MEDEV: MoCA Pilot Project
Stage 2: Revised Terms of Reference
(Consolidated Version Adopted 25 January 2016)

- **Definition of MoCA**

  MoCA provides a mechanism for European countries to collaborate on coordinated access to Orphan Medicinal Products (OMPs) in a voluntary, dialogue-based approach, intended to create a fluid set of interactions between key stakeholders and across all aspects of an OMP. The roles, responsibilities and prerogatives of each of the participating stakeholders should be respected.

  It is important to stress that, to date, nowhere else in Europe does such a platform exist where companies' issues around reimbursement or financing schemes can be discussed with such a variety of jurisdictions and societal perspectives.

- **Participation in MoCA**

  Participation in MoCA is open to several stakeholder groups, as per the following list:

  - National competent authorities for pricing and reimbursement: Currently represented by a selection of such national authorities, that are sitting on the Medicines Evaluation Committee (MEDEV). It is agreed that participation of other committed organisations on a more regular basis is highly desirable.
  - Patients: Currently represented on a horizontal basis by EURORDIS, the European Organisation for Rare Diseases, with individual patients or patients' organisations relevant to a specific Orphan Medicinal Product to be invited on an ad hoc basis.
  - Candidate marketing authorisation applicant/holders willing to be involved in a pilot focused on a particular product of theirs.
  - Pharmaceutical industry at large: It is recognised that the approach for industry participation in MoCA should be further explored, taking into consideration the individual development programmes under discussion within MoCA and the sensitivity that may derive from this, as seen from a company's perspective.
  - Others: Experts from the EMA, EUnetHTA or other scientific committees/HTA agencies as well as individual medical experts or selected representatives from the pharmaceutical industry may also be invited and involved in specific pilots as appropriate and relevant, and upon the general agreement of all other regular participants.

- **Objectives of MoCA Pilots**

  The objectives of pilot projects retained within MoCA shall be to establish and facilitate "early dialogue" between companies and competent authorities for pricing and reimbursement, focusing discussions on a company's strategy, the targeted disease, the product and its development, the approach and identified challenges to access markets with a view to ultimately helping to speed up access for patients in EU Member States to the product in question.

- **Potential Benefits of MoCA Pilots for All Stakeholders and Vision for the Future**

  The benefits for the stakeholders to participate in a pilot project are likely to be the following:

  - Marketing Authorisation Applicant/Holder: More predictable uptake of an Orphan Medicinal Product; more effective gathering of information about the real-life outcomes in a clinical setting; increased predictability of the product development, based on enhanced collaboration and dialogue; better knowledge and understanding of EU Member States' expectations regarding pre-requisites for pricing and reimbursement.
  - National Competent Authorities: Better identification and prediction of patient numbers; better budget impact visibility and predictability; increased qualitative and quantitative information to support well-informed decision-making at national level; sharing of expertise, resources and knowledge with other EU Member States.
  - Patients: Quicker and broader availability of the Orphan Medicinal Product in question, increased equity between patients in different EU Member States; better and more coordinated follow-up and collection of patient-reported outcomes and real-life experiences.

  MoCA does not exclude the option that, after a period of time reflecting a gain of experience from ongoing pilots, deal negotiations between several EU Member States/national authorities and a company for a given product could be opened to develop a prompt and structured approach to market and patients, and also to support a coordinated approach for post-marketing research activities with a view to speeding up the generation of additional evidence.

  At this stage, it seems too early to integrate these sessions of "early dialogue with payers" in other ongoing European mechanisms of collaboration (e.g. Scientific Advice and Protocol Assistance at EMA, Parallel Scientific Advice between EMA and HTA agencies, Scientific European Early Advice at EUnetHTA, etc), considering that greater experience should be gained in order to allow a considered and fact-based recommendation about such a possibility.

  It is recognised that the payer input is difficult to streamline (in light of different health care systems, economies, priorities…) and that, therefore, the idea of an unequivocal mandate for a given representative from payers to speak with one voice is seen as not possible yet. We need further experience on how to handle these differences in a productive way and MoCA appears as the right platform to continue garnering that experience.

- **Constitutive Elements of MoCA Pilots**

  - Which company? Open to all companies with an Orphan Medicinal Product at any stage of development
  - Which product? An OMP or a designated OMP. Products may include small molecules, biologics as well as gene therapies.
  - At which stage? Any stage from horizon-scanning through to Marketing Authorisation and, in exceptional cases, post-MA (e.g. in situations where coordination at the European level could be beneficial to support post-MA research and continuous evidence generation activities).
  - Which materials will the candidate company need to provide to MoCA? This aspect shall be left to the discretion of the candidate company, as no formal list of requirements has been defined to date.
    However, lessons learned from current practice and pilots conducted up to now indicate that the candidate company should want to identify all available documentation that will support its

conversation with MoCA, e.g. a dossier submitted to the EMA, EUnetHTA or any other relevant organisation, with a view to highlighting aspects such as (and not exclusively):

  - the specific disease addressed by the candidate product, with an overview of its epidemiology in Europe as well as the standard patient journey;
  - the state of the unmet medical need addressed by the candidate product;
  - the clinical and economic value proposition of the candidate product;
  - the regulatory state of play for the candidate product, incl. all interactions to date with European national authorities and foreign equivalents;
  - the design of, and available outcomes/learnings from, clinical trials under way, particularly with regard to proposed endpoints.

- Confidentiality: A confidentiality agreement is mandatory and will be provided by the pharmaceutical company to other parties participating in the conversations, keeping in mind that MEDEV members are by principle bound to confidentiality. It is deemed understood and agreed that the fact that a company is engaging in the process shall *not* be confidential itself – what shall be confidential is everything else beyond that, starting with the name and nature of the product, the detailed content of the exchanges with MoCA members at regular meetings and remotely via email, etc.
- Legal Considerations:
  - As laid down in the original MoCA Report and Recommendations, participation is on a strictly voluntary basis for all stakeholders.
  - Proceedings and conclusions shall be confidential and non-binding up to and until a formal agreement may be signed by all interested parties.
  - An "opt-out" option for candidate companies exists at all times during preliminary discussions as well as the rest of the MoCA process.

- **Key Success Factors**

  The ability of MoCA to deliver on its purpose will largely depend on all participants' capacity to:

  - avoid whenever possible duplication of other similar activities, projects or workstreams with similar or related scope, and build instead on what is already existing or on-going (e.g. projects like IMI's ADAPT-SMART and current attempts at forging a more seamless process between the EMA, HTA and payers under the umbrella of Medicines Adaptive Pathways for Patients-MAPPs);
  - be flexible, constructive and open-minded;
  - move in both a timely but measured, step-by-step approach.

- **Template Process for MoCA Pilots**

  - A MoCA pilot may be initiated upon expression of interest on the part of a candidate Marketing Authorisation Applicant/Holder and submission of an initial overview of the relevance of a given Orphan Medicinal Product compared to the overall purpose of MoCA.
  - Once identified, the recommended way to proceed is to organise an initial presentation on the occasion of a regular MoCA meeting, in order to start the dialogue between MoCA participants and the candidate company, test the validity of the proposed OMP within the broader purpose of MoCA, and agree upon next steps to be taken in the process.

- If a general agreement is found that a pilot can be mutually beneficial to all parties indeed, then the dialogue shall continue all along the development of the candidate OMP in a planned way and at the pace determined by all parties as most appropriate, in the form of follow-up interactions at regular MoCA meetings or close to other events attended by regular MoCA members.
- In order to drive and sustain the dialogue process towards successful outcomes, it is recognised as beneficial to appoint preferably two, but at least one, MoCA members per pilot as main contact persons and discussion coordinators. That role shall encompass the preparation and handling of all face-to-face discussion with candidate companies, the proper follow-up to such interactions and liaison with companies and other MoCA members in-between regular meetings in order to address pending questions and needs for clarification or more detailed information.
- In the same spirit, and as a first step towards greater integration, it has been proposed that such "leads" and/or "co-leads" (when two of them) could also be mandated to attend with observer status whenever the candidate product they are responsible for within MoCA does go to EMA-HTA Parallel Scientific Advice, in collaboration with the company or organisation responsible for its development.

- **Follow-Up, Sharing of Expertise and Outcomes**
  - Indicatively, a MoCA pilot may take around 18 months to complete. This timeline may be accelerated or lengthened as per the specificities of the OMP in question.
  - Upon successful completion, a report narrating the experience of the pilot shall be developed jointly by MoCA members directly involved in the said pilot and by the Marketing Authorisation Applicant/Holder, with support and input of other involved stakeholders as appropriate, with the objective of drawing useful lessons to be taken as well as recommendations for the future.
  - The report described above may be complemented by a dedicated workshop to share the outcomes, experiences and learnings – both positive and negative – from the process. The principle for such a workshop and its very contents shall be agreed with the Marketing Authorisation Applicant/Holder, pursuant of course to the provisions of any previously signed confidentiality agreement.
  - With the above in mind, it is hereby put to the attention of any Marketing Authorisation Applicants/Holders willing to take part in a MoCA pilot that, prior to expressing interest and getting involved, they should assure themselves of their willingness and readiness to share such learnings (mostly process-, not content-related) that can be used to evaluate the potential of such a process to contribute to the stated objectives and to propose changes or refinements with the aim of further improving the ability of the process to deliver on its objectives.

- **Secretariat to MoCA**
  - Competence: The MoCA secretariat, in conjunction with each pilot's coordinators and the concerned company, shall aim at providing documentation to all members well in advance of a meeting in order to ensure optimal preparation and conduct of the discussions. After the meeting, a summary of the proceedings shall be prepared as well as a list of key points in discussion. The MoCA secretariat shall also monitor and keep up-to-date the rolling programme for planned discussions with companies.

- Secretariat to MoCA is provided by courtesy of MEDEV and ESIP, the European Social Insurance Platform, with added financial or in-kind contributions from other member organisations, e.g. INAMI/RIZIV and EURORDIS.

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