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**COMMISSION STAFF WORKING DOCUMENT**

**Executive summary of the impact assessment**
**accompanying the document**
**Proposal for a**
**COUNCIL REGULATION**
**on the Innovative Medicines Initiative 2 Joint Undertaking**

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**EXECUTIVE SUMMARY OF THE IMPACT ASSESSMENT**
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**COUNCIL REGULATION**
**on the Innovative Medicines Initiative 2 Joint Undertaking**

This document summarises the impact assessment for the Joint Technology Initiative (JTI) on innovative medicines (IMI), established as a joint undertaking under the 7th Research Framework Programme. The proposal has been produced in the context of the Union's Multiannual Financial Framework (2014-2020), and will contribute to the implementation of the next EU Framework Programme for Research and Innovation, Horizon 2020.

## 1. PROBLEM DEFINITION

### 1.1. The problem that requires action

An ageing population increases the burden of chronic and degenerative diseases, putting additional pressure on health and care systems at a time of stretched public finances. Effective measures represent a significant part of the solution. However, the role of research and development (R&D) in developing therapies is declining, incentives for some classes of therapy (e.g. antibiotics) are all but absent and structural issues stand in the way of multidisciplinary cooperation, required to solve complex scientific problems that are characteristic of this field. Failure to act is neither in the interest of European public health nor of European competitiveness.

The process of developing therapies is costly, involving many tests before marketing approval can be given. These tests often demonstrate that the therapy in question is unsuitable and thus the investment is lost. This creates an incentive for manufacturers to invest in developing therapies which have a greater chance of success, either because they resemble existing therapies or because the potential return is very high. While this is a sensible business decision, it is not necessarily in the general interest of the EU citizen.

### 1.2. Key problem drivers

The relatively low investment in the biotechnology sector (*vis-à-vis* competitor regions) combined with the fragmented, closed innovation model of drug development in Europe and the complexity of the process act as a disincentive to risk taking by industry. The nature of the scientific challenges is such that data must be shared among various stakeholders. Without a framework enabling this to happen in a controlled environment, cooperation will not take place.

### 1.3. Need for public intervention

A controlled environment will not evolve naturally in the commercial environment, nor can it be achieved by the public sector alone. It can only come about through public cooperation, where the various players (academia, industry, SMEs, clinicians, regulators and patients) share resources, data and expertise, while ensuring that the fruits of their collaboration are shared, risks and costs reduced and productivity increased. The creation of such a risk-sharing environment will reduce the failure rate and those carrying out tests will have a greater incentive to test a wider variety of therapies to the benefit of all concerned, both in terms of promoting public health and legitimately protecting commercial interests.

### 1.4. The EU's right to act and the application of the subsidiarity principle

The right of the EU to act in this field is provided under Article 187 of the Treaty on the Functioning of the EU, authorising the setting up of 'joint undertakings or any other structure necessary for the efficient execution of Union research, technological development and demonstration programmes'.

*1.4.1. The required public intervention can only be provided at European level*

Measures at EU level to support trans-national and cross-sector cooperation between firms on strategic research agendas will help to establish 'critical mass', in particular through joint agenda setting, mobilisation of additional funding and increased leverage on industrial R&D investment.

*1.4.2. Investing at EU level can produce savings in healthcare costs and services*

The research programme will lead to a better classification of diseases, significantly improving diagnoses and treatments. This will prevent patients' unnecessary exposure to the adverse effects of ineffective treatments during clinical development or medical practice. In the latter case, savings have been achieved by discontinuing an ineffective or inappropriate treatment. For example, an analysis carried out in France has demonstrated the monetary benefit of molecular diagnosis of cancer patients. Investing €1.7 million in molecular diagnosis has resulted in savings of €34 million by not administering the cancer drug Iressa® to patients for whom it is ineffective. Even bigger savings can be expected from the classification of chronic diseases.

### 1.5. The achievements of the current IMI

The IMI Joint Undertaking has produced a number of important results:

- considerable leverage effect on industrial R&D investment by virtue of a €1 bn contribution from the European Commission and a contribution in kind of €1 bn from the European Federation of Pharmaceutical Industries and Associations (EFPIA);
- enhanced cooperation — the IMI Joint Undertaking brings together large-scale industry, SMEs and research organisations from across the European Union;
- joint production of comprehensive strategic research agendas and coordination of other policies due to the involvement of patient organisations and regulatory bodies;
- an open innovation model — the IMI Joint Undertaking has contributed to the transition from a closed to an open model of innovation in biomedical and pharmaceutical research.

### 1.6. The lessons learnt from IMI

Despite these achievements, the implementation of IMI and the 2011 interim evaluation have revealed a number of shortcomings:

- the legal instruments used for setting up the JTIs, and in particular their status as Union bodies, need to be made more flexible;
- the participation rules applied to/by JTI joint undertakings, in reflecting the needs of the various partners, add to the complexity of the initiative;
- monitoring and evaluating achievement of the targets included in the strategic research agenda and technical work plans need to be improved;
- horizontal policy coordination needs to be strengthened (e.g. the advisory potential of the European Medicines Agency (EMA) should be fully exploited);

- internal and external communication needs to be strengthened.

The shortcomings identified stem from the initial design and constitute a starting point for improving the design of the IMI Joint Undertaking, under Horizon 2020.

## 2. OBJECTIVES

The general and specific objectives that have been identified are based on the outcomes of the public consultation, the problems and drivers and the achievements and lessons learnt from IMI.

### 2.1. Overall objectives

The overall objective is to improve European citizens´ health and wellbeing by providing new and more effective diagnostics and treatments while helping safeguard the future international competitiveness of the European biopharmaceutical and life science industries such as diagnostics, vaccines, biomedical imaging and medical information technologies. The IMi2 Joint Undertaking will implement Horizon 2020 objectives, in particular as defined in the Health, demographic change and wellbeing societal challenge, and will address the public health challenges identified in the World Health Organisation report on priority medicines for Europe and the World.

### 2.2. Operational objectives

The operational objectives of this initiative are to:

- provide structures that facilitate partnerships along the entire life science research and innovation cycle, such as from early discovery to product development, to pharmacovigilance research and surveillance, in an effective innovation-driven collaborative setting that is focused on optimising life sciences research and innovation for diagnostics, prevention and therapeutic agents and approaches, and support for the development of evidence-based regulation;
- establish networks for open innovation along the whole innovation cycle of novel medical research and technologies, bringing public research institutions, academia, life science industries, SMEs, patient organisations, regulators, payers, public health authorities and the animal health sector;
- reduce the fragmentation of research and innovation and increase the level of private-sector spending in Europe;
- develop and implement strategic agenda setting in a pan-European structure with the necessary critical mass and budget, ensuring continuity and allowing life science industries to make long term investment plans;
- facilitate research that provides evidence earlier in the drug and vaccine development process through risk-sharing mechanisms.

### 2.3. Specific objectives

The specificl objectives are to:

- improve by 2020 the success rate in clinical trials by 30% in diseases identified in the ‘Priority Medicines for Europe and the World WHO Report’;
- reduce to five years the time taken to reach clinical proof of concept in immunological, respiratory, neurological and neurodegenerative diseases;

- develop at least two new therapies for diseases for which there is a high, unmet need and limited market incentives: antimicrobial resistance (two new classes in the past 30 years) or Alzheimer's disease (only two treatments of limited efficacy have ever been developed);
- develop diagnostic markers for four diseases (among those mentioned above), clearly linked to clinical relevance and approved by regulators;
- develop a transparent and comprehensive infrastructure model to gather data on disease incidence and the medico- and socio-economic burden of major infectious diseases;
- develop tested novel biomarkers to predict vaccine efficacy and safety (two markers each) early on in the process, to improve multiple-candidate screening to achieve a 50% reduction in the failure rate in phase III clinical trials;
- develop two novel adjuvants for human use to increase the body's immune response to vaccines, boosting in particular reaction in specific target groups such as the elderly and non-responders;
- identify, for two major infectious diseases and two types of cancer or chronic disorder (e.g. autoimmune diseases), at least: two novel predictive models for efficacy and two novel predictive models for safety;
- strengthen the link between human and veterinary vaccine research.

## 3. POLICY OPTIONS

The impact assessment considered four main policy options:

1. Business-as-usual: continuation of the current IMI JTI under Horizon 2020, managed by the Joint Undertaking. Under this option, IMI remains focused on building a collaborative system for biomedical R&D in Europe and speeding up the development of effective and safer medicines for patients.

2. No public-private partnership (PPP) or 'zero option': use of Horizon 2020 collaborative projects only. This option facilitates the formulation of common objectives at project level but does not accommodate the formulation of cross-project execution of strategic agendas. Industry participation takes place on a project-by-project basis.

3. Contractual PPP to implement Horizon 2020 actions falling under the 'Health, demographic change and wellbeing' societal challenge. Under this option, an industry partnership agreement is concluded and industry proposes a strategy and advises on work programmes. Whilst EU commitment and contribution is set at the launch of PPP, financing amounts and topics are subject to approval under an annual work programme.

4. Modernised JTI: expands the objectives and activities of the IMI Joint Undertaking in line with Horizon 2020 objectives; broadens the scope of the current programme and improves its governance.

## 4. ASSESSMENT OF IMPACTS AND COMPARISON OF OPTIONS

The four policy options were compared on a range of key parameters, assessing public involvement in life sciences research and innovation.

The outcome of this comparison is that the 'Modernised JTI' option is the preferred option. It achieves critical mass at programme and project level; fosters scientific excellence in biopharmaceutical and life science research, which impacts on innovation and is enhanced through financial support from scientific ideas to the market, a stronger output orientation and better dissemination of research results; greater scientific and innovation impact translates into larger downstream economic, competitiveness, social and public health impacts; and allows for more flexibility and reduced administrative costs for applicants and participants, and publically funded entities, such as academic and SMEs, benefit from administrative simplification. This option also maximises cost-effectiveness.

The case of the 'zero option' makes the formulation of cross-project execution of strategic agendas difficult. Critical mass is compromised and the level of flexibility, accessibility and broader horizontal policy coordination is lower than with the 'Modernised JTI' option. This would translate into smaller economic, competitiveness, social and public health impacts.

The 'Contractual PPP' option accommodates the formulation of cross-project execution of strategic agendas, but it constitutes a 'light' approach to a public-private partnership, with an indicative budget only and a rather limited commitment from industry.

***Summary comparison of options (impact compared with the BAU scenario)***

| | No PPP | PPP | Modernised JTI |
|---|---|---|---|
| Public health impacts | -- | - | +++ |
| Social impacts | -- | - | ++ |
| Economic and competitiveness impacts | - | - | ++ |
| Innovation impacts | -- | - | ++ |
| Critical mass of resources | -- | - | + |
| Leverage effect (overall R&I resource mobilisation) | -- | - | = |
| Participation of industry and SMEs | -- | - | ++ |
| Strategic agenda | -- | - | + |
| Addressing fragmentation | - | - | ++ |
| Administrative cost and efficiency of governance | - | -- | = |
| Coherence | = | = | ++ |
| Efficiency | -- | = | ++ |
| Effectiveness | -- | = | ++ |

## 5. MONITORING AND EVALUATION

An appropriate monitoring and evaluation system set up at programme and project level, including a set of approved key performance indicators, will enable an assessment to be made of whether the IMI2 Joint Undertaking is achieving its objectives, with the Governing Board overseeing the work of the Executive Director and the Programme Office.

The external evaluation for the entire programme will be organised by the Commission. An interim evaluation will be carried out before the end of 2017 and a final evaluation after the conclusion of the programme in 2024.