D6.01 HARMONY Stakeholder Forum

116026 – HARMONY

Healthcare Alliance for Resourceful Medicines Offensive against Neoplasms in Hematology

WP6: Stakeholder Forum, Patients, Payer/Provider, HTA, EMA alignment and optimization

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List of Acronyms

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<tr>
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<th>Description</th>
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<tr>
<td>HTA</td>
<td>Health Technology Assessment</td>
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<tr>
<td>PHSFF</td>
<td>Policy Health Stakeholder Feedback Forum (&quot;Stakeholder Forum&quot;)</td>
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<td>WP</td>
<td>Work Package</td>
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<tr>
<td>EFPIA</td>
<td>European Federation of Pharmaceutical Industries and Associations</td>
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<td>EMA</td>
<td>European Medicine Agency</td>
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<td>IMI</td>
<td>Innovative Medicines Initiative</td>
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<tr>
<td>SF</td>
<td>Same as PHSFF (&quot;Stakeholder Forum&quot;)</td>
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<tr>
<td>LT</td>
<td>Leadership Team</td>
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<tr>
<td>TC</td>
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1. PUBLISHABLE SUMMARY:

The Policy Health Stakeholder Feedback Forum (PHSFF, "Stakeholder Forum") acts as a key platform of interaction and consultation between all stakeholder groups from HARMONY and outside of HARMONY. Collecting input and discussing viewpoints from patient organisations, haematologists/clinicians, regulators and HTA bodies will be crucial to shape the work of HARMONY, especially in WP2, WP5, WP6 and WP7, alongside the implementation of the HARMONY project.

2. SCOPE:

This document presents the objectives of the The Policy Health Stakeholder Feedback Forum (PHSFF, "Stakeholder Forum"), the implementation of the PHSFF and the way to consult the PHSFF for the different HARMONY WPs.

3. PURPOSE AND OBJECTIVES (as described in the DoA):

The Policy Health Stakeholder Feedback Forum (PHSFF, "Stakeholder Forum") acts as a key platform of interaction and consultation between all stakeholder groups. Collecting input and discussing viewpoints from patient organisations, haematologists/clinicians, regulators and HTA bodies will be crucial to shape the work of HARMONY, especially in WP2, WP5, WP6 and WP7.

The PHSFF will discuss barriers, gaps, and needs potentially leading to a consensus on innovative solutions in the area of Big Data. There will be three face2face meetings, after which a report will be written. The report will document enablers and barriers, opportunities and constraints. Depending on the topics in consultation, the reports can provide the basis of white papers which will be developed and disseminated in collaboration with WP7.

The PHSFF will be structured by the following stakeholder clusters:

1. Patient organisations
2. Haematologists / clinicians
3. Medicines authorities (EU/EMA and national level)
4. HTA bodies
5. Payers
6. Pharmaceutical industry

The PHSFF will liaise with these stakeholder clusters to understand viewpoints, concerns and requirements which support the work in HARMONY.

The PHSFF’s discussion and consultations with stakeholders will consider the outcomes definitions and validation (coordinated by WP2), the evidence frameworks for innovative technologies (developed in Task 6.2), and the Clinical Value framework to quantify therapeutic value of innovative technologies for HMs (task 6.3). It will also consider the research question developed by WP2, whenever accurate,

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1 to the extent that payers will agree to participate in HARMONY meetings
including the pilot research questions, as well as the discussions of novel methodologies and proof-of-principle study discussed in task 6.4. Finally, SF will also be a place to collect research questions from all these stakeholders.

The clusters will meet in virtual meetings plus three face2face meetings over the whole 5-year project period.

The key to successful and effective stakeholder integration is the generation of relevant evidence to address uncertainties and support decision-making in relation to regulatory approval, HTA recommendations, reimbursement approval and patient access. Evidence from randomized controlled trials (RCTs) has traditionally been the gold standard for decision-making regarding market access of cancer drugs. In recent years, considerable amounts of data have been generated from increasingly diverse sources (e.g. electronic health records, real-world data), and these offer new opportunities for evidence collection. HMs are characterised by remarkable heterogeneity, and patient sub-populations are often not adequately characterized in RCTs. Despite the great potential of aggregating individual-level data sets, there are still many practical difficulties, including constraints to data sharing between different healthcare systems.

Different initiatives have been proposed to identify and address these problems (EUnetHTA projects; INNO-HTA, INTEGRATE-HTA; Advance_HTA; IMI GetReal; IMI ADAPT-SMART, European Medical Information Framework/EMIF), but none has specifically considered the particular challenges of HMs.

HARMONY is in a privileged position to provide a discussions forum between all relevant stakeholders developing or using high-quality data sets; discussions that will address uncertainties in decision-making regarding access to new drugs for HMs, brainstorm around and set the path for development of treatment guidelines and new clinical treatment pathways, and likely provide a solid evidence base to inform increasingly personalised therapeutic approaches.

WP6 will develop a new model for multi-stakeholder dialogue by creating a Policy Health Stakeholder Feedback Forum (PHSFF) involving the HARMONY partners and associated members, and external experts at the EU and Member State level. This will enable broad representation of the diverse models of healthcare systems in the EU, and include patients and clinicians, regulators, HTA bodies and payers, as well as stakeholders involved in evidence generation. This Forum will be essential to provide input and advice into the WP tasks to ensure the generalisability and applicability to the agencies within EU member states of the frameworks for evidence and clinical value. The following issues will also be discussed in the PHSFF: e.g. behavioural, ethical, legal, social implications as well as gender and age dimensions).

Regarding the EMA/IMI-funded initiatives, some of their topics or approaches might overlap the ones of the HARMONY project, in particular regarding the consultation of decision-makers. So in order to stay aware of the potential collaboration and get some learnings from these initiatives, a meeting can be set with relevant participants of these initiatives in order to inform the WP6 members about them.

Regarding relationship with Policy Makers, given that their responsibilities are linked to legislations which are difficult to influence, this stakeholders cluster will be created later on and in collaboration with WP7, with the idea to bring them information on the HARMONY project, in particular about the objective of bringing the right therapy to the right patient at the right time.
4. IMPLEMENTATION:

4.1. Stakeholder Clusters

The Stakeholder Forum consists of 6 different clusters. Each cluster is chaired by a cluster lead taken from the WP6 members. The Cluster Lead will propose a list of experts or organisations within that stakeholder cluster, get in touch with them to obtain their approval for participating into the SF consultations, maintain the list of stakeholders and stakeholders’ participation active (or replace them whenever necessary), check their availability prior to schedule any F2F or teleconference meeting, formally invite them to consultations, send them feedback about results of the consultation, and communicate them about the progress of the HARMONY project. The coordination of the consultation of cluster members will be done by the task 6.1 lead and its subteam; it means organisation of the consultation (by email, phone or F2F meeting), setting a date for the consultation, providing briefing book to experts, keeping track of feedback received, producing the summaries.

To avoid asynchronous point-to-point communication and a disconnect clusters, the coordination between cluster leads will be done by the Task 6.1 Lead, the subteam and cluster leaders, keeping the WP6 Leads informed.

4.1.1. Patient organizations

The patient organisations cluster is organised by the seven disease areas of the HARMONY project. LeukaNET (Jan Geissler and Tamas Bereczky) will act as the cluster lead and budget holder to coordinate overall patient input into the project and provides the interface to all WPs through the HARMONY Steering Committee.

Each disease area will have a named person as a disease area lead. Each disease lead in this cluster is liaising with e.g. WP2 and WP5 on disease-specific questions, but also makes sure that it provides a report on that interaction to the WP6 coordinators, to keep discussions on the HARMONY Steering Committee level in sync.

The disease-specific umbrella organisations identified so far are:

- Multiple Myeloma: Myeloma Patients Europe
- AML: Acute Leukemia Advocates Network / Leukemia Patient Advocates Foundation and International MDS Alliance
- ALL: Acute Leukemia Advocates Network / Leukemia Patient Advocates Foundation
- CLL: CLL Advocates Network
- NHL: Lymphoma Coalition Europe (tbc)
- MDS: International MDS Alliance
- Pediatric Hematological Malignancies: Childhood Cancer International

The above umbrella organisations encompass about 200 disease-specific patient organisations (mostly organisations operating on a country level) in their membership.

Additional patient organisations (e.g. national organisations or umbrellas in hematology or
cancer) can be included in the patient organisations cluster's consultations whenever necessary.

In addition, an interface to cross-disease patient initiatives is being established, to allow consultation of the wider patient community on disease-unrelated issues, e.g. on Big Data and value. More specifically:

- EUPATI (especially its Alumni Network and National Platform Network)
- ePAG of the EuroBloodNet ERN (for the link to EuroBloodNet)
- European Patients’ Forum (for EU policy)
- EURORDIS (for rare diseases)

4.1.2. Hematologists / clinicians

The hematologists/clinicians cluster consists of two subgroups:

1. The clinicians through HARMONY consortium members and associate members
2. Medical associations

The leads of this cluster will be Celgene (Yann Guillevic) and EHA (Carin Smand).

Members of the clinicians include the clinical leads of the seven disease areas in HARMONY:

- Multiple Myeloma: Sonneveld, San Miguel, Boccadoro
- AML: Ossekoppele, Huntly, Lo Coco
- ALL: Gökbüget, Dombret, Ribera
- CLL: Ghia, Pospisilova, Bosch
- NHL: Salles, Dreyling, Montoto
- MDS: Fenaux, Kuendgen, Santini
- Pediatric Hematological Malignancies: Moorman, Reinhardt, Locatelli

The medical associations will be consulted on transversal fields. They include:

- EHA (for hematology and value framework)
- ESMO (for medical oncology)
- ECCO (for multidisciplinarity and EU policy)

4.1.3. Medicines authorities (EU/EMA and national level)

The medicines authority cluster will be coordinated by Janssen and will collect input from regulatory authorities into HARMONY’s activities both in terms of methodology and experience. Members of the medicines authorities cluster may be:

- EMA
- BfArM
- AEMPS
- European Medicines Agency
- Swedish Medical Products Agency
- MHRA
4.1.4. HTA bodies and projects

The cluster lead of HTA experts will be the NICE (Katy Harrison).

The HTA cluster of the Stakeholder Forum will aim to involve a representation of HTA bodies from across Europe, up to 7 HTA agencies in Europe. The agencies will be chosen according to their willingness to participate in these consultations. The aim is to ensure that there is an understanding of processes and policies that affect the selection and definition of health outcomes and explore big data offers potentials to reduce decision uncertainty in HTA assessment of innovative technologies. The cluster already has a good connection with EUnetHTA through NICE and will connect with relevant European projects as appropriate.

4.1.5. Payers

The cluster lead will by Celgene (Hélène Chevrou-Séverac).

Payers have a very different profile across European healthcare systems and into countries as well.

Payers are defined as any decision-makers who are involved in setting therapies price and/or reimbursed price or in deciding about regional or hospital healthcare budget allocation.

When taking the example of the French healthcare system, the reimbursed price of therapies is negotiated by the CEPS (Comité Economique des Produits de Santé) sitting in the Ministries of Health and Finance; while the hospital budget allocation for drugs is decided by the hospital pharmacist in collaboration with physicians. In Germany, the reimbursed price of drugs is negotiated between the drug manufacturer and the Sickness Funds association (GKV-Spitzenverband). Prices can also be re-negotiated by hospital whenever the drug is used into hospitals settings. In England, the price negotiation can happen with the National Health System (NHS) under the PPRS (Pharmaceutical Price Regulation Scheme) or with each Clinical Commissioning Groups (CCGs). Therefore, a list of stakeholders will be made according to this complexity, and involving ‘Payers’ of the different levels.

However, it is important to pay attention to the fact that collaboration of these stakeholders can be difficult to set for projects done in a Private-Public partnership. Indeed, both at the national and local levels, it can be challenging sometimes to find ‘Payers’ willing to collaborate with the pharmaceutical industry. Additionally, for national ‘Payers’ the price negotiation is 1/ based on the HTA appraisal of the comparative clinical benefit of new therapies; 2/ highly driven by national law and regulations, and very little by research; therefore, it might not be a highly relevant stakeholders group for the HARMONY project. The cluster lead will therefore assess first the willingness of these stakeholders to participate to the SF.
4.1.6. Pharmaceutical industry

This cluster will be led by Celgene (Hélène Chevrou-Séverac).

The following companies, members of the HARMONY consortium, as well as member of the EFPIA, will be the key stakeholders of this cluster:

- Novartis
- Celgene
- Amgen
- Janssen
- Bayer AG
- Menarini Ricerche S.p.A
- Takeda

5. CONSULTATIVE MECHANISM:

5.1. Type of consultations

The Stakeholder Forum has identified three mechanisms for eliciting stakeholder feedback through consultation and discussion within the clusters, or across multiple clusters at a time.

5.1.1. Face2Face Meetings / Feedback in person

Three face2face meetings of the Stakeholder Forum are foreseen within the duration of the HARMONY project. Three F2F meetings will occur during the five years of the project. The budget planned for these meetings is €30’000 (within Leukanet). This budget is expected to cover the flights of participants from the patients’ association clusters, potentially from the HTA agencies, Medicine Agencies and Payers clusters whenever necessary and aligned with the countries’ law and codes covering the relationship of the industry with public agencies members. The budget should also cover the catering services. The venue of the F2F meeting will be organized into the offices of the Pharmaceuticals Companies participating to HARMONY.

A written Stakeholder Forum Feedback Report will be produced, providing summaries of the discussions, describing the consensus and disagreement on the topics discussed. The report will be done by the task 6.1 lead and subteam.

5.1.2. Feedback by electronic surveys and E-Mail

An electronic system owned or located on the HARMONY internet platform will be used to elicit feedback from the clusters on a specific topic, either through mechanisms established by WP7 or WP6. The survey will be distributed to stakeholders of each cluster by the cluster lead. A Stakeholder Forum Feedback Report will be produced by task 6.1 lead and subteam providing summaries of the qualitative and quantitative feedback received.
E-Mail may also be used for consultations, however is not seen as very efficient to collect and document feedback in a structured way.

5.1.3. Feedback and discussion by teleconference

A teleconference system will be used for group discussions. The system to be used for such consultation will be clarified later. A Stakeholder Forum Feedback Report will be produced in the form of minutes by the task 6.1 and subteam.

5. PROCESS TO SET UP A CONSULTATION:

5.1. Requesting a consultation

Channel 1:

When a WP member (thereafter called the applicant) would like to activate a consultation of the SF, an email request should be sent to WP 6 leadership team (thereafter called WP6 LT) with the following information (with copy to the WP leads to which the applicant belongs to):

- Objectives of the consultation
- Clusters the applicant would like to consult and eventually number of stakeholders per cluster
- Involved partners in HARMONY (other WP members, etc...)
- Type of consultation: individual TC or group TC with length of the TC, or electronic consultation or F2F meeting (please note that some restrictions apply to the F2F meetings)
- Expected date to consult them

Channel 2:

Through the HARMONY Steering Committee, the WP6 LT will map out when, how and where the stakeholders’ feedback from the clusters across the different WPs is required, so WP6 LT can plan ahead accordingly.

Remarks: the SF consultation doesn’t intend to replace the Early Scientific Advice or Parallel Scientific Advice consultation of the EMA and HTA agencies on drugs in development or in pre-launch or post-launch phase.
5.2. Validation of the objectives of the consultation

For both channels, the WP6 LT will communicate the topic of the consultation to the Steerco to obtain their approval to go to the next step of the consultation process. Feedback is expected in a timeframe of a working week (5 days).

Once the SteerCo has given a positive feedback on the topic of the consultation, the Task 6.1 Lead and subteam will distribute the objective of the consultation to the cluster leads. The Task 6.1 and cluster leads will:

- assess and validate which clusters are relevant to be consulted on the topic of the consultation,
- give recommendation regarding the type of the consultation
- assess feasibility of the timeline for consultation

The output of this step will be a written answer on these three points to the applicant, with copy to his/her WP leads.

Once the principle of making a consultation for the topic submitted by the applicant has been agreed by Task 6.1, then the next steps are implemented to organize the consultation.

5.3. Preparing the consultation

The Task 6.1 Leadership Team (thereafter, Task6.1 LT) will set a TC with the APPLICANT and his/her team to get the supplementary details of the consultation: more detailed objective of the consultation, targeted stakeholders, type of consultation, length of consultation, and possible dates for the consultation; as well as information to send stakeholders ahead to maximize the consultation, type of feedback expected, format of feedback; any logistical resources needed.

The following steps are required to be done by the APPLICANT and his/her team to prepare the consultation

- Provide to the Task 6.1 Leadership Team (thereafter, Task6.1 LT) an invitation letter which will be provided to the stakeholders. The letter should follow the HARMONY format with related logo, present the objectives of the consultation, the type of consultation and include expected dates / timelines (depending on the type of consultation) of the consultation and output required to the stakeholders
- Set themselves the TC consultations with stakeholders, in case of TC with only one stakeholder at a time
- Prepare a presentation or a briefing book and/or questionnaire to submit to the stakeholders
- Take minutes of the TC or F2F meeting with the stakeholders
The following steps will be set by the Task6.1 LT for organizing the consultation:

- Submit the stakeholders lists to the APPLICANT and pick up with him/her the names of the ones to be consulted
- Set up the venue of the consultation for F2F meeting and support for organizing the trips of the Stakeholders invited to the meeting; and manage the budget for the event
- Support for group TC (consultation of multiple stakeholders in one TC), to the extend it’s no more than one TC per cluster
- Ask the Cluster Leads to:
  - Contact the chosen stakeholders and send them the invitation letter
  - Secure the consultation with a minimum of stakeholders from their clusters. Or find replacement whenever necessary
  - Attend the meetings when appropriate from APPLICANT standpoint or wished by the Lead

5.4. Dissemination and Tracking of feedback by each cluster

The following steps are required to be done by the APPLICANT and his/her team to finalize the consultation:

- Prepare a summary report of the output of the consultation (following the HARMONY internal review process)
- Send the summary report to each stakeholder consulted in a joint email with the Cluster Lead, plus the HARMONY SC
- Collaborate with Task6.1 LT and WP6 LT and WP7 LT for developing and implementing a dissemination plan of the findings of the consultation to other HARMONY participants and externally whenever appropriate.

The following steps will be set by the Task6.1 LT for ending the consultation:

- Ask the Cluster Leads to:
  - Send the summary report to each stakeholder consulted in a joint email with the applicant
  - Thank the stakeholders for their participation by an email or a letter with official template of HARMONY project
- Collaborate with the applicant, WP6 LT and WP7 LT for developing and implementing a dissemination plan of the findings of the consultation to other HARMONY participants and externally whenever appropriate

The aforementioned reports about the feedback received will be provided to all cluster members as well as to the HARMONY Steering Committee, consisting of the Work Package leads and to the WP6 members.
The WP leads are responsible to disseminate the feedback to the WP members concerned. They are also responsible to feed back to the Stakeholder Forum and the HARMONY Steering Committee where the feedback has been incorporated in the work and implementation of the WP.