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Accelerating Clinical Trials in the European Union (ACT EU)

Priority Action 3 concept paper: an EU multi-stakeholder platform for improving clinical trials

Sent for comments to ACT EU co-leads	June 2022
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The aim of this concept paper is to provide an outline of the ACT EU multi-stakeholder platform.

This paper describes a proposal for the creation of a multi-stakeholder platform (MSP) that aims to promote dialogue and collaboration for improving clinical trials in the EU. The success of clinical trials relies on a multitude of stakeholders. The creation of a common platform will provide further opportunities for interactions between stakeholders, therefore promoting a shared understanding of the respective roles, needs and perspectives. This is ultimately expected to increase collaboration and build mutual trust, thus improving the EU clinical trials landscape for the benefit of innovation and all European citizens.

Background

For the clinical trial environment to evolve with advances in regulation, methodologies, technology and science, there is a need for multi-stakeholder discussions to drive and support change. This was reflected in stakeholder responses to the consultations for EMA's Regulatory Science Strategy to 2025, the European Medicines Agencies Network (EMRN) strategy to 2025 and the European Commission's Pharmaceutical Strategy. The Regulatory Science Strategy to 2025 included the recommendation to 'establish a multi-stakeholder, neutral, platform, to enable new approaches to clinical studies and to position the EU as a preferred location for innovative clinical research'. On this basis, establishing a multi-stakeholder platform is listed in EMA's multi-annual work programme 2021-2023. The European Commission, HMA and EMA Management Board have endorsed the establishment of Accelerating Clinical Trials in the EU (ACT EU), which includes the Priority Action (PA) to establish a multi-stakeholder platform.

Innovation in clinical trials will help to demonstrate the effects of medicines. Innovation may come through the use of novel trial designs, endpoints, techniques for gathering data, use of 'omics' to stratify populations, real world data to generate comparator groups or follow up, for example. The experience with the COVID-19 pandemic has clearly demonstrated the need to accelerate change and innovation in the way that clinical trials are designed, regulated and conducted to maximise their efficiency and utility to patient access to treatments.

It is therefore crucial to ensure that the perspective of all stakeholders is included in the platform in order to address the objectives of ACT EU.

Proposal for a multi-stakeholder platform

Scope

The scope of the platform will encompass all aspects of clinical trials including design, conduct, statistical analysis, proposal of revision of regulation(s), transparency of data and patient engagement.

Objectives of a multi-stakeholder platform

As outlined in Figure 1, MSP objectives are to:

- accelerate change and innovation in the approach to how EU clinical trials are regulated, designed, conducted and evaluated, to maximise efficiency and value to patients and citizens:
  - enable and support related projects and their wider implementation;
  - identify, discuss and address challenges linked to the implementation of new legislation, guidance and methodologies;
- bring together key stakeholders in a neutral forum for regular, and balanced discussions;
- build trust through a better understanding of the perspectives and roles of different stakeholders and thereby open avenues and drive change;
- identify training/capacity building needs;
- encourage and inform change across scientific, operational, legal and regulatory areas;
- ensure transparency and sharing of discussions outcomes.

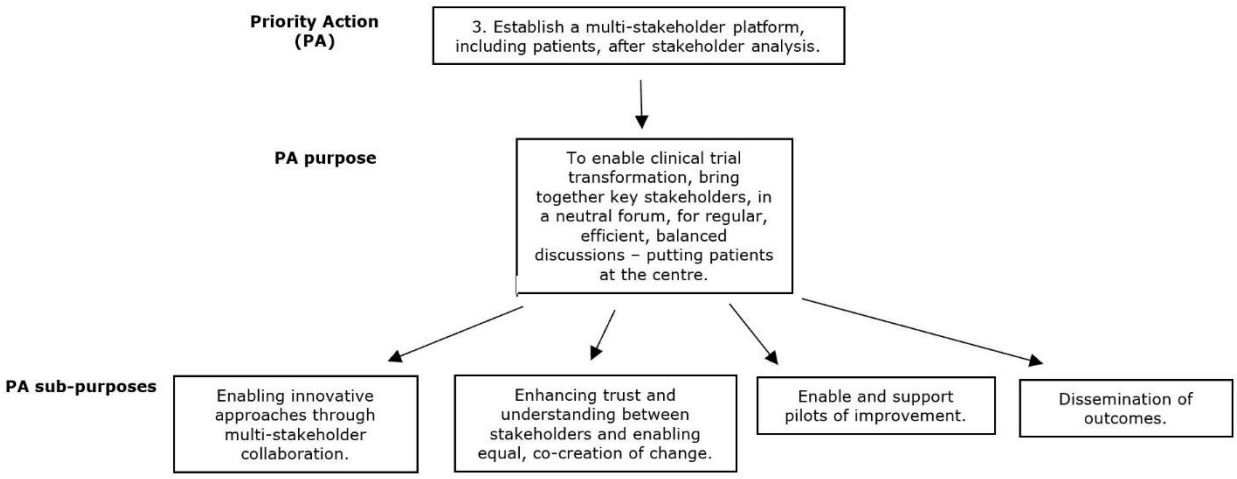

Figure 1: Objective breakdown structure indicating the overarching aim and sub-objectives

MSP composition

The composition of the MSP will be agreed following a public call for expressions of interest. Representatives of interested organisations from key stakeholder groups will then be invited to become members of the platform. Additional participants can be invited to attend the meetings depending on the topic under discussion.

Key stakeholders include:

- Patients and patient organisations
- Healthcare professionals (HCP) and HCP organisations
- Academics as users of clinical trial data
- Clinical trial investigators
- Clinical Research Organisations (CRO) and other clinical trial service providers, including consultants
- Sponsors, incorporating academia and pharmaceutical companies, notably small and medium-sized enterprises (SMEs)
- Ethicists and ethics committee members
- Research funders
- Regulators: medicines approval regulators, clinical trial assessors, safety (pharmacovigilance in clinical trials) assessors, clinical development advisors, and medical device bodies
- Inspectorates
- Health technology assessment (HTA) bodies
- Payers
- Policy makers

Once the platform is established, additional stakeholders (e.g., international partner organisations and authorities) could be considered, as observers.

MSP organisational aspects

The MSP reports to the ACT EU Steering Group[1] and is expected to meet quarterly (or when required) via virtual/hybrid meetings. In addition, *ad hoc* topic groups can be formed for technical discussions and convened as needed. The *ad hoc* topic groups are expected to be composed by relevant experts selected amongst the MSP members. If needed, additional external experts can be invited as appropriate. Each *ad hoc* topic group will have one rapporteur responsible for coordinating, leading and reporting on the work of the group. The activities of MSP and of the *ad hoc* topic groups are outlined in Figure 2.

[1] The group is composed of a chair (provided by the EC) and includes up to five nominated members from the Heads of Medicines Agencies (HMA), two members from the European Commission (EC) and two members from EMA. In addition, membership will include the chairs of the EMA Management Board, Clinical Trials Coordination Group, Committee for Medicinal Products for Human Use (CHMP) and a representative from the Network Portfolio Advisory Group (NPAG).

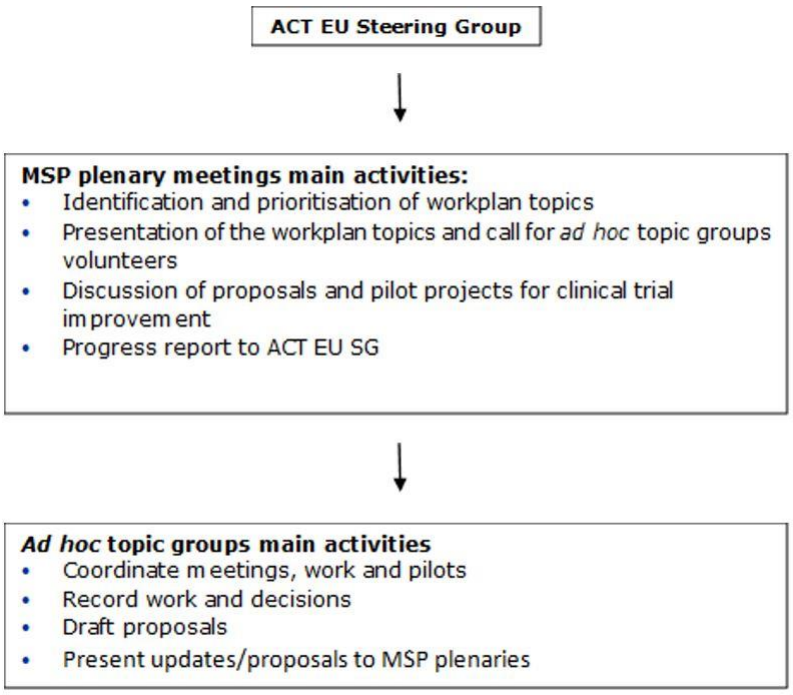

Figure 2: Organisation of topics for discussion at MSP and ad hoc topic groups

MSP governance structure

The MSP will work through plenary and *ad hoc* topic group meetings and will report to the **ACT EU Steering Group** who will be responsible for overseeing the establishment and high-level work planning of the platform.

The organisational aspects of the platform and of the *ad hoc* topic groups will be supported by an established secretariat. The MSP secretariat will initially comprise network resources followed by the identification of a sustainable model.

The **MSP secretariat is expected to be responsible for:**

- Liaising with ACT EU Steering Group and MSP
- Agenda setting
- Support drafting of workplan and operational documents
- MSP and *ad hoc* topics groups operational support including progress monitoring
- Managing MSP composition and related declaration of interest
- Dissemination of meeting highlights and MSP outputs

Transparency

MSP agendas, highlights and key outputs are expected to be published in respect of confidentiality.

Platform operation and phased approach

The work of the MSP is proposed to be implemented gradually through a series of workshops allowing stakeholders to familiarise themselves with the MSP proposal, agree on the workplan priorities in addition to fine tuning group operational aspects. The following staggered approach is proposed:

1. Implementation Q1/Q2 2023

An initial stakeholder consultation on MSP design, priorities for discussions and interest in being part of the platform will be followed by a first workshop where the aims will be to:

- Present the MSP governance proposal.
- Agree MSP preliminary workplan based on the feedback received from the initial consultation and start discussion on priority topics.

2. Implementation 2023-2024

The MSP will keep working on the priority topics and will finalise its operating model based on the experience gained.

3. Implementation from 2025 onwards

As of 2025 onwards, it is expected that the ACT EU MSP will transition to a more sustainable resourcing model.

Meeting frequency

- Plenary meetings: anticipated 2-4 times per year.
- *Ad hoc* topic group meetings: as needed.

The platform will mainly meet virtually with the possibility of hybrid plenary meetings as needed.

Evaluation criteria

The work of the MSP will be evaluated against pre-defined key performance indicators (KPI) that will be drafted as part of governance documents and agreed with MSP members.