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European Medicines Agency

Outcome of public consultation on ACT EU multi-stakeholder platform (ACT EU MSP) participation and priorities for discussion

1. Summary of findings and next steps

The public stakeholder consultation was conducted over 4 weeks (3rd February 2023-3rd March 2023) in order to obtain feedback on interest in being part of the multi-stakeholder platform, priorities to be included in MSP workplan and comments on the proposed ACT EU MSP concept paper.

A total of 230 valid responses were received from multiple stakeholders, with the greatest number of responses coming from Sponsors, Clinical Research Organisations (CROs) and Healthcare Professionals.

In terms of priorities for the MSP discussions, many responses related to ensuring adequate and harmonised implementation of legislations (CTR[1], MDR[2], IVDR[3]); updating methodologies and guidance in order to foster innovation and efficiency, with particular focus on special areas such as rare diseases, paediatrics and oncology; facilitating digital health at national and international level; and ensuring adequate regulatory support for evidence generation and Clinical Trial (CT) assessment outcome predictability.

The design of the MSP was generally supported by stakeholders. Additional details were requested on composition, organisational aspects, and objectives, and particular attention was requested to ensure adequate multi-stakeholder representation.

A total of 179 responders expressed the interest in being part of the MSP. The feedback received is expected to be presented and discussed at the ACT EU multi-stakeholder platform kick off workshop currently scheduled for the 22nd and 23rd of June 2023. The MSP formal composition, operational documents and workplan will be finalised thereafter.

2. Introduction

The EC-HMA-EMA initiative Accelerating Clinical Trials in the EU (ACT EU), launched on 13 January 2022, acknowledges that the success of clinical trials relies on a multitude of stakeholders. Regular

[1] Clinical Trials Regulation (Regulation (EU) 536/2014)
[2] Medical Device Regulation (Regulation (EU) 2017/745)
[3] In Vitro Diagnostic Regulation (Regulation (EU) 2017/746)

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dialogue between these stakeholders enables advances in clinical trial methods, technology, and science as well as the identification of roadblocks and solutions to overcome them.

ACT EU outlines a set of 10 priority actions (ACT EU 2022-2026 workplan), with a fundamental action being the establishment of a multi-stakeholder platform.

To initiate the platform and define its workplan, a public stakeholder consultation was launched from the 3rd of February 2023 to the 3rd of March 2023 with the aim of gathering stakeholders' feedback on:

- interest in being part of the multi-stakeholder platform;
- priorities to be included in MSP workplan;
- comments on the proposed ACT EU multi-stakeholder platform concept paper (the design of the platform).

This report is a summary of the feedback received. A total of 232 responses were submitted, of these 230 were considered valid responses while 2 were left blank and therefore excluded.

3. Results

3.1. Affiliation

Participants were asked to indicate their affiliation by choosing from a pre-defined set of options. Where none of the provided options was suitable, it was possible to select 'others' and provide further details. If the additional details provided allowed for respondents to be placed in more informative category, this was done. In addition, to facilitate a graphical representation of the results, affiliation categories were either grouped and/or abbreviated as shown in Table 1.

Table 1. List of affiliations used in the public consultation (a) and grouped/abbreviated affiliations (b) used for the graphical representation of results.

Original affiliation category (a)	Amended affiliation category (b)
Academics as users of clinical trial data	Academics (CT data users)
Clinical Research Organisations (CRO) and other clinical trial service providers, including consultants	CROs
Ethicists and ethics committee members	Ethics Committees
Healthcare professionals (HCP) and HCP organisations	Healthcare professionals
Clinical Trial Investigators	Clinical Trial Investigators
Health technology assessment (HTA) bodies	HTA bodies
Inspectorates	Regulators/ Inspectorates
Patients and patient organisations	Patients
Payers	Payers

Original affiliation category (a)	Amended affiliation category (b)
Policy makers	Policy makers
Regulators: medicines approval regulators, clinical trial assessors, (Pharmacovigilance in clinical trials) assessors, clinical development advisors, and medical device bodies	Regulators/ Inspectorates
Research funders	Research funders
Sponsors, incorporating academia and pharmaceutical companies, notably small and medium-sized enterprises (SMEs)	Sponsors (commercial and non-commercial)
Other	Other

The majority of contributions were received from Sponsors, followed by CROs and Healthcare Professionals (figure 1). Responders identified as "others" were not otherwise classifiable; this group contained a mix of available affiliations or altogether different categories (e.g. private/public consortium, citizens, non-profit organisation, etc) which were not included in the survey list of affiliations nevertheless their feedback was also taken into account.

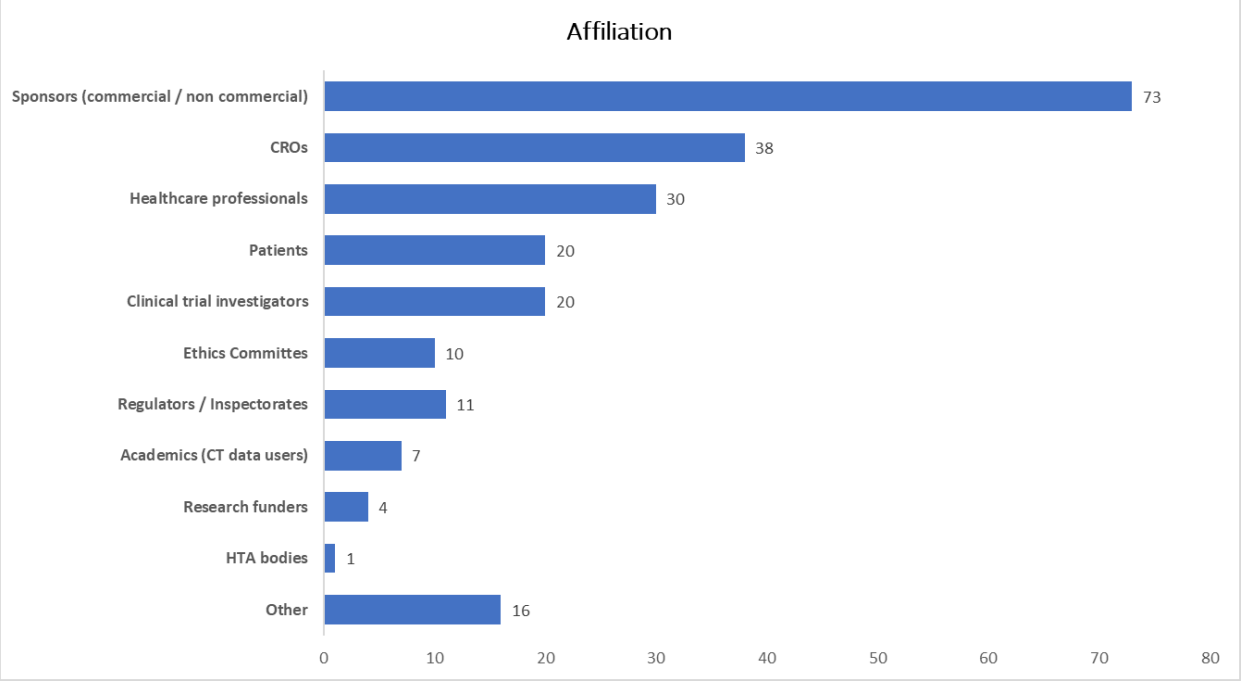

Figure 1. Number of responders to the public consultation divided per affiliation

3.2. Priority topic selection

Participants were given a list of 6 topics (see table 2) linked to ACT EU Priority Actions (PAs) and were asked to select the 3 areas they would prioritise in the multi-stakeholder platform discussions. For each selection, the option to provide further comments in the free text field was available.

Table 2. List of topics proposed in the public consultation (a) and the shortened version (b) used for the graphical representation of results.

Original text (a)	Shortened text (b)
The successful and timely implementation of the Clinical Trials Regulation (CTR) and its implementing acts.	CTR implementation
Good Clinical Practices (GCP) modernisation informed by the revision of ICH guidance.	GCP modernisation
The analysis of clinical trial data to support policymaking, funding on research outputs, and to support evidence-based decision making.	CT data support for decision making
Need for methodologies guidance such as on Machine Learning/Artificial Intelligence impacted CTs, decentralised CTs and In Vitro Diagnostics Regulation/CTR interface (to strengthen links between innovation and scientific advice fora).	Methodologies guidance
Clinical trials training curriculum including modules on drug development and regulatory science with links to universities and SMEs (serving as an educational "ecosystem").	CT training curriculum
Regulatory support structures for evidence generation and enabling innovation.	Regulatory support for evidence generation

As shown in figures 2, stakeholders are of the opinion that the MSP should initially focus on:

- CTR implementation;
- methodologies guidance;
- regulatory support for evidence generation.

Moreover, priority topics selected by the participants to the public consultation are also presented divided per affiliation group in order to show the most important topics for each stakeholder (figure 3).

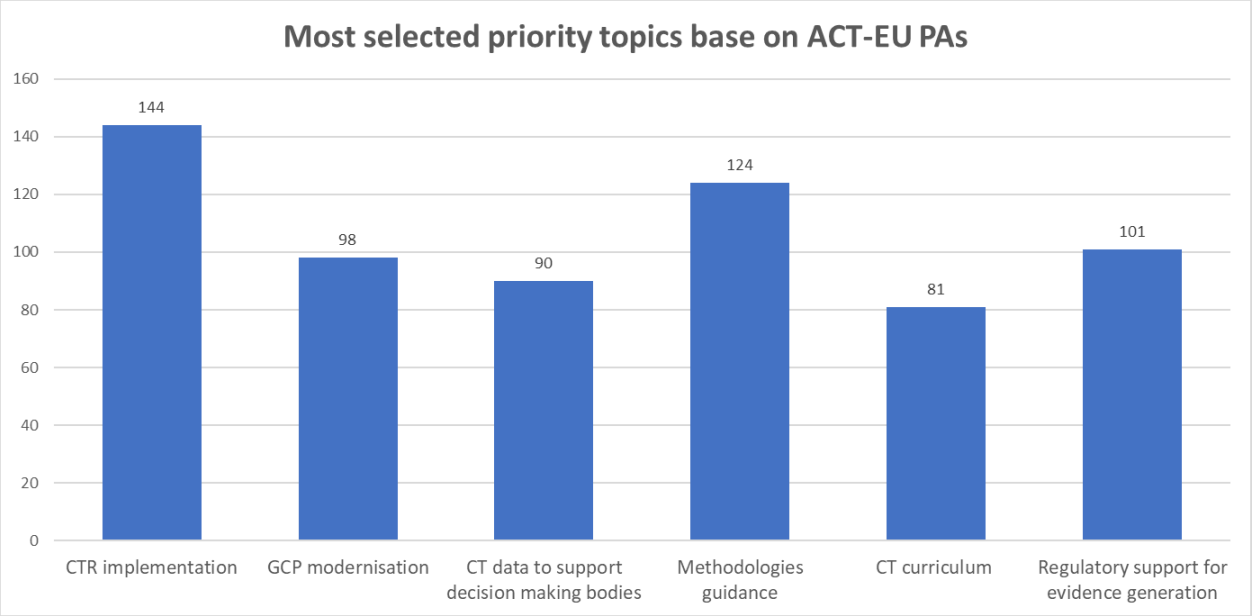

Figure 2. Priority topics selected by respondents to the public consultation.

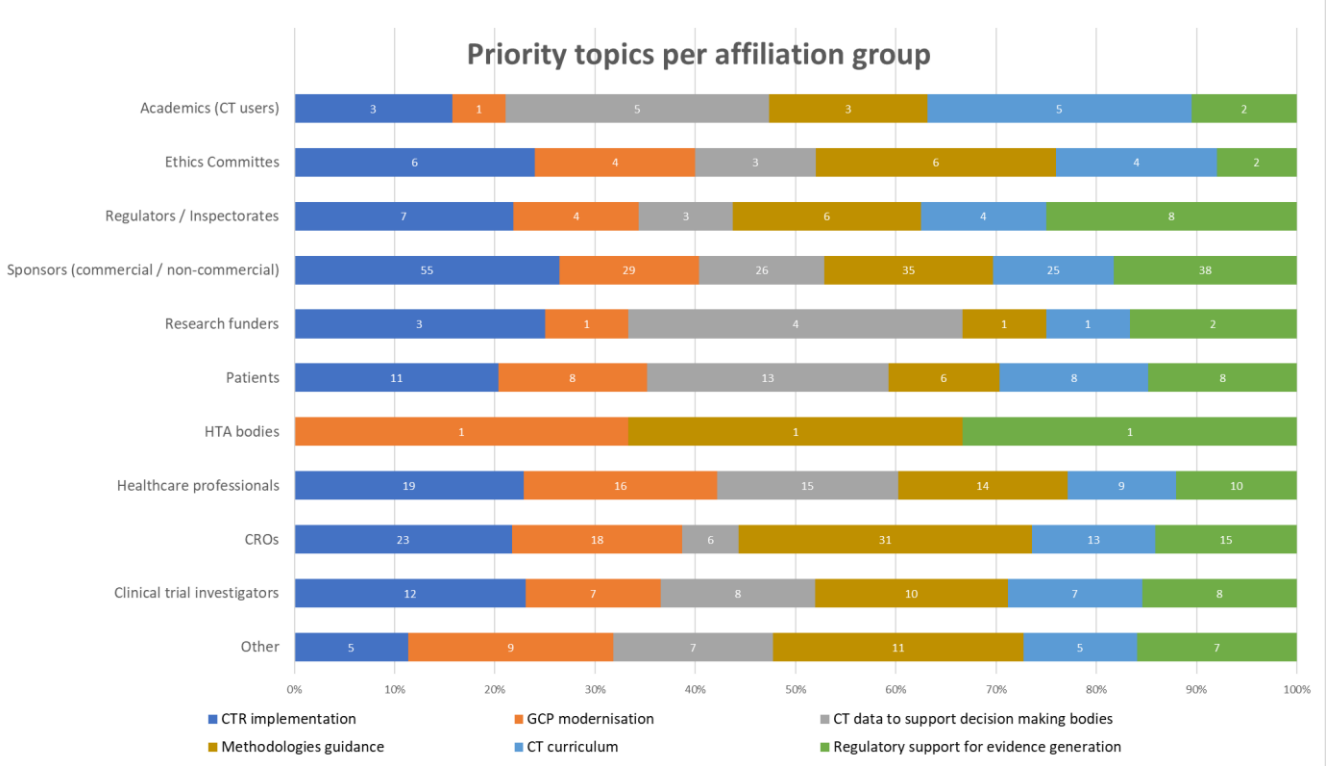

Figure 3. Priority topics selected by respondents to the public consultation divided by affiliation (percentage values group).

For each of the 3 topics selected, free text comments were received; a summary of the comments is reported below.

1. ***CTR implementation***

The successful and timely implementation of the CTR is considered vital to ACT EU's success. To achieve this, the harmonisation of requirements, and reduced administrative burdens and delays are needed. Successful CTR implementation will also require the optimization of the Clinical Trials Information System (CTIS). To achieve all this, cooperation and alignment among member states and ethics committees - in dialogue with stakeholders – will be necessary, the MSP is well placed to support this dialogue.

Additional points made:

- Need to keep the implementation of CTR up to date with modern trial designs and methods, particularly for trials in special populations.
- Clinical trial advice should be iterative, and this should be facilitated under the CTR.
- The focus should remain on the needs of trial participants but aid for implementation of the CTR should be given to academic sponsors, who lack the resources to navigate it and Member States (MSs) who lack the resources to implement it.
- Transparency requirements of the CTR should be enforced.

To implement the above, and the CTR in general, KPIs are required. These could also measure the competitiveness of the EU.

2. ***GCP modernisation***

Modernisation of Good Clinical Practices is considered crucial for clinical trials research in the EU. Its implementation must result in flexible, simplified and patient-centred requirements which enable new clinical trial methodologies and technologies. Paediatric trials are one area where guidance is needed. The work to implement GCP should ensure consistency across MS.

3. ***CT data to support decision making bodies***

According to stakeholders, data transparency and exchange is vital to improve decision-making and public health research. This is true in the EU, but it should also be fostered internationally via data exchange and the harmonisation of standards.

Data analysis by decision-makers should include the totality of evidence, such as real-world data (RWD) and extrapolated data. This is especially true for ultra-rare diseases where clinical trials are difficult.

To enhance data generation for decision-making, guidance, tools and training are needed on how to use these alternative forms of evidence, and on the incorporation of data from new sources such as wearables.

To incentivise improved decision-making itself, indicators should be developed to track regulatory agencies' approval performance.

4. ***Methodologies guidance***

In light of the speed of innovation, its implementation is only possible with adequate methodological guidance. This should include how to best integrate and share data from innovative trial designs, technologies, and methods, including, decentralised clinical trials, medical devices and artificial intelligence. Guidance should be developed through multi-stakeholder interaction, be patient-centred and harmonised across member states.

5. ***CT training curriculum***

The need to provide training to all staff involved in clinical trials was highlighted by several stakeholders. This includes hospital staff, academia, SMEs and patients. In addition, the training curriculum should include regulatory science, drug development, medical devices, innovative methodologies, and permit communication between all parties.

6. ***Regulatory support for evidence generation***

Stakeholders are of the opinion that regulatory support is essential (especially for academic trials) and should be available at all stages of evidence generation. Communication between with stakeholders and regulators should also be facilitated. In addition, focus is required on ensuring interlinks between Member States. Reinforced regulatory support on evidence generation, including exploring innovative ways to obtain data is flagged especially in areas such as paediatric trials, rare diseases, biosimilars and ATMPs.

3.3. Additional priority topics free text field

Participants were given the opportunity to propose additional priority topics other than those included in the previous question. In this case the comments received were also grouped into categories using the ACT EU PAs to facilitate the graphical representation of the results, as shown in figure 4; however, to remain faithful to the original comments proposed, summarized extracts are also reported in the sub-paragraphs below. Some of these additional topics overlap with the priority topics above in section 3.2. Due consideration will be given on how to address each specific proposal.

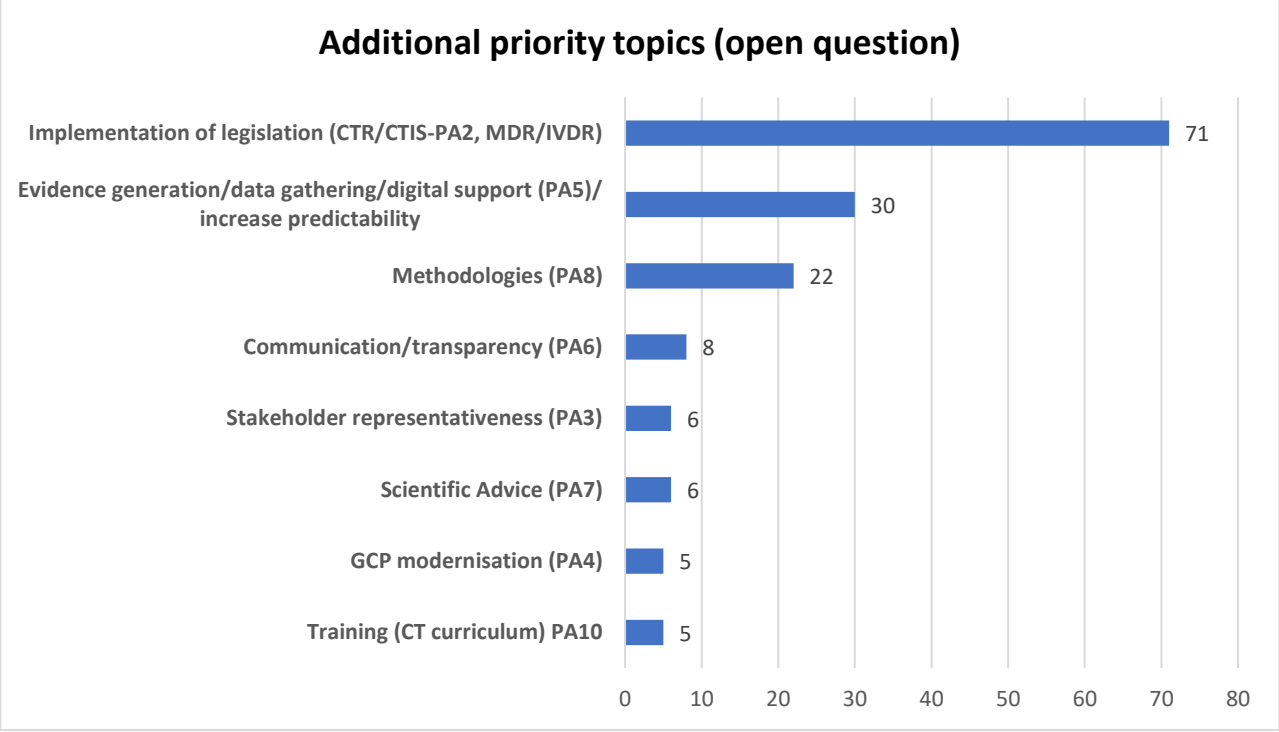

Figure 4. Additional priority topics for the MSP, as suggested by survey respondents. Responses were analysed and summarised in categories as shown.

From the feedback received the following topics are identified as a priority for the MSP discussion:

1. ***Implementation of legislation (CTR/CTIS-PA2, MDR/IVDR)***

Ensuring adequate implementation not only of the Clinical Trial Regulation (and related CTIS) but also of Regulation (EU) 2017/745 on Medical Devices (MDR) and Regulation (EU) 2017/746 on In vitro Diagnostic Medical Devices (IVDR) is considered *the* main priority by stakeholders. Under this category reoccurring themes were:

- further simplification of CTR/CTIS requirements;
- patient centricity and personal data protection;
- ethics committees support and streamlining assessment;
- simplification and harmonisation of national requirements;
- international alignment and cooperation;
- contractual agreements and pricing;
- academic clinical trials.

2. ***Stakeholder representativeness (PA3)***

The need to include all concerned players in the composition of the MSP, including representatives of special populations (i.e. paediatric, rare diseases, oncology) and ethics committees, was reported.

3. ***GCP modernisation (PA4)***

Under this category the following objectives were proposed:

- GCP simplification;
- ICH E6 revision;
- CROs/vendors centralised qualification.

4. ***Evidence generation and submission /data gathering/digital support (PA5)/increase predictability***

In this context the general comments received were linked to the need for adequate digital support facilitating data generation, submission and use in order to inform CT assessment and increase CT outcome predictability. Such support is needed at national and international level.

5. ***Communication/transparency (PA6)***

Ensuring that MSP and *ad-hoc* working group outcomes are adequately communicated globally across all stakeholders.

6. ***Scientific advice (PA7)***

Harmonised and flexible scientific advice supported by network experts, patients and patients' representatives.

7. ***Methodologies (PA8)***

Update and develop guidance, implementing the learnings from COVID-19, in order to foster innovation and efficiency with a particular focus on rare diseases, paediatric and oncology.

8. ***Training (CT curriculum) (PA10)***

Create an adequate training infrastructure ensuring effective knowledge transfer, including practical aspects of clinical trials.

3.4. MSP concept paper

Participants were asked to provide comments on the MSP concept paper in terms of proposed scope, objectives, and organisational aspects. In this case the comments received were also grouped into categories (figure 5).

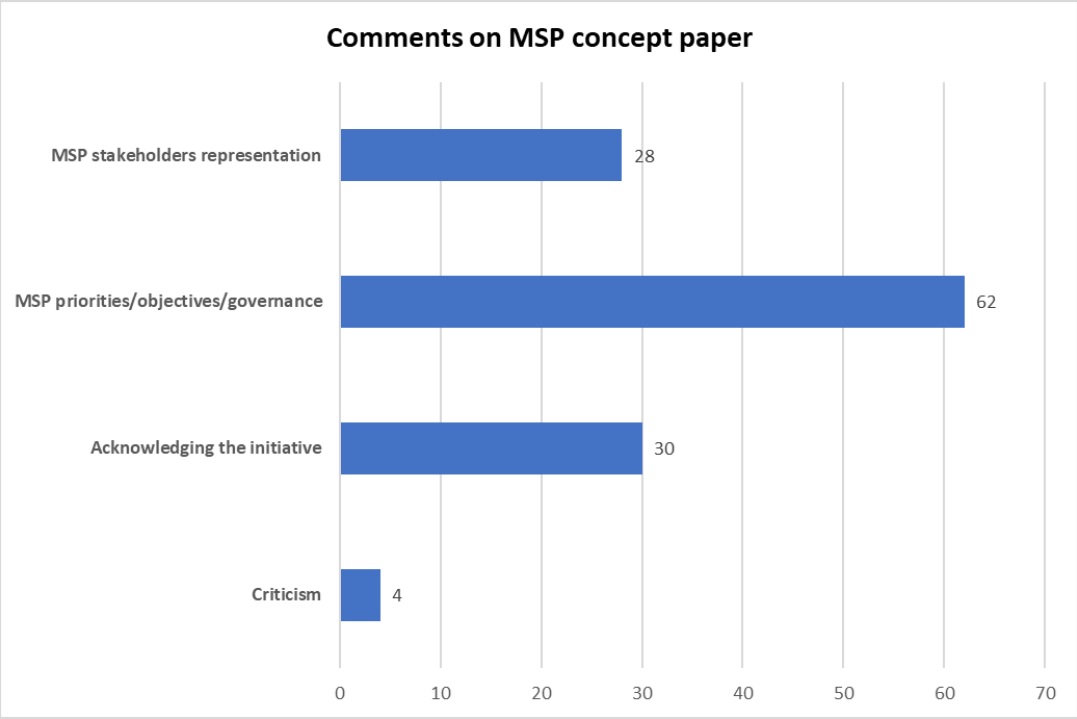

Figure 5. Results obtained for MSP concept paper

The initiative is generally appreciated and welcomed. Further refinement of MSP objectives and governance is flagged. The comments will be taken into account when drafting the MSP operational documents.

Stakeholders stressed the need to ensure adequate representation of key groups including patients, clinicians, national and international authorities, and ethics committees in the platform composition.

Several comments were made regarding MSP priorities and objectives, although this duplicates with the results above, the below areas were flagged for prioritisation:

- innovative technology/digital health;
- rare diseases, paediatrics, oncology;
- harmonisation of requirements of EU and non-EU initiatives and national support.
- patient equity and acceleration of diagnosis.

3.5. MSP interest

Participants had the option to express their interest in being part of the MSP. Where a positive answer was given, participants were asked to provide contact details, which are not included in this report.

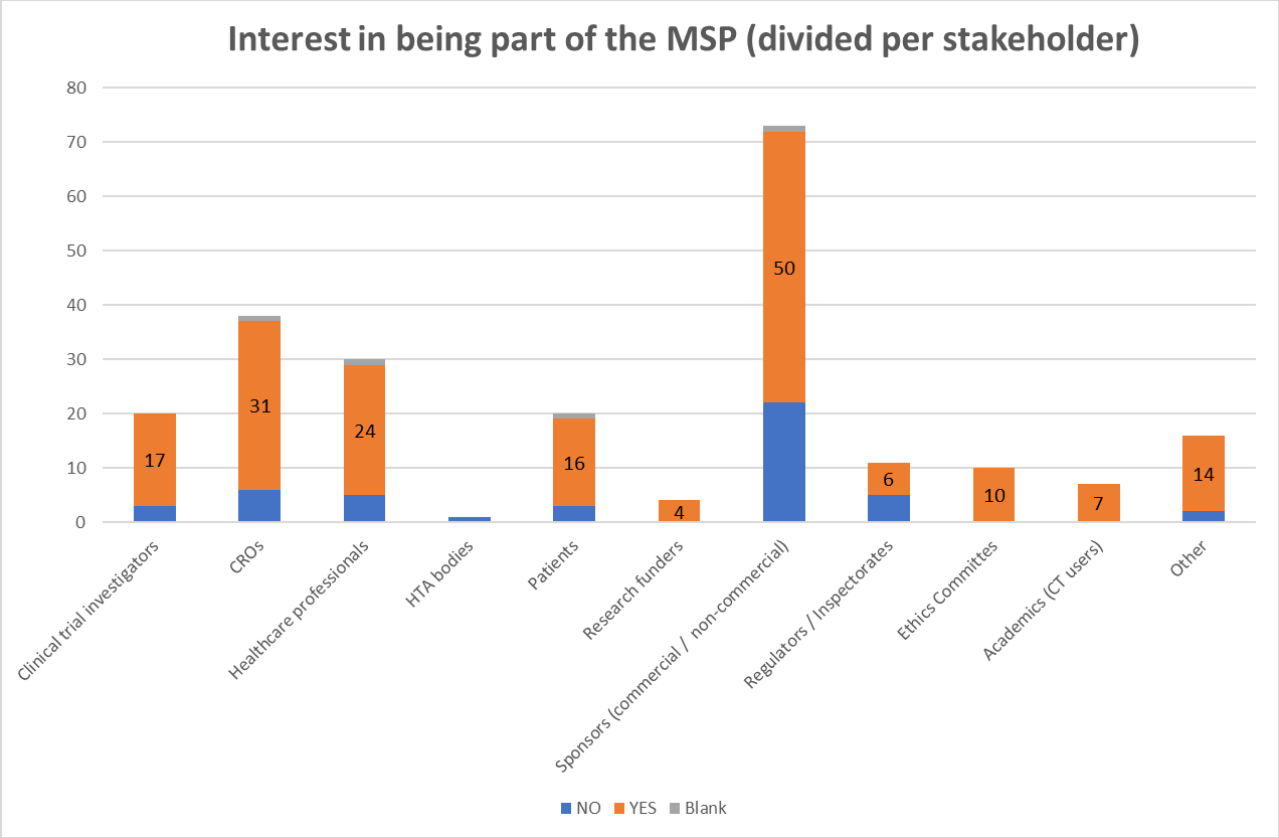

Figure 6. Respondents' interest in being part of MSP, grouped by respondent affiliation.