Glossary of Activities & Agencies with Potential Relevance for MoCA

Activities involving early access highlighted in green

Name of Agency/Activity	Why is This Important	Further Information
1. MARKETING AUTHORISATION		
1.1. European Medicines Agency (EMA)	EMA is responsible for orphan designation and the marketing authorisation of Orphan Medicinal Products (OMPs)in the EU	www.ema.europa.eu
1.1.1. Orphan Designation	Legal basis for incentives: market exclusivity if approved, reduced fees	http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000029.jsp&mid=WC0b01ac05800240ce
1.1.2. Authorisation as Orphan Medicinal Product (OMP)	Legal basis for market exclusivity, upon review of the orphan status	http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000554.jsp&mid=WC0b01ac058061ecbb
1.1.3. Scientific Advice and Protocol Assistance	Ensures that the company performs the appropriate tests and studies, so that no major objections regarding the design of the tests are likely to be raised during evaluation of the marketing-authorisation application. Facilitates development, Important for SMEs	http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000049.jsp&mid=WC0b01ac05800229b9
1.1.4. Parallel Scientific Advice	As above but together with other institutions/agencies	
1.1.4.1. With HTAIs (Heath Technology Assessment Institutions)	Advice in parallel from EMA and European Health Technology Assessment bodies selected by the sponsor; helps ensure that evidentiary requirements for HTA	http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/q_and_a/q_and_a_detail_000156.jsp&mid=WC0b01ac0580a11c96

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	(and, ultimately, reimbursement and patient access are met)	
1.1.4.2. With the Food and Drug Administration of the USA (FDA)	US Food and Drug Administration, via tele- or videoconference; helps ensure that study design is acceptable to both regulators	http://www.ema.europa.eu/docs/en_GB/document_library/Other/2009/11/WC500014868.pdf
1.1.5. PRIME (PRIority MEdicines)	Prioritization for medicines with promise of a major therapeutic advantage through continuous support & guidance from EMA	http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000660.jsp&mid=WC0b01ac05809f8439
1.1.6. Adaptive Pathways	Programme of iterative marketing authorisation, whereby an early, initial authorisation in areas of high medical need is later confirmed or expanded Dialogue with HTAIs to ensure commitments are met Adaptive pricing models	http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000601.jsp&mid=WC0b01ac05807d58ce
1.2. Compassionate Use	Making a medicinal product available for compassionate reasons to a group of patients with a chronically or seriously debilitating disease or whose disease is considered to be life-threatening, and who cannot be treated satisfactorily by an authorised medicinal product (the medicinal product concerned must either be subject of an application for a central marketing authorisation or must be undergoing clinical trials). Compassionate use is distinct from 'named-patient basis' treatments, whereby doctors obtain medicines directly from manufacturers before authorisation.	http://adaptsmart.eu/wp-content/uploads/2016/04/D2-02-ADAPT-SMART-Glossary-first-edition.pdf

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	Funding for both is regulated at the national level	
2. HEALTH TECHNOLOGY ASSESSMENT		
2.1. Health Technology Assessment EUnetHTA	European network of heath technology assessment institutions (HTAIs) HTA ensures that the technology provides good use of public resources.	www.eunethta.eu
2.1.1. Early Dialogue	Advice by HTAIs on the prerequisites of Phase III trials to ensure that these answer not only questions raised in the context of risk/benefit assessment for marketing authorisation, but also of added benefit assessment for reimbursement	http://www.eunethta.eu/sites/5026.fedimbo.belgium.be/files/EUnetHTA%20early%20dialogue%20consolidated%20procedure_%20November%202015.pdf
2.1.2. Relative Effectiveness Assessment	Assessment of added patient benefit vs available therapies. This is usually a prerequisite for national reimbursement decisions. Such assessments are done nationally, but they can be based on joint assessments by EUnetHTA collaborators.	http://eunethta.eu/joint-assessments
2.2. National HTAIs (NICE, IQWIG, HAS, etc.)	Will not be discussed here further	
3. POSTMARKETING ACTIVITIES		
3.1. "Heads of Agencies"	National Marketing Authorities, responsible for postmarket monitoring	http://www.hma.eu/
3.2. Orphanet's List of Rare Disease Registries in Europe	Registries are important for postmarketing data collection (as well as for information about the	http://www.orpha.net/orphacom/cahiers/docs/GB/Registries.pdf

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	natural history of a rare disease, if available)	
3.3. Further EC activities supporting rare disease research	Supporting rare diseases registries and providing a European Platform for rare diseases registration	http://ec.europa.eu/health/rare_diseases/policy/registries/index_en.htm
4. PRICING AND REIMBURSEMENT		
4.1. Competent authorities on Pricing & reimbursement (CAPR)	Meeting of policymakers and decision-makers from all member states to share knowledge and explore common positions concerning developments in medicines and the pricing of medicines, organized by each EU presidency. EURORDIS and EPF have called on the Network to support access via the establishment of a negotiating table and scaling-up of Early Dialogues	https://english.eu2016.nl/events/2016/03/22/competent-authorities-on-pricing-and-reimbursement-capr (each meeting has a new website)
4.2. WHO Collaborating Centre for Pharmaceutical Pricing and Reimbursement Policies	Exchange of information and experience about pharmaceutical policies based on shared values such as solidarity and equity, supported by scientific evidence and a common understanding and language on pharmaceutical issues	http://whocc.goeg.at/
4.3. National Payers (Health insurance, Health Ministries, local health authorities	Responsible for financing medicines, incl. orphan medicinal products	Will not be discussed here further
5. POLICY AND LEGISLATION		
5.1. European Commission (EC) Directorate General (DG) for Health and Food Safety (DG Sante)	Health strategy, policy legislation, monitoring and enforcement, incl. EMA	http://ec.europa.eu/dgs/health_food-safety/index_en.htm

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5.2. EC DG Internal Market, Industry, Entrepreneurship and SMEs (DG GROW)	Identify and remedy obstacles to the biotechnology industry, achieving a single market for pharmaceuticals that also guarantees a high level of protection for public health – Transparency Directive	http://ec.europa.eu/growth/sectors/biotechnology_en
5.3. EC Expert Groups and Advisory Committees	Expert advice to the EC	
5.3.1. Commission Expert Group on Safe and Timely Access to Medicines for Patients (STAMP)	Advice to the EC on EU pharmaceutical legislation, as well as programmes and policies in this field.	http://ec.europa.eu/health/documents/pharmaceutical-committee/stamp/index_en.htm
5.3.2. Pharmaceutical Committee	Committee of senior experts in public health matters from the Member States' administrations Advice on all human medicines including matters related to the preparation of new legislative initiatives	http://ec.europa.eu/health/documents/pharmaceutical-committee/index_en.htm
5.3.3. "Notice to Applicants"	The aim of the group is to draw up and discuss the (non-binding) guidance intending to facilitate the interpretation and the application of the EU pharmaceutical human and veterinary legislation.	http://ec.europa.eu/health/documents/pharmaceutical-committee/notice_to_applicants/index_en.htm
5.3.4. European Expert Group on Rare Diseases (EGRD)	Advisory group on legislation, policy, cooperation and monitoring of actions in the field of rare diseases	http://ec.europa.eu/health/rare_diseases/expert_group/index_en.htm
5.4. European Council (conclusions on strengthening the balance in the pharmaceutical systems in the EU and its Member States)	"NOTES WITH CONCERN an increasing number of examples of market failure in a number of Member States" and "calls for an evidence based analysis" of the impact of the incentives in the EU legislative instruments	http://www.consilium.europa.eu/en/press/press-releases/2016/06/17-epsco-conclusions-balance-pharmaceutical-system/

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6. STAKEHOLDER GROUPS (& LOBBYING)		
6.1. EURORDIS	Alliance of patient organisations representing 716 rare disease patient organisations in 63 countries covering over 4000 diseases Participants in MoCA and several other projects; promoting use of patient-relevant outcomes	http://www.eurordis.org/
6.2. Medicines Evaluation Committee (MEDEV)	Informal grouping of representtatives from 18 organisations responsible for the reimbursement of pharmaceuticals in Europe. Participants in MoCA	www.esip.eu
6.3. European social Insurance Platform (ESIP)	Alliance representing 34 social insurance organisations with the aim of promoting cooperation and information exchange	www.esip.eu
6.4. International Association of Mutual Benefit Societies (AIM)	umbrella organisation of non-profit healthcare payers, health mutuals and health insurance funds in Europe and in the world. with 63 members from 28 countries	http://aim-mutual.org/
6.5. EFPIA (European Federation of Pharmaceutical Industries and Associations)	33 European national pharmaceutical industry associations as well as 41 companies undertaking research, development and the manufacture in Europe of medicinal products for human use	www.efpia.eu
6.6. EuropaBio (European Association for Bioindustries)	Biotech trade group representing 77 corporate and associate members and bio regions, and 16 national biotechnology associations in turn representing over 1800 biotech SMEs	http://www.europabio.org/

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6.7. EUCOPE (EUCOPE – the European Confederation of Pharmaceutical Entrepreneurs)	Lobbying; The association provides its members with an early understanding about regulatory developments.	http://www.eucope.org/en/
7. NETWORKS & CONSORTIA		
7.1. Mechanism of Coordinated Access to Orphan Medicinal Products (MoCA)	A mechanism of collaboration among patient groups, payers and pharmaceutical companies to enhance access new to orphan medicinal products.	http://www.eurordis.org/content/moca
7.2. IMI (Innovative Medicines Initiative)	public-private initiative between the European Union and the pharmaceutical industry association EFPIA. It supports collaborative research projects and builds networks of industrial and academic experts in order to boost pharmaceutical innovation in Europe. IMI has been involved in projects on real-world data and patient-reported outcomes.	https://www.imi.europa.eu
7.3. ADAPT-SMART ("Accelerated Development of Appropriate Patient Therapies - a Sustainable, Multi-stakeholder Approach from Research to Treatment-outcomes)")	Enabling and coordination platform accompanying Adaptive Pathways, funded as an IMI project.	www.adapt-smart.eu
7.4. GetReal	Another IMI project. It is devoted to incorporating real-life clinical data into drug development	https://www.imi.europa.eu/content/getreal
7.5. OrphaNet	Reference portal for information on rare diseases and orphan drugs	www.orpha.net

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7.6. EUROPEAN REFERENCE NETWORKS	Network of specialists and experts for complex or rare medical conditions that require highly specialised healthcare and a concentration of knowledge and resources	http://ec.europa.eu/health/ern/policy/index_en.htm
7.7. BeNeLux+AT	to exchange data, share records and coordinate their evaluation methods, as well as to jointly examine how governments can best prepare for the arrival of new, expensive drugs on the market	http://www.chronicle.lu/categories luxembourgpolitics/item/17622-austria-joins-benelux-countries-collaboration-to-lower-orphan-drugs-prices http://www.bmgf.gv.at/home/Presse/Pressemeldungen/Oberhauser_Oesterreich_tritt_Benelux_Kooperation_fuer_Arzneimittelpolitik_bei
7.8. CEE/SEE Group	co-operation on co-ordinating approaches to the problem of access to high-cost medicines, and joint price negotiations	https://www.ihs.com/country-industry-forecasting.html?ID=10659116512