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SCIENCE MEDICINES HEALTH

Enabling Discovery, Development, and translation of treatments for Cognitive Dysfunctions in Depression: A Workshop
Session IV

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Disclaimer

The views expressed in this presentation are the personal views of the speaker and may not be understood nor quoted as being made on behalf of or reflecting the position of EMA or one of its committees or working parties or any of the national agencies.

Other positions:

•Vice Chair of the Psychopharmacology Special Committee of the Council of the Royal College of Psychiatrists, UK.

•Previous : Consultant Psychiatrist & Co-Director of Psychopharmacology Evaluation Unit at the South London & Maudsley NHS trust in London and Honorary Senior Lecturer in the Department of Forensic and Neurodevelopmental Sciences at the Institute of Psychiatry, Kings College London, UK.

Goals of this session:

- Provide an overview of the Qualification of Novel Methodologies procedure, SAWP & CHMP
- Establish the Regulatory framework for the Qualification Procedure
- Procedural aspects and definitions
- Parallel FDA-EMA qualification procedure
- Examples: EU-AIMS, MSOAC, CAMD….

CHMP centralised evaluation

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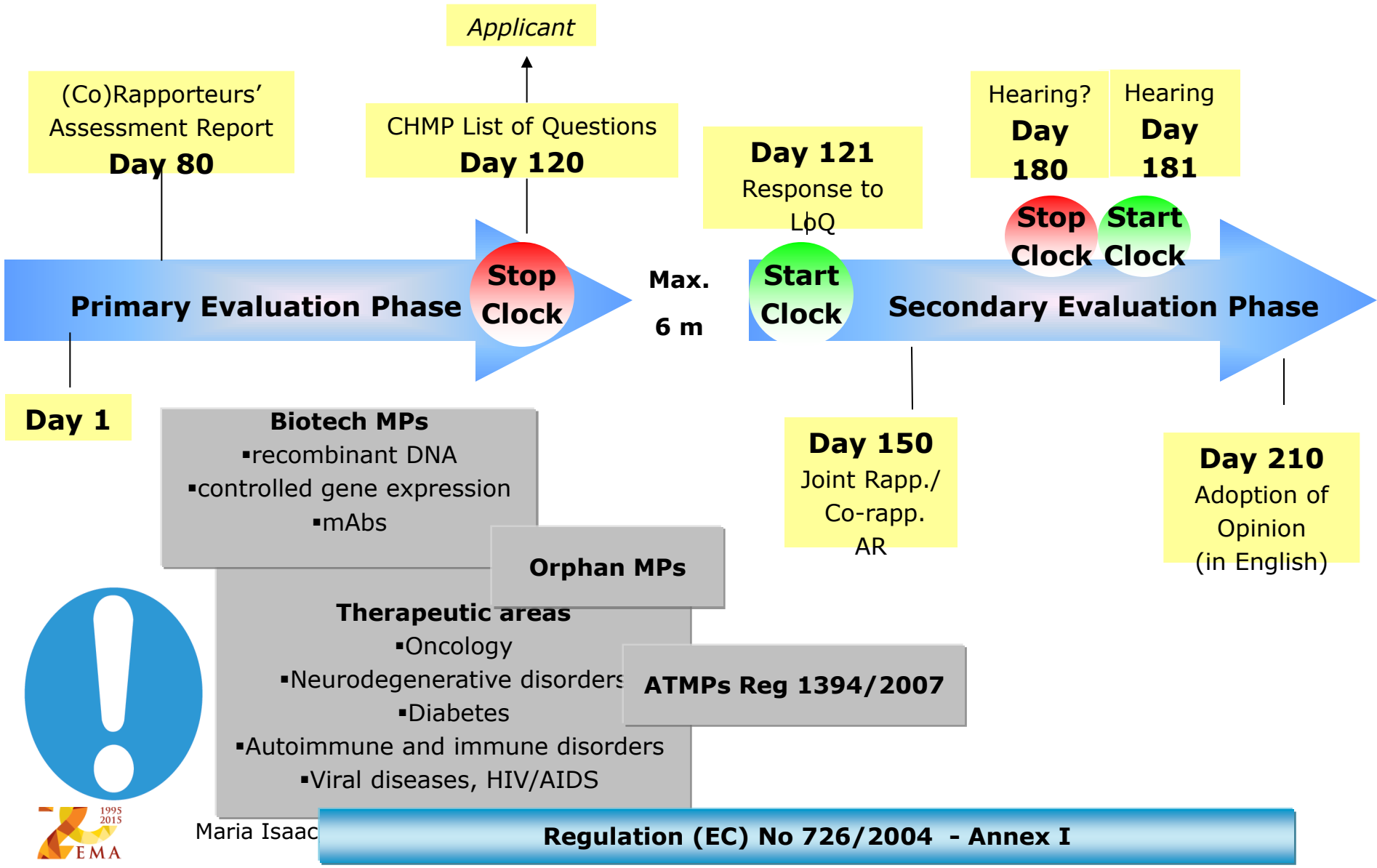

Scientific *A*dvice *W*orking *P*arty

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standing WP of the CHMP (Reg. 726/2004)

multidisciplinary expert group (28) selected by expertise (not MS)
16 NCAs, 12 academia; members of EMA committees 3 COMP, 1 CAT, 2 PDCO

CHMP peer-review, ad hoc discussions, adoption final advice letter

CMC: starting materials, specs, comparability, bridging…
non-clinical: overall toxicology plan registration, innovative models…
clinical pharmacology: PK/PD, modeling & simulation, BE…
clinical therapeutic areas: endpoints, population, comparator…
methodology, statistics: interim A, adaptive/seamless design…

network of external experts

EU Guidance for Qualification of Novel Methodologies

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- New regulatory procedure (2008, revised in 2014)
- Voluntary, scientific pathway for innovative methods or drug development tools (e.g. biomarkers) not yet integrated in the drug development and clinical management paradigm
- One procedure with two outcomes:
  - Qualification Advice, OR
  - Qualification Opinion

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09 January 2012
EMA/CHMP/SAWP/72894/2008 Rev.1[1]
Scientific Advice Working Party of CHMP

Qualification of novel methodologies for drug development: guidance to applicants

Agreed by SAWP	27 February 2008
Adoption by CHMP for release for consultation	24 April 2008
End of consultation (deadline for comments)	30 June 2008
Final Agreed by CHMP	22 January 2009

Keywords	*EMA. CHMP. Novel methodology. Qualification. Scientific Advice. Biomarker.*

Long-term benefits from EMA prespective: Speed-up the time to regulatory acceptance of novel approaches and time to new marketing authorisation applications

1995 2015 EMA

Qualification of Novel Methodologies

- **Vision**: Speed up/optimise drug development and utilisation, improve public health
- Procedure to guide the development of new more efficient ways to develop drugs, e.g. development of new endpoints for clinical trials:

  E.g. Can changes in chemicals (biochemistry) or structures (imaging/MRI) in the brain predict the development of Alzheimer's disease before the patients lose their memory and cannot function so that a medicine can intervene early on and be more effective?

- Started 2008: 60 procedures so far

Qualification Advice	Qualification Opinion
Prospective	Validation
PLANS i.e.: Draft protocols and development plans for future studies to establish the use of a defined novel methodology for a specific purpose and any data available to support these plans	**DATA** i.e.: Protocols, study reports and supportive data to establish the use of a defined novel methodology for a specific purpose in drug development
Advice on future protocols and methods for further development towards qualification	Acceptability of a specific use of the proposed methodology
Based on the evaluation of scientific rationales and preliminary data	Based on the assessment of submitted data
Confidential	Public – issued for public consultation
100 day-procedure	190 day-procedure

PARALLEL FDA-EMA Qualification ADVICE

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Voluntary, at request of sponsor

Questions on new methodology development put to both FDA and EMA

Discussions between FDA-EMA, and joint discussion with sponsor

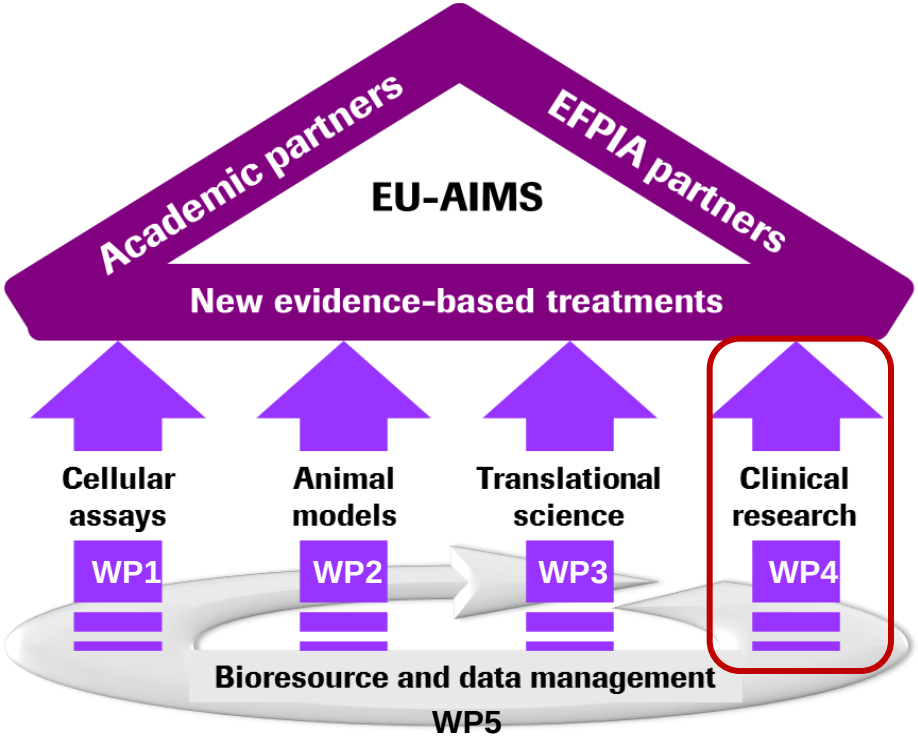
Each Agency will issue separate responses to sponsor's questions in line with usual procedures

→ Increased dialogue between Agencies and sponsor from early stages of development

→ Exchange views, share expertise

→ Optimise and facilitate global development, meeting both agencies requirements

EU-AIMS – Work Packages



- ✓ Identify BMs of ASD preceding onset of clinical symptoms
- ✓ Validate BM of ASD in children, adolescents and adults
- ✓ Identify molecular-physiologic pathways of drug responses
- ✓ Develop clinical infrastructure – extend COST and ECNP networks
- ✓ Develop better outcome instruments, clinical trials methodology, and regulatory framework
- ✓ Standards, training and dissemination

Qualification: IMI - EU-AIMS

V.1. **Measures of Executive Function and Theory of Mind Deficits in ASD**; EU-AIMS Consortium (IMI) (EMEA/H/SAB/044/1/QA/0000/PED)

V.2. **MRI and EEG in ASD**; EU-AIMS Consortium (IMI) (EMEA/H/SAB/045/1/QA/0000/PED)

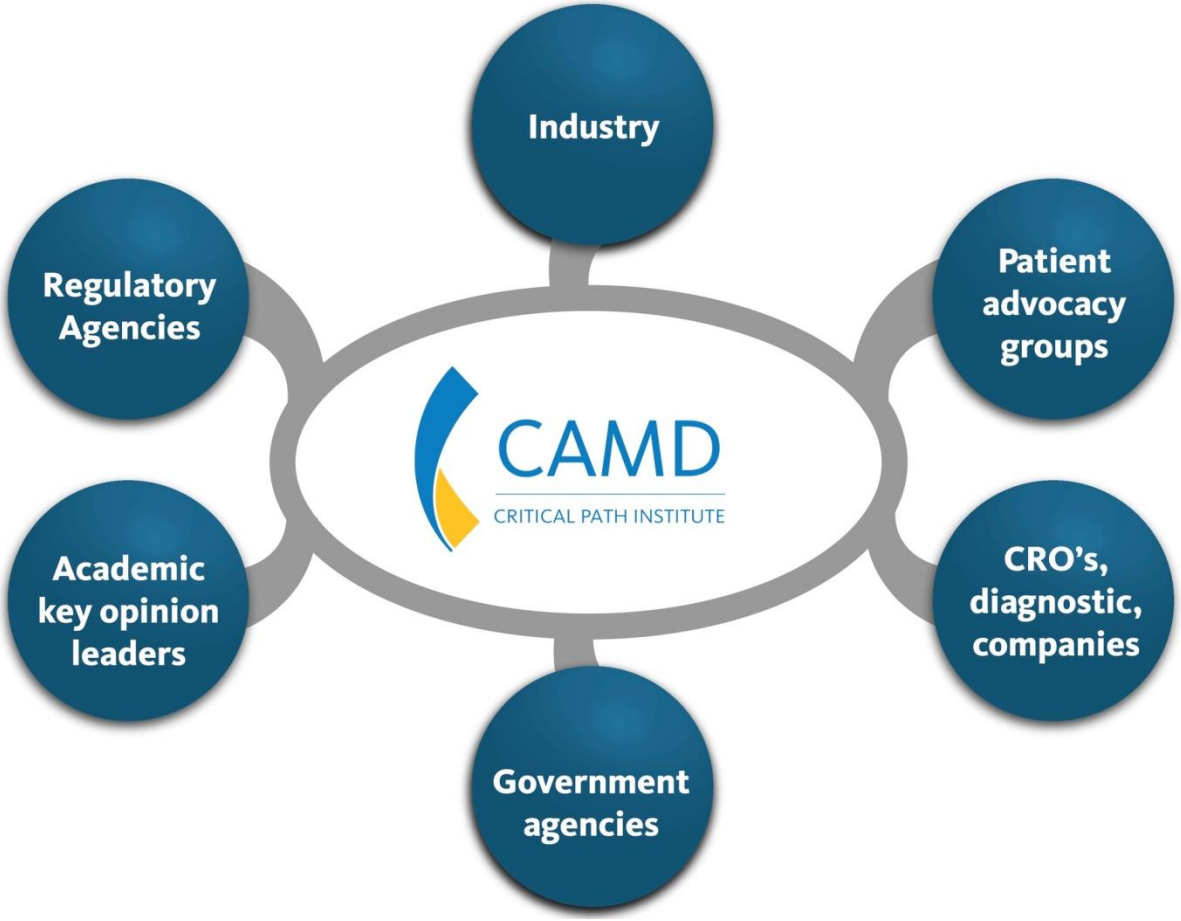
V.3. **5HT and p-Cresol Chemistry Biomarkers**; EU-AIMS Consortium (IMI) (EMEA/H/SAB/046/1/QA/0000/PED)

V.4. **Eye Tracking in ASD**; EU-AIMS Consortium (IMI) (EMEA/H/SAB/047/1/QA/0000/PED)

V.5. **Clinical Outcomes in ASD**; EU-AIMS Consortium (IMI) (EMEA/H/SAB/048/1/QA/0000/PED)

CAMD's Mission

Critical Path Institute's Coalition Against Major Diseases (CAMD) accelerates the development of therapies for Alzheimer's and Parkinson's diseases by generating the best methods and tools for evaluating drug efficacy, expediting clinical trials, and streamlining review by regulatory agencies.



Qualification of a Clinical Outcome Assessment Methodology for Multiple Sclerosis

CRITICAL PATH INSTITUTE

National Multiple Sclerosis Society

EMA SAWP
and
FDA QRT

Multiple Sclerosis Outcome Assessments Consortium (MSOAC)

Links

EMA guidance for companies requesting SA or PA

http://www.emea.europa.eu/pdfs/human/sciadvice/426001en.pdf

Qualification of novel methodologies for drug developments

http://www.emea.europa.eu/pdfs/human/biomarkers/7289408en.pdf

Scientific guidelines

Http://www.emea.europa.eu/htms/human/humanguidelines/background.htm

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