommendations on how to improve regulatory participation in IMI2 projects. This includes a short reflection on the potential roles of regulators in the projects and on the definition and aims of regulatory research. Notably, the latter is often mistaken as infrastructural measures for improving existing regulatory procedures, thus underestimating its potential to support and sometimes drive innovation, in particular to achieve wide-spread implementation.

Specific reflections on the recommendations

1. Identification of the project-relevant regulatory authorities (Recommendation 1)

Usually researchers are well acquainted with the regulatory requirements and procedures for their routine work (e.g. approval of clinical trials, animal legislation, use of genetically modified organisms, etc.). However, they are less informed about the regulatory requirements for implementation of their project outputs in IMI2 (e.g. regulatory qualification or approval of a method, a drug, a device or a novel therapeutic concept). However, in areas where regulatory coherence is required, understanding of the regulatory framework is key to success. This knowledge gap was identified early in the course of IMI and was at least partially met by the implementation of the PharmaTrain (https://www.pharmatrain.eu/the-federation.php) Medicines development curriculum, which started out as an IMI project and was sustained by a federation as EU-wide diploma or master course since 2014. Additionally, the European Commission is currently funding a coordinative support action called CSA-STARS (Strengthening Training of Academia in Regulatory Science), a project of a network of National Competent Authorities (NCA) for training and information of academic institutions in regulatory issues in the EU (https://www.csa-stars.eu/).

In light of the developmental scope of the IMI2 projects it is necessary to identify the relevant regulatory bodies at all levels (national, European and international) and seek contact to the responsible regulatory authorities early on. Ideally, the regulatory agencies (European Medicines Agency (EMA), European Environment Agency (EEA), etc.) and the associated regulatory requirements could be identified during the Topic development and/or Call formulation stage. This may help to avoid delays due to unfulfilled or unclear regulatory requirements and would support stringent project implementation once all requirements have been fulfilled.

Notably, the need to define the relevant regulatory authorities is mentioned in the call topic template but presentation of a regulatory concept could also be required in proposals (stage 2 in two-stage procedures). As required, regulatory interaction and further measures such as early involvement of decision makers including health technology assessment (HTA), payers and health care personnel might be necessary. It is, therefore, considered necessary that project consortia are well informed about the regulatory landscape relevant to their projects, about the major regulatory guidance documents and how to contact the regulatory institutions. The project partners have to actively screen their projects for assets that require involvement of regulatory authorities. Early interactions with regulators are particularly valuable in newly evolving fields such as digital therapeutics and medical devices where there is many uncertainties next to rapid dynamics in development and high innovation potential. While the regulatory framework is being developed there often is a complex interplay of intersecting regulatory bodies and it can sometimes be challenging to understand the regulatory pathways.

A very helpful guidance tool was published by IMI2 in 2015 [1] along with a webinar from 2017 (https://www.imi.europa.eu/resources-projects/guidelines-engaging-regulators). Additionally, the EFPIA partners have recently set up a regulatory support team to tackle this issue. They have gathered regulatory experts from the EFPIA companies to provide support because it became clear that the researchers who manage the projects both on the private and public side are usually inexperienced with regulatory affairs.

The EMA, the regulatory agency with most relevance to drug development and, thus, also to IMI2 projects, provides extensive information on the contact points and the different forms of advice and licensing pathways (https://www.imi.europa.eu/resources-projects/guidelines-engaging-regulators) including the specific EFPIA and IMI guidance document [1]. However, contact to national competent authorities (NCA) and to regulatory institutions with other responsibilities might become relevant for certain project assets. For example, advice from the European Directorate for the Quality of Medicines (EDQM) may be relevant for new methods in quality control of drug manufacturing and the European Environment Agency (EEA) might need to be consulted when the products developed contain genetically modified organisms. Furthermore, agencies on national and EU level can provide help concerning the identification and contact of the important regulatory stakeholders on a global level, which may be necessary for more rapid and widespread implementation.

2. Regulatory research in IMI2 (Recommendation 2 and 3)

There is some confusion with the terms of regulatory science and regulatory research. It is not within the scope of this recommendation paper to provide an in-depth analysis of this matter, which has been covered elsewhere [2][3]. However, it is important to distinguish regulatory affairs as the administrative component, regulatory law as the legal component and regulatory science as the science-based approach used by regulators in decision-making processes. Of note, specialist scientific knowledge, e.g. in toxicology, microbiology, epidemiology, biology, entomology, immunology, public health, pharmacology and food science, has been quoted as being key to managing critical incidents in the regulatory sector [4]. Considering this, in some cases, we refer to regulators as “regulatory science experts”.

For the present purpose, the IMI2 Scientific Committee proposes to distinguish the regulatory decision-making process based on the current scientific knowledge (often referred to as “regulatory science”) from what we prefer to call “regulatory research”, e.g. research driving the improvement of the “regulatory science” area. This type of research enables regulatory decision making and provides a scientific basis for closing regulatory gaps or developing new regulatory pathways, developing regulatory coherence, as well as the development of tools that facilitate regulatory evaluation and surveillance of regulatory measures (“vigilance”).

Regulatory science and translational research are closely interlinked because together they represent the seedling enabling the development and subsequent availability of new medicines [3][4]. While most of the work carried out in IMI2 projects is considered as translational science based on a previously identified clinical need, IMI2 also comprises projects that lie in the area of classical regulatory research, e.g. they aim at generating tools, methodologies and test cases for improvement of the scientific basis for regulatory decision-making. This includes the evaluation of safety and efficacy in preclinical and clinical trials as well as post-marketing vigilance and generation of evidence to align with requirements of HTA bodies to better inform and facilitate reimbursement decisions [4][6]. Notably, both EMA and FDA have set priorities and developed roadmaps for “regulatory science” [7][8] but in many areas practical implementation is left to other initiatives and funders, one of them being IMI2. This is relevant to note because it highlights the need for research in this field and a potential role for IMI2 and subsequent programs.

As with any other form of research regulatory research should be based on scientific methodology and demonstration of solid evidence supporting or rejecting a hypothesis. No other standards are deemed acceptable by the SC. Reviewers need to pay attention to compliance with high quality research standards and adherence to the requested novelty and originality of the work. Although an information gathering exercise can be justifiable as part of the project, it is crucial that researchers in this area focus on the generation of original scientific evidence for new and better outlined regulatory solutions or on new tools and methodology that serve regulators to improve their standards. This can include proposals for regulatory guidance documents, mock templates exemplifying specific aspects of regulatory dossiers on chemistry, manufacturing and controls (CMC) and in some cases activities that educate researchers on how to engage with one or more regulatory agencies.

Ideally, the research teams will be interdisciplinary and consist of teams of researchers from within the regulatory field (e.g. governmental employees), those who have to adhere to the regulations (e.g. manufacturers, marketing authorisation holders) and those whose research is affected by the regulations or who have a specific expertise needed for the projects (e.g. researchers in academia and industry). Thus, it is clearly encouraged to include (professional) regulators in the teams with note that it is made clear that the role of these regulators within the project is that of a researcher, not a regulator.

Regulatory research can address pre- and postmarketing activities [4][6]. In IMI2 and health PPP there are four aspects that require regulatory research:

1) IMI2 project outputs often require novel regulatory solutions and/or engagement with multiple regulatory agencies. This should be identified early in the project life span and addressed by providing a scientific basis for regulatory decision-making and, where necessary, specific research on the implications of different regulatory measures.

2) IMI2 projects can address topics that improve the output and quality of pre- and postmarketing regulatory tasks, e.g. development of concepts and tools that improve the evaluation of efficacy and safety of novel medicinal products or facilitate the surveillance of effectiveness and safety of authorized drugs and devices.

3) In some cases, this definition can be extended to compiling evidence for societal and individuals´ perception of risk and benefits of innovative medicines and technologies [2][4].

4) IMI2 project outputs can also provide novel insights into the innovation potential, impact and relevance of novel technologies (such as innovative laboratory techniques, big data analyses or artificial intelligence) for regulatory decision-making processes.

The SC has observed that when new regulatory concepts are required project consortia often limit themselves to dissemination of information to regulators but refrain from implementing “regulatory research” that could provide scientific evidence to justify the change. However, the overall goal is to improve the scientific basis for regulatory decision-making and the work of the regulators. The SC would like to emphasise that this implies a clear definition of the (regulatory) research questions and the development of a research program that delivers tangible scientific output.

From the very start, IMI has raised awareness on engaging with EMA and FDA as highlighted in the guidance [1] (https://www.imi.europa.eu/resources-projects/guidelines-engaging-regulators). In collaboration with EMA and FDA, the IMI programme has also established the bi-annual “Regulatory Science Summit” as an excellent forum for exchange and discussion on regulatory gaps and innovation-driven change in regulatory perspectives. This collaborative effort highlights the need to consider the regulatory framework on a global level. In views of the need for global harmonisation of regulatory standards, beyond the participating regulators coming from EMA, FDA and NCA it has also reached out to other regulators from non-EU countries i.e Health Canada, PDMA. They are challenged with present and future trends pursued by IMI2. The aim of the meeting is to receive input on the proposed research priorities, to discuss questions/gaps of particular interest to regulatory agencies to further maximise the transformational impact of the IMI by means of the proposed topics on drug development and patients’ access to innovation [9][10].

3. Participation of individual regulators in IMI2 projects (Recommendation 4)

Regulators have many important roles. Their responsibilities range from advisory roles (i.e. for developers), identification and management of specific risks, to formulating policies and guidelines, participating in legislative processes and informing the public on current developments from a scientific point of view [4].

IMI2 offers a wide range of opportunities for regulators to engage: they can either participate as researchers based on their individual specific scientific expertise or engage in their role as regulators, communicators and

policy makers. Both of these roles are welcome and needed but they should not be mixed up and it is difficult, if not impossible to fulfil both of these functions at a time.

Of note, when acting as a researcher the regulator joins forces with the other project members and provides expertise and skills to achieve the project goals, e.g. develop and test new methodology and concepts. In many cases, the specific regulatory expertise will be needed to carry out the scientific work plan, in particular to define the goals and feasibility. In some cases, regulators will be interested in participating because they will later on become the main users of a new technology or they want to gain experience in regards to the potential and limitations of applications that will become relevant in future regulatory procedures. In any case, the regulator joining the project in this role should have a track record in research. Importantly, participation based on these intentions is considered as justified and encouraged because the regulatory view point can be very relevant and beneficial for the success of the project. However, despite their knowledge, regulators who are participating as “regulatory science experts” or “regulatory researcher scientists” can provide their regulatory view and knowledge but, importantly, should not get involved from a formal regulatory perspective. The latter would create a conflict of interest arising from the involvement in the project. Thus, whenever a regulator intends to join a project this potential conflict of interest should be critically examined and the governance model of the project adjusted to ensure that the regulatory responsibilities of the individual regulator and his/her agency are not compromised by the participation in the project (see paragraph on Governance structure). It is also important that all participants in the project are aware of the potential issues and adhere to the predefined roles and the terms of involvement and do not attempt to reach out to the participant regulator in his/her formal regulatory capacity but rather they seek regulatory advice through the official routes.

Regulators can further contribute to IMI2 projects by providing their regulatory expertise, e.g. in the evaluation of the specific public health needs, by providing regulatory advice, in identifying implementation hurdles at an early stage, or in the development of new regulatory solutions as well as in supporting global harmonisation of novel regulatory procedures. Importantly, this role is much closer to their formal duties and responsibilities. In the EU, this participation can be organised through formal procedures such as regulatory scientific advice at a national competent agency (NCA) or the European Medicines Agency or other regulatory bodies, including foreign regulatory agencies, that may be relevant for specific project assets. When formal regulatory advice is sought, it is preferable that the regulators providing advice are not partners in the project. Additionally, regulatory expertise can be provided as an in-kind contribution by regulatory experts from EFPIA or by independent regulatory consultants. This support can facilitate the development of a regulatory concept and the initial exchange with the authorities and the preparation of the relevant documents.

However, if the project itself deals with regulatory questions and the scope is to develop new regulatory pathways, the participating regulator again is involved as a scientist. He/she will investigate different possibilities and develop model procedures but this does not require his/her acting in his formal regulatory function and the regulatory involvement at this stage does not render subsequent formal regulatory procedures of regulatory authorities unnecessary. To avoid the conflict of interest, it should be ensured that the participating regulator in the project is not (or not alone) the final decision maker in the subsequent regulatory procedures.

It is very important to be aware of the fact that it can be the scientific goal of a research project to evaluate different regulatory solutions using research tools. However, it should not be expected that regulatory implementation can be pursued within the project. Regulatory implementation remains the domain of the regulatory bodies involved; they will respond to and act according to the current research knowledge, which can arise from an IMI2 project. This highlights that it is essential that regulators are well informed about the project results that are relevant from a regulatory perspective and potentially require change in the regulatory pathways and framework. The formal processes, however, go far beyond the scope of a research project and will ensure independent decision making of the authorities.

Lastly, participation of regulatory staff as individuals or as representatives of their agency in a scientific advisory board of an IMI2 (PPP) project can also result in a conflict of interest. Generally, the work in an advisory board is ill-defined, because it strongly depends on the evolvement of the project, which is not always foreseeable. To be more transparent regarding potential conflicts of interest, some projects have established separate scientific and regulatory advisory boards. Notably, a conflict of interest arises when providing advice on regulatory matters becomes prejudicial to the formal scientific or methodological advice provided by the agency of provenance or the regulator himself is biased because the matter involves his/her specific regulatory responsibility. For this reason, some NCA are restrictive in supporting this type of involvement.

4. Participation of regulatory agencies in IMI2 (PPP) (Recommendation 5)

The regulatory agencies with expertise relevant to IMI2 (PPP) projects should play an active role in projects where they can provide specific expertise complementary to that of other participants. The participation of the regulatory agencies should be incentivised to participate because in many circumstances regulatory agencies can have a preparatory role and later help to pick up speed in both translation and implementation of new developments.

An important prerequisite for their engagement is that the agencies need to clearly define the circumstances that enable or limit their capacity to join IMI2 projects. To avoid any negative impact of their participation in a project on the performance of their regulatory duties, the regulatory agencies with personnel whose scientific and/or regulatory expertise falls into the project area, need to clearly define, which tasks are feasible, and where a conflict of interest could arise. In terms of participation of their staff it is helpful if the regulatory agencies develop guidance and terms on participation in IMI2 (PPP) and provide legal advice on a case-by-case basis to support the decision making of their staff on participation. If the expertise of the regulatory experts is found to be important for success of the project, the regulatory agency may give advice on which type of governance structure could be established to avoid any potential conflicts of interest. On the NCA level, the process of developing guidance on governance structures that enable the participation of regulators could be actively pursued in the recently established EU innovation network.

The EMA has published two documents [11][12] that on the EMA perspective on engagement of the EMA as a regulatory body in externally funded research projects such as those executed under IMI2. These documents can serve as an excellent basis for similar decision making on conflict of interest scenarios and on how to define where the participation of regulators or regulatory agencies in a project is warranted and beneficial.

A key asset for participation of regulators in IMI2 projects is that the governance structure of the individual projects can be designed to avoid conflict-of-interest. Although IMI2 research topics have rarely been categorised as “regulatory research”, many IMI2 projects have explicitly covered this area and have successfully involved regulators and regulatory agencies. It is further important that the governance structures of the projects take into account that potential conflicts of interest can impede collaboration, before it is even sought. The establishment of project governance models that turn IMI2 into the often cited “neutral broker” that enables collaboration of stakeholders with competing interests within a PPP is a special achievement of IMI and is partially based on the dedicated establishment of these governance structures.

5. Role of regulatory affairs experts and independent consultants (Recommendation 1 and 4)

In pharmaceutical companies the regulatory affairs teams form the counterpart to the governmental regulators. Both meet and act at the interface of the private and public sectors and are supposed to represent the respective interests. The industrial representatives are responsible for managing of the administrative process leading to the authorisation of novel products and for developing concepts for deployment and implementation in health care. Thus, regulatory affairs experts from companies are often well acquainted with the regulatory procedures and the potential hurdles on a global level. They can provide

valuable input before and during the projects. Regulatory affairs experts from EFPIA companies could, thus, provide support at the topic description stage, in the preparation of full proposals (stage 2) and during project execution. The latter can be provided as an in-kind contribution.

As an alternative option, independent regulatory consultants can be contracted by the consortia or become project partners (beneficiaries) providing support in developing a regulatory strategy for the project, mediating the necessary contacts and the providing support in the preparation of the necessary dossiers.

6. IMI Projects with regulatory relevance

Table 1: **Regulatory aspects of IMI projects.** The table highlights a few examples of projects that involved regulators to seek for new regulatory solutions engaged in regulatory procedures enabling implementation of specific project assets or addressed specific regulatory research questions. Some of them also adopted a project governance structure, which helped to avoid conflicts of interest:

IMI Project	Regulatory relevance	Governance, Stakeholder involvement, Regulatory application
ADVANCE	Benefit/risk assessment of vaccines	This consortium developed a code of conduct for studies and new governance models to ensure transparency in regards to conflict of interest and credibility of outputs (13, 14)
ADAPT SMART	The project dealt with adaptive licensing. It focussed on the need to gain acceptance for this new regulatory pathway from regulators and HTA.	The project enabled multi-stakeholder involvement including regulators from EMA and NCAs, HTA bodies (NICE, ZIN and HAS) and payers.
BioVacSafe	Use of new biomarkers and technologies to develop new ways to identify, classify and record adverse reactions to vaccines	"Regulatory Core" group with expertise to generate and disseminate classifications, guidelines, reference standards for vaccine development
ConcePTION	Development of new concepts on how to retrieve trusted information on safety of medicines during pregnancy and lactation	Direct regulatory application of outputs
DRIVE	Influenza vaccine effectiveness data represent a regulatory requirement but obtaining these data is difficult	The project is developing and testing methodology to fulfil the regulatory requirements; a particular challenge was the creation of a governance model that secures public oversight and excludes industry-bias [15]
DRIVE-AB	Market incentives and value assessment for reimbursement decision-making in an area viewed as non-profitable because of low return for investment	Involvement of HTAs in the consortium was sought but not successful due to the perceived conflict-of-interest; their engagement in the project was as stakeholders.
EbolaMoDRAD and EBOLA+	Development of rapid diagnostics for Ebola	Regulatory coherence needed for use in clinical trials and lab routine
PREFER	A project studying when to employ patients´ preference studies to facilitate decision making by regulators and HTA bodies	HTA bodies involved; regulatory stakeholder advisory group to represent regulatory perspective
PRO-active	The project developed an innovative tool for measuring physical activity in patients with COPD. This device was qualified by the EMA and can now be used in clinical trials.	Qualification opinion from EMA [16]
VAC2VAC	3R project on substituting of animal experiments in QC testing and batch	Several Official Medicines Control Laboratories (OMCL) and EDQM involved

	release of vaccines by in vitro assays	
WEB-RADR	Improvement of pharmacovigilance by electronic solutions (App) for drug monitoring.	Regulatory approval required
ZAPI	Regulatory framework for platform technologies for vaccine development against emerging infections	Guidance is needed for rapid development of new vaccines on established platforms. This could include CMC master files for a platform technology and guidance on safety and efficacy study requirements. Presentations at different NCAs

7. Areas for regulatory research

The Scientific Committee supports regulatory research projects in areas where there is a public health need or where access of patients to innovative therapies can be facilitated by this research. The IMI Scientific Committee members have identified areas that would benefit from regulatory research:

- Clinical trial design and stratification instruments in precision medicine
- Innovative technologies for rapid development of medicines and diagnostics, e.g. meeting urgent clinical needs in epidemic and pandemic settings
- Validation concepts for continuously evolving digital devices
- Bacteriophage therapy with individualized patient-specific compositions
- Tools for identification of areas for drug repurposing and innovative clinical trial designs facilitating evidence generation
- Quality requirements for big data analysis and artificial intelligence tools
- Data sharing practices and identification of areas where data sharing is needed

Of note, this list is not exclusive. However, with these examples, the Scientific Committee would like to raise awareness for the need for this type of research. Nevertheless, many other fields in research and development may require or benefit from regulatory research.

On behalf of the Scientific Committee

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